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Filed Pursuant to Rule 424(b)(4)
Registration No. 333-222990

PROSPECTUS

5,454,545 Shares

BioXcel Therapeutics, Inc.

Common Stock

This is the initial public offering of shares of common stock of BioXcel Therapeutics, Inc. We are offering 5,454,545 shares of our common stock. No public market currently exists for our stock.

We have been approved, subject to notice of issuance, to list our common stock on The Nasdaq Capital Market under the symbol "BTAI." Upon completion of this offering, we will be a "controlled company" as defined in the corporate governance rules of The Nasdaq Capital Market.

We are an "emerging growth company" as that term is used in the Jumpstart Our Business Startups Act of 2012 and, as such, have elected to comply with certain reduced public company reporting requirements.

Investing in our common stock involves risks. See "Risk Factors" beginning on page 13.

	Per Share	Total
Price to the public	$ 11.00	$ 59,999,995.00
Underwriting discounts and commissions	$ 0.77	$ 4,199,999.65
Proceeds to us (before expenses)[1]	$ 10.23	$ 55,799,995.35

[1] We refer you to "Underwriting" beginning on page 170 of this prospectus for additional information regarding underwriting compensation.

We have granted the underwriters a 30-day option to purchase up to 818,181 additional shares at the initial public offering price, less the underwriting discount.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or determined if this prospectus is truthful or complete. Any representation to the contrary is a criminal offense.

The underwriters expect to deliver the shares on or about March 12, 2018.

Joint Book-Running Managers

Barclays **UBS Investment Bank** **BMO Capital Markets**

Lead Manager

Canaccord Genuity

Prospectus dated March 7, 2018

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We have not, and the underwriters have not, authorized anyone to provide any information or to make any representations other than those contained in this prospectus or in any free writing prospectus prepared by or on behalf of us or to which we have referred you. We take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may give to you. The information contained in this prospectus is accurate only as of the date of this prospectus, regardless of the time of delivery of this prospectus or any sale of our common stock.

You should rely only on the information contained in this prospectus. No dealer, salesperson or other person is authorized to give information that is not contained in this prospectus. This prospectus is not an offer to sell nor is it seeking an offer to buy these securities in any jurisdiction where the offer or sale is not permitted. The information in this prospectus is accurate only as of the date of this prospectus, regardless of the time of delivery of this prospectus or of any sale of these securities.

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PROSPECTUS SUMMARY

The following summary highlights selected information contained elsewhere in this prospectus and is qualified in its entirety by the more detailed information and financial statements included elsewhere in this prospectus. It does not contain all the information that may be important to you and your investment decision. You should carefully read this entire prospectus, including the matters set forth under "Risk Factors," "Management's Discussion and Analysis of Financial Condition and Results of Operations," and our financial statements and related notes included elsewhere in this prospectus. In this prospectus, unless context requires otherwise, references to "we," "us," "our," "BTI" "BioXcel Therapeutics," or "the Company" refer to BioXcel Therapeutics, Inc. and references to "BioXcel" refer to our parent, BioXcel Corporation.

Overview

We are a clinical stage biopharmaceutical company focused on drug development that utilizes novel artificial intelligence, or AI, to identify the next wave of medicines across neuroscience and immuno-oncology. Our drug re-innovation approach leverages existing approved drugs and/or clinically validated product candidates together with big data and proprietary machine learning algorithms to identify new therapeutic indices. We believe that this differentiated approach has the potential to reduce the cost and time of drug development in diseases with substantial unmet medical need. Our two most advanced clinical development programs are BXCL501, a sublingual thin film formulation of dexmedetomidine, or Dex, designed for acute treatment of agitation resulting from neurological and psychiatric disorders, and BXCL701, an immuno-oncology agent designed for treatment of a rare form of prostate cancer and for treatment of pancreatic cancer. We initiated a Phase 1b pharmacokinetic/pharmacodynamic, or PK/PD, safety study using the IV formulation of Dex in mild probable Alzheimer's Disease, or AD, in December 2017 and we plan to initiate a Phase 1b PK/PD safety study using the IV formulation of Dex in schizophrenia patients in the first half of 2018. We expect to report data from both studies by the second half of 2018. We also intend to commence Phase 2 proof of concept, or PoC, open label clinical trials in 2018 for both programs. We expect that a data readout from the planned Phase 2 PoC clinical trials for the BXCL501 program will be available by the end of 2018, potentially leading to the start of registration trials, and that preliminary data from the planned Phase 2 PoC clinical trial of BXCL701 will be available in the first half of 2019. We retain global development and commercialization rights to these two programs.

We were formed to develop first-in-class, high value therapeutics by leveraging EvolverAI, a research and development engine created and owned by our parent, BioXcel Corporation, or BioXcel. We believe the combination of our therapeutic area expertise and our ability to generate product candidates through our exclusive collaborative relationship with BioXcel in the areas of neuroscience and immuno-oncology gives us a significant competitive advantage. EvolverAI was developed over the last decade and integrates millions of fragmented data points using artificial intelligence and proprietary machine learning algorithms. After evaluating multiple product candidates using EvolverAI, we selected our lead programs because our analysis indicated these drugs may have utility in new therapeutic indices where there is substantial unmet medical needs and limited competition. By focusing on clinical candidates with relevant human data, we believe our approach will help us design more efficient clinical trials, thereby accelerating our product candidates' time to market.

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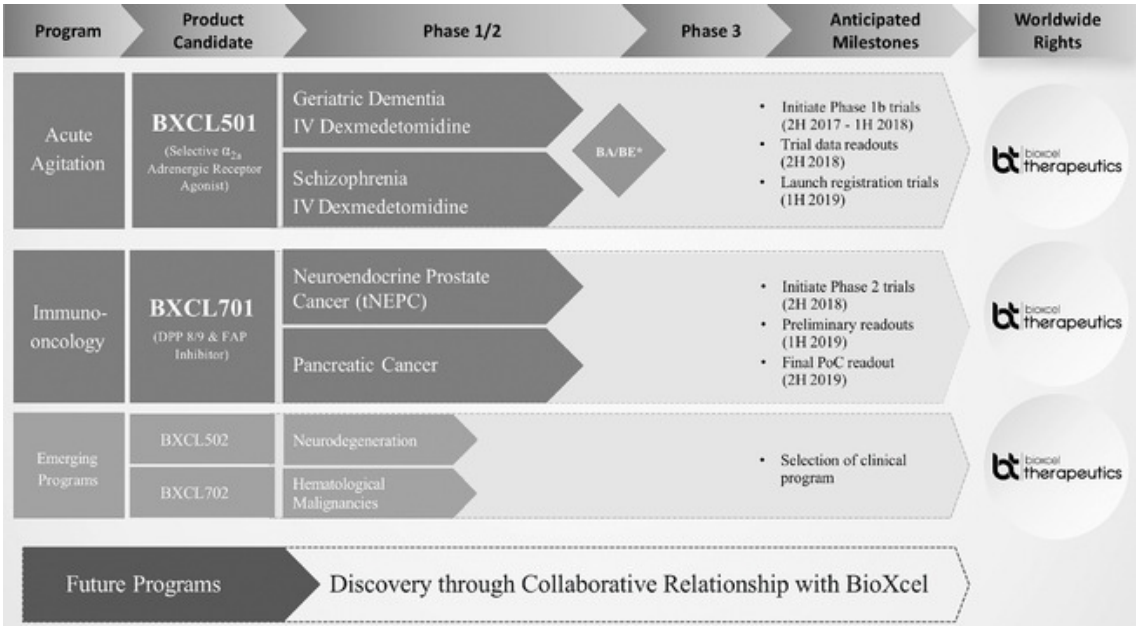
Product Candidates

The following table summarizes our lead development programs. We believe our product candidates have the potential to be first-in-class treatment options for their indications:



* Bridging bioavailability/bioequivalence (BA/BE) study for optimizing BXCL501 sublingual thin film dose for Phase 3 registration trials

There is currently no active IND for any of our product candidates in the United States, however, our initial clinical trial of the IV formulation of Dex for mild probable AD was granted an IND exemption by the FDA on September 25, 2017. There has been no authorization received from any other drug regulatory authority.

BXCL501, Potential First-in-Class Sublingual Thin Film, α_{2a}Adrenergic Receptor Agonist, for Acute Treatment of Agitation

BXCL501 is a potential first-in-class sublingual thin film formulation of Dex designed for acute treatment of agitation in neurodegenerative and psychiatric disorders. Dex has been well tolerated, having been prescribed in millions of patients as the sedative and anesthetic Precedex and has been studied in over 130 clinical trials to date. BXCL501 is designed to be a non-invasive, easy to administer agent that has a rapid onset of action, which is critical for the acute treatment of agitation. We estimate that over 500,000 patients who suffer from AD in the United States annually could be eligible for the acute treatment of agitation with BXCL501. In schizophrenia and bipolar disease, we estimate that over 600,000 patients in the United States annually could be eligible for the acute treatment of agitation with BXCL501. The current treatment options for agitation utilize antipsychotics and benzodiazepines, which have suboptimal safety and compliance issues. Antipsychotics have a black box warning for use in the elderly, can produce debilitating side effects when given acutely and should only be considered for invasive intramuscular, or IM, delivery in highly aggressive patients requiring restraint. Benzodiazepines are predominantly in pill form, which require swallowing and can produce excessive sedation. We believe that BXCL501, with its differentiated pharmacology and ease of administration, if approved, could potentially be a first-in-class, non-invasive acute treatment for mild to moderate agitation.

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We have designed a dual clinical development program intended to take advantage of the U.S. Food and Drug Administration's, or FDA, Section 505(b)(2) regulatory pathway and leverage the existing clinical and safety dataset of intravenous, or IV, formulation of Dex. We initiated a Phase 1b single ascending or descending dose study using the IV formulation of Dex in mild probable AD in December 2017 and we plan to initiate a Phase 1b single ascending or descending dose study in schizophrenia patients in the first half of 2018, followed by a PoC open label clinical trial. We expect to report data from both studies by the second half of 2018. We intend to initiate a bridging bioavailability/bioequivalence, or BA/BE, study with the sublingual thin film formulation in the second half of 2018 that, if successful, could potentially lead to the start of a registration trial in the first half of 2019. We plan on submitting an NDA for BXCL501 in 2020.

BXCL701, Potential First-in-Class DPP 8/9 and FAP Inhibitor for the Treatment of tNEPC and Pancreatic Cancer

BXCL701 is a potential first-in-class, highly potent oral small molecule immuno-modulator that is designed to stimulate both the innate and acquired immune systems by inhibiting dipeptidyl peptidase, or DPP, 8/9 and fibroblast activation protein, or FAP. DPP 8/9 have been show recently to behave as an "immuno-checkpoint" of the immune system, as their inhibition results in a potent pro-inflammatory, anti-tumor activity by way of the induction of cell death in the macrophages and the downstream stimulation of multiple tumor-killing immune cells. BXCL701 is differentiated among DPP inhibitors for its specificity to inhibit DPP 8/9 and FAP, whereas most other approved or clinical stage DPP inhibitors, developed to treat diabetes, are selective for DPP 4. Based on our analysis, we believe that BXCL701 establishes a differentiated immuno-oncology platform by modulating multiple steps in the cancer immunity cycle, and in combination with checkpoint inhibitors can convert immuno-resistant tumors to immuno-sensitive tumors ("cold" to "hot" tumors). BXCL701 has been tested in more than 700 healthy subjects and cancer patients across multiple clinical trials, providing evidence of being well tolerated, proof of mechanism, and single agent anti-tumor activity in patients with melanoma, an immuno-sensitive tumor. We believe that we can leverage this clinical data to determine the dose to use in future clinical trials and support accelerated clinical development. BXCL701 is a potential novel therapy for treatment-emergent neuroendocrine prostate cancer, or tNEPC, a segment of prostate cancer patients that have progressed on second-generation androgen inhibitors (Zytiga and Xtandi), and is also a potential treatment for pancreatic cancer, both of which are rare diseases.

We selected tNEPC and pancreatic cancer as our lead indications after evaluating more than 100 different tumor types because they are two of the top three cancers that overexpressed or amplified DPP 8/9 and FAP. Additional data points to a functional role of DPP 8/9 in the biology of tNEPC. The combined global sales of Zytiga and Xtandi, which are only approved for prostate cancer treatment, were over $4.5 billion in 2016. Approximately one in three patients on these drugs are expected to develop tNEPC and could be eligible for treatment with BXCL701 based on information in an article published in the Journal of the National Comprehensive Cancer Network in 2014 by Agarwal et. al. and an article published by the Journal of Clinical Oncology in 2014 by Wang et. al. In pancreatic cancer, we estimate that approximately 20,000 patients will be eligible for treatment with BXCL701 annually as about 50% of pancreatic cancer patients can receive 2nd line therapy based on information in an article published in the Annals of Oncology in 2013 by Rahma et. al. We plan to initiate two Phase 2 PoC open label clinical trials in the second half of 2018, as a single agent and in combination with Keytruda in patients with tNEPC, and in combination with Keytruda in pancreatic cancer. We expect to receive preliminary data in the first half of 2019 and intend to pursue breakthrough therapy designation and accelerated approval pathways for both indications. BXCL701 has already received orphan drug designation by the FDA for the treatment of pancreatic cancer.

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Emerging Programs

We intend to grow our pipeline with additional development candidates by leveraging our management team's therapeutic area expertise with EvolverAI. We believe EvolverAI is a novel method of finding potential product candidates because it combines the comprehensiveness and efficiency of machine learning and big data analytics with the expertise and intuition of human experience in drug development. We are also exploring development of BXCL502, a novel approach to the treatment of symptoms resulting from neurological disorders, and BXCL702, an immuno-oncology agent targeting hematological malignancies for which we have received orphan drug designation from the FDA for the treatment of acute myeloid leukemia, or AML. We retain global development and commercialization rights to these two programs. We intend to select our next clinical program in 2018 from our emerging or future programs.

Our Strategy

Our goal is to become a leader in the field of neuroscience and immuno-oncology. The key elements to achieving this goal are to:

- **Advance BXCL501 for the acute treatment of agitation through the FDA Section 505(b)(2) pathway.** We are pursuing a dual clinical development program and plan to initiate two Phase 1b single ascending or descending dose studies of the IV formulation of Dex in mild probable AD and schizophrenia patients in the first half of 2018, followed by PoC open label clinical trials. We intend to initiate a bridging BA/BE study with the sublingual thin film formulation in the second half of 2018 to identify the optimal dose range for our planned registration trial in the first half of 2019.

- **Advance BXCL701 into Phase 2 trials to assess its potential to be the first approved therapy for tNEPC and for the treatment of pancreatic cancer.** We plan to initiate two Phase 2 PoC open label clinical trials in the second half of 2018, as a single agent and in combination with Keytruda in patients with tNEPC and in combination with Keytruda in pancreatic cancer. We expect to receive preliminary data in the first half of 2019 and intend to pursue breakthrough therapy designation and accelerated approval pathways for both indications.

- **Maximize the therapeutic and commercial potential of BXCL501 and BXCL701 by exploring their use for multiple indications.** Based on the broad applicability of the mechanisms of action of our two lead product candidates, we intend to explore a series of follow-on indications for BXCL501 (acute treatment of agitation resulting from delirium, substance abuse withdrawal and PTSD) and BXCL701 (potential as a combination agent for multiple tumor indications, offering a "pipeline in a product" platform).

- **Identify biomarkers to select patients who have the highest likelihood to respond to our product candidates.** Predicting optimal drug responses in patients requires the identification and validation of predictive biomarkers, specifically in cancer. The indications for our product candidate BXCL701 were chosen in part because they are known to overexpress DPP 8/9 and FAP. Our planned PoC clinical trial of BXCL701 will examine biomarkers related to its molecular and cellular targets to identify those that may correlate with clinical efficacy and increase our likelihood of success.

- **Enhance our R&D pipeline by leveraging our therapeutic area expertise with EvolverAI to identify, develop and commercialize new product candidates in neuroscience and immuno-oncology.** In addition to our leading clinical programs and our emerging and future pipeline, we intend to select our next clinical program during 2018. We have established translational and development expertise, which we believe will help us advance the present and future product candidates in these fields. We may also opportunistically in-license additional

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product candidates identified through our AI platform approach within our core areas of expertise.

- **Maximize the commercial potential of our product candidates.** We have worldwide development and commercialization rights to our BXCL501, BXCL701, BXCL502 and BXCL702 product candidates. If BXCL501 and BXCL701 are approved in the United States, we would consider building a specialty sales force in the United States and/or collaborate with third parties to maximize the potential of our product candidates. Furthermore, we intend to commercialize BXCL501 and BXCL701 outside the United States through collaborations with third parties.

Our Team

We have assembled a management team with extensive experience in the discovery, development and approval of more than 10 drugs and who have held senior executive roles at leading pharmaceutical companies, including: our co-founder and Chief Executive Officer, Vimal Mehta, Ph.D., our Chief Scientific Officer, Frank Yocca, Ph.D., our Chief Medical Officer, Vince O'Neill, M.D., our Vice President—Oncology R&D, Luca Rastelli, Ph.D., and our Chief Financial Officer, Richard Steinhart. We are also supported by our experienced board of directors and advisory board, which includes Drs. Peter Mueller (Vertex, Boehringer Ingelheim), Steven Paul (Voyager Therapeutics, Sage Therapeutics, Eli Lilly) and Sheila Gujrathi (Receptos, Bristol-Myers Squibb, Roche), who contribute to our strategy with their expertise in building public companies. We believe that our team is ideally positioned to leverage our highly differentiated platform to develop the next wave of innovative medicines.

Our Relationship with BioXcel

We are currently a 93% owned subsidiary of BioXcel. After the closing of this offering, we expect to be a "controlled company" within the meaning of the corporate governance rules of The Nasdaq Capital Market. Assuming we sell the number of the shares set forth on the cover page of this prospectus, BioXcel will own, in the aggregate, approximately 61% of our outstanding common shares, or approximately 58% if the underwriters exercise their option to purchase additional common shares in full. BioXcel will be able to exercise control over all matters requiring shareholder approval, including the election of our directors and approval of significant corporate transactions.

We have entered into an asset contribution agreement, effective June 30, 2017, with BioXcel, as amended and restated on November 7, 2017, or the Contribution Agreement, pursuant to which BioXcel agreed to contribute to us, and we agree to acquire from BioXcel, all of BioXcel's rights, title and interest in and to BXCL501, BXCL701, BXCL502 and BXCL702, collectively, the Candidates, and all of the assets and liabilities associated with the Candidates. See the section titled "Certain Relationships and Related Person Transactions—Amended and Restated Asset Contribution Agreement with BioXcel" for additional information.

We have entered into a separation and shared services agreement with BioXcel that took effect on June 30, 2017, as amended and restated on November 7, 2017, or the Services Agreement, pursuant to which BioXcel will allow us to continue to use its office space, equipment, services and leased employees based on the agreed upon terms and conditions for a payment of defined monthly and/or hourly fees. Under this agreement, BioXcel will continue to make such product identification and related services available to us for at least five years. See the section titled "Certain Relationships and Related Person Transactions—Amended and Restated Separation and Shared Services Agreement with BioXcel" for additional information.

We refer to the agreements set forth above and the series of transactions related to our separation from BioXcel, collectively, as the "Separation."

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We believe that a distribution of BTI shares by BioXcel to BioXcel shareholders would be advantageous to the market for our shares by increasing liquidity, would accelerate our ability to become independent from BioXcel by decreasing BioXcel's ownership of our common stock and would be beneficial for BioXcel's stockholders who would have a direct opportunity to participate in the BTI value proposition. BioXcel has advised us that, following the completion of this offering and subject to the expiration of any applicable lock-up periods or other agreements we have or may have with BioXcel described herein, it does not have any near-term plans to distribute our shares held by BioXcel to the BioXcel stockholders. The decision to conduct any such distribution is at the sole discretion of BioXcel's board of directors. There is no assurance that the distribution will ever occur. Presently, it is expected that any potential distribution will be taxable to BioXcel and its stockholders. We refer to any such potential distribution as the "Distribution."

Risks Associated with Our Business

Our business is subject to a number of risks of which you should be aware before making a decision to invest in our common shares. These risks are discussed more fully in the "Risk Factors" section of this prospectus. These risks include the following:

- We have a limited operating history and have never generated any product revenues, which may make it difficult to evaluate the success of our business to date and to assess our future viability.
- We have incurred significant operating losses since inception and anticipate that we will continue to incur substantial operating losses for the foreseeable future and may never achieve or maintain profitability.
- Even if this offering is successful, we will need substantial additional funding, and if we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate our product development programs or commercialization efforts.
- We have limited experience in drug discovery and drug development, and we have never had a drug approved.
- In the near term, we are dependent on the success of BXCL501 and BXCL701. If we are unable to initiate or complete the clinical development of, obtain marketing approval for or successfully commercialize BXCL501, BXCL701 and our other product candidates, either alone or with a collaborator, or if we experience significant delays in doing so, our business could be substantially harmed.
- The regulatory approval processes of the FDA and comparable foreign authorities are lengthy, time consuming, expensive and inherently unpredictable, and if we are ultimately unable to obtain regulatory approval for our product candidates, our business will be substantially harmed.
- BioXcel's approach to the discovery and development of product candidates based on EvolverAI is novel and unproven, and we do not know whether we will be able to develop any products of commercial value.
- We will be substantially dependent on third parties for the manufacture of our clinical supplies of our product candidates. Therefore the development of our products could be stopped or delayed, and our commercialization of any future product could be stopped or delayed or made less profitable if third party manufacturers fail to provide us with sufficient quantities at acceptable prices.
- BioXcel controls the direction of our business, and the concentrated ownership of our common stock will prevent you and other stockholders from influencing significant decisions.

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- Following this offering, we will continue to depend on BioXcel to provide us with certain services for our business.
- We may be a "controlled company" within the meaning of the Nasdaq rules and, as a result, may qualify for, and may rely on, exemptions from certain corporate governance requirements that provide protection to stockholders of other companies.
- It is difficult and costly to protect our proprietary rights, and we may not be able to ensure their protection. If our patent position does not adequately protect our product candidates, others could compete against us more directly, which would harm our business, possibly materially.
- An active trading market for our common stock may not develop, and you may not be able to sell your common stock at or above the initial public offering price.
- We are an "emerging growth company" and will be able to avail ourselves of reduced disclosure requirements applicable to emerging growth companies, which could make our common stock less attractive to investors.

Corporate Information

We were incorporated as a Delaware corporation on March 29, 2017 as a wholly-owned subsidiary of BioXcel. Our principal executive offices are located at 780 East Main St., Branford, CT 06405 and our telephone number is (203) 643-8060. Our website address is www.bioxceltherapeutics.com. The information contained on our website is not incorporated by reference into this prospectus, and you should not consider any information contained on, or that can be accessed through, our website as part of this prospectus or in deciding whether to purchase our common shares.

We have proprietary rights to a number of trademarks used in this prospectus which are important to our business, including the BTI logo. Solely for convenience, the trademarks and trade names in this prospectus are referred to without the ® and ™ symbols, but such references should not be construed as any indicator that their respective owners will not assert, to the fullest extent under applicable law, their rights thereto. All other trademarks, trade names and service marks appearing in this prospectus are the property of their respective owners.

Implications of Being an Emerging Growth Company

As a company with less than $1.07 billion in revenues during our last fiscal year, we qualify as an emerging growth company as defined in the Jumpstart Our Business Startups Act, or the JOBS Act, enacted in 2012. As an emerging growth company, we expect to take advantage of reduced reporting requirements that are otherwise applicable to public companies. These provisions include, but are not limited to:

- being permitted to present only two years of audited financial statements, in addition to any required unaudited interim financial statements, with correspondingly reduced "Management's Discussion and Analysis of Financial Condition and Results of Operations" disclosure in this prospectus;
- not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, as amended;
- reduced disclosure obligations regarding executive compensation in our periodic reports, proxy statements and registration statements; and
- exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and shareholder approval of any golden parachute payments not previously approved.

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We may use these provisions until the last day of our fiscal year following the fifth anniversary of the completion of this offering. However, if certain events occur prior to the end of such five-year period, including if we become a "large accelerated filer," our annual gross revenues exceed $1.07 billion or we issue more than $1.0 billion of non-convertible debt in any three-year period, we will cease to be an emerging growth company prior to the end of such five-year period.

We have chosen to opt out of the extended transition periods available to emerging growth companies under the JOBS Act for complying with new or revised accounting standards. Section 107 of the JOBS Act provides that our decision to opt out of the extended transition periods for complying with new or revised accounting standards is irrevocable.

The JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. We have irrevocably elected not to avail ourselves of this exemption and, therefore, we will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

To the extent that we continue to qualify as a "smaller reporting company," as such term is defined in Rule 12b-2 under the Securities Exchange Act of 1934, after we cease to qualify as an emerging growth company, certain of the exemptions available to us as an emerging growth company may continue to be available to us as a smaller reporting company, including: (i) not being required to comply with the auditor attestation requirements of Section 404(b) of the Sarbanes Oxley Act; (ii) scaled executive compensation disclosures; and (iii) the requirement to provide only two years of audited financial statements, instead of three years.

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THE OFFERING

Common stock offered by us	5,454,545 shares
Common stock to be outstanding immediately after this offering	15,645,545 shares (16,463,726 shares if the underwriters exercise their option in full)
Option to purchase additional shares	The underwriters have an option for a period of 30 days to purchase up to an additional 818,181 shares of our common stock.
Use of proceeds	We estimate that the net proceeds from this offering will be approximately $54.2 million, or approximately $62.6 million if the underwriters exercise their over-allotment option in full, based on an initial public offering price of $11.00 per share and after deducting the underwriting discounts and commissions and estimated offering expenses payable by us. We intend to use the net proceeds from this offering to fund our planned clinical development of BXCL501 through Phase 2 clinical development and potentially commence one registration trial, to fund our planned clinical development of BXCL701 through Phase 2 clinical development, to make certain payments to BioXcel, and for general corporate purposes and working capital. We may also use a portion of the net proceeds to in-license, acquire or invest in complementary businesses or products, however, we have no current commitments or obligations to do so. See "Use of Proceeds" for a more complete description of the intended use of proceeds from this offering.
Controlled company	Upon the closing of this offering, BioXcel Corporation will beneficially own a controlling interest in us and we expect to be a "controlled company" under Nasdaq rules. As a controlled company, we may elect to avail ourselves of the controlled company exemption under the corporate governance requirements of the Nasdaq.
Directed share program	At our request, the underwriters have reserved up to 5% of the common stock being offered by this prospectus for sale at the initial public offering price to our directors, officers, employees and other individuals associated with us and members of their families. The sales will be made by UBS Financial Services Inc., a selected dealer affiliated with UBS Securities LLC, an underwriter of this offering, through a directed share program. We do not know if these persons will choose to purchase all or any portion of these reserved shares, but any purchases they do make will reduce the number of shares available to the general public. Any reserved shares not so purchased will be offered by the underwriters to the general public on the same terms as the other shares of common stock. Participants in the directed share program who purchase $100,000 or more of shares of our common stock will be subject to a 30-day lock-up with respect to any shares sold to them pursuant to the program. Any shares sold in the directed share program to our directors or executive officers will be subject to a 180-day lock-up. All of these lock-up agreements will have similar restrictions to the lock-up agreements described herein. See "Underwriting—Directed Share Program."

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Risk factors	See "Risk Factors" on page 14 and other information included in this prospectus for a discussion of factors to consider carefully before deciding to invest in shares of our common stock.
Nasdaq Capital Market symbol	"BTAI"

The number of shares of our common stock to be outstanding after this offering is based on 9,907,548 shares of our common stock outstanding as of December 31, 2017, and excludes:

- the sale of 283,452 shares of common stock in January and February 2018, at a price of $6.88 per share;
- 2,303,877 shares of common stock issuable upon exercise of stock options outstanding as of December 31, 2017, 2,219,979 of which are at an exercise price of $0.41 per share and 83,898 of which are at an exercise price of $5.55; and
- 1,158,693 shares of common stock reserved for future issuance under our 2017 Equity Incentive Plan.

Except as otherwise indicated herein, all information in this prospectus, including the number of shares of common stock that will be outstanding after this offering, assumes or gives effect to

- a 237-for-1 stock split of our common stock effected on March 7, 2018;
- no exercise by the underwriters of their option to purchase an additional 818,181 shares of common stock;
- no exercise of outstanding options after December 31, 2017; and
- the effectiveness of our amended and restated certificate of incorporation and the adoption of our amended and restated bylaws immediately prior to the closing of this offering.

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Summary Financial Data

The following table sets forth our summary financial data as of the dates and for the periods indicated. We have derived the summary statement of operations data for the years ended December 31, 2017 and 2016 from our audited financial statements included elsewhere in this prospectus. The following summary financial data should be read with "Management's Discussion and Analysis of Financial Condition and Results of Operations" and our financial statements and related notes and other information included elsewhere in this prospectus. Our historical results are not necessarily indicative of the results to be expected in future periods.

Our historical results of operations presented below may not be reflective of our financial position, results of operations and cash flows had we operated as a stand-alone public company during all periods presented. Prior to June 30, 2017, we operated as part of BioXcel and not as a separate stand-alone entity. Our financial statements prior to June 30, 2017 have been prepared on a "carve-out" basis from the financial statements of BioXcel to represent our financial position and performance as if we had existed on a stand-alone basis during each of the fiscal years presented in the financial statements. The financial information for the period beginning January 1, 2017 through June 30, 2017 have been carved out of the financial statements of BioXcel. Our financial information for the period beginning July 1, 2017 through December 31, 2017 have been prepared as if we are a standalone entity. These results reflect amounts specifically attributable to our business, including the costs BioXcel incurred for the assets that were contributed to us by our parent under the Contribution Agreement and the Services Agreement. The agreements provide us with certain general and administrative and development support services that became effective June 30, 2017. However, during the carve-out period, consistent with accounting regulations, we have assumed that we were a separate business within BioXcel and we have reflected the related assets, liabilities and expenses in our results for periods prior to and post incorporation. We believe that such allocations have been made on a reasonable basis, but may not necessarily be indicative of all of the costs that would have been incurred if we had operated on a standalone basis.

Statement of Operations Data:

(in thousands, except share and per share data)

	Years Ended December 31,	
	2017	2016
Revenues	$ —	$ —
Operating costs and expenses		
Research and development	2,690	1,399
General and administrative	1,847	721
Total operating expenses	4,537	2,120
Loss from operations	(4,537)	(2,120)
Other expense		
Interest expense	(2)	—
Net loss	$ (4,539)	$ (2,120)
Net loss per share—basic and diluted[1]	$ (0.47)	$ (0.22)
Weighted average shares outstanding—basic and diluted[1]	9,685,005	9,480,000

[1] See Note 3 to our financial statements for an explanation of the method used to compute basic and diluted net loss per share.

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Balance Sheet Data:

(in thousands)

	December 31, 2017		
		(unaudited)	
	Actual	Pro Forma[1]	Pro Forma, As Adjusted[2]
Cash and cash equivalents	$ 887	$ 2,836	$ 57,036
Working capital (deficit)	(1,447)	502	54,702
Total assets	1,355	3,304	57,504
Total liabilities	2,337	2,337	2,337
Share capital	10	10	15
Accumulated deficit	(4,450)	(4,450)	(4,450)
Total stockholders' equity/(deficit)	$ (982)	$ 967	$ 55,167

1 On a pro forma basis to reflect the sale of 283,452 shares of common stock, at a price of $6.88 per share in January and February 2018.

2 On a pro forma as adjusted basis to give further effect to our issuance and sale of 5,454,545 shares of common stock in this offering at the initial public offering price of $11.00 per share, after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

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RISK FACTORS

An investment in our common stock involves a high degree of risk. Before making an investment decision, you should give careful consideration to the following risk factors, in addition to the other information included in this prospectus, including our financial statements and related notes, before deciding whether to invest in shares of our common stock. The occurrence of any of the adverse developments described in the following risk factors could materially and adversely harm our business, financial condition, results of operations or prospects. In that case, the trading price of our common stock could decline, and you may lose all or part of your investment.

Risks Related to Financial Position and Need for Capital

We have a limited operating history and have never generated any product revenues, which may make it difficult to evaluate the success of our business to date and to assess our future viability.

We were incorporated in March 2017 and our operations to date have been largely focused on organizing and staffing our company, raising capital and acquiring the rights to, and advancing the development of, our product candidates, including conducting preclinical studies. We have not yet demonstrated an ability to successfully complete clinical trials, obtain marketing approvals, manufacture products on a commercial scale, or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful commercialization. Consequently, predictions about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully developing and commercializing products.

We expect our financial condition and operating results to continue to fluctuate from quarter to quarter and year to year due to a variety of factors, many of which are beyond our control. We will need to eventually transition from a company with a research and development focus to a company capable of undertaking commercial activities. We may encounter unforeseen expenses, difficulties, complications and delays, and may not be successful in such a transition.

We have incurred significant operating losses since inception and anticipate that we will continue to incur substantial operating losses for the foreseeable future and may never achieve or maintain profitability.

Since our inception, we have incurred significant operating losses. Our net loss was $4.5 million, and $2.1 million for the years ended December 31, 2017 and 2016, respectively. As of December 31, 2017, we had an accumulated deficit of $4.5 million. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. None of our product candidates have been approved for marketing in the United States, or in any other jurisdiction, and may never receive such approval. It could be several years, if ever, before we have a commercialized product that generates significant revenues. As a result, we are uncertain when or if we will achieve profitability and, if so, whether we will be able to sustain it. The net losses we incur may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase substantially as we:

- continue the development of our product candidates;
- initiate preclinical studies and clinical trials for any additional indications for our current product candidates and any future product candidates that we may pursue;
- continue to build our portfolio of product candidates through the acquisition or in-license of additional product candidates or technologies;
- continue to develop, maintain, expand and protect our intellectual property portfolio;
- pursue regulatory approvals for our current and future product candidates that successfully complete clinical trials;

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- ultimately establish a sales, marketing and distribution infrastructure to commercialize any product candidate for which we may obtain marketing approval;
- hire additional clinical, regulatory, scientific and accounting personnel; and
- incur additional legal, accounting and other expenses in operating as a public company.

To become and remain profitable, we must develop and eventually commercialize one or more product candidates with significant market potential. This will require us to be successful in a range of challenging activities, including completing clinical trials of our product candidates, developing commercial scale manufacturing processes, obtaining marketing approval, manufacturing, marketing and selling any current and future product candidates for which we may obtain marketing approval, and satisfying any post-marketing requirements. We are only in the preliminary stages of most of these activities and, in some cases, have not yet commenced certain of these activities. We may never succeed in any or all of these activities and, even if we do, we may never generate sufficient revenue to achieve profitability.

Because of the numerous risks and uncertainties associated with product development, we are unable to accurately predict the timing or amount of expenses or when, or if, we will obtain marketing approval to commercialize any of our product candidates. If we are required by the U.S. Food and Drug Administration, or FDA, or other regulatory authorities such as the European Medicines Agency, or EMA, to perform studies and trials in addition to those currently expected, or if there are any delays in the development, or in the completion of any planned or future preclinical studies or clinical trials of our current or future product candidates, our expenses could increase and profitability could be further delayed.

Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of our company also could cause you to lose all or part of your investment.

Even if this offering is successful, we will need substantial additional funding, and if we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate our product development programs or commercialization efforts.

We anticipate that our expenses will increase substantially if and as we continue to develop and begin clinical trials with respect to BXCL501, BXCL701 and our other product candidates; seek to identify and develop additional product candidates; acquire or in-license other product candidates or technologies; seek regulatory and marketing approvals for our product candidates that successfully complete clinical trials, if any; establish sales, marketing, distribution and other commercial infrastructure in the future to commercialize various products for which we may obtain marketing approval, if any; require the manufacture of larger quantities of product candidates for clinical development and, potentially, commercialization; maintain, expand and protect our intellectual property portfolio; hire and retain additional personnel, such as clinical, quality control and scientific personnel; add operational, financial and management information systems and personnel, including personnel to support our product development and help us comply with our obligations as a public company; and add equipment and physical infrastructure to support our research and development programs.

We plan to use the net proceeds of this offering primarily to fund our ongoing research and development efforts over the coming months. We will be required to expend significant funds in order to advance the development of BXCL501, BXCL701 and our other product candidates. In addition, while we may seek one or more collaborators for future development of our current product candidate or any future product candidates that we may develop for one or more indications, we may not be able

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to enter into a collaboration for any of our product candidates for such indications on suitable terms, on a timely basis or at all. In any event, the net proceeds of this offering and our existing cash and cash equivalents will not be sufficient to fund all of the efforts that we plan to undertake or to fund the completion of development of our product candidates or our other preclinical programs. Accordingly, we will be required to obtain further funding through public or private equity offerings, debt financings, collaborations and licensing arrangements or other sources. Other than our grid note with BioXcel, we do not have any committed external source of funds. Further financing may not be available to us on acceptable terms, or at all. Our failure to raise capital as and when needed would have a negative impact on our financial condition and our ability to pursue our business strategy.

We believe that the net proceeds from this offering, together with our existing cash and cash equivalents as of December 31, 2017, will enable us to fund our operating expenses and capital expenditure requirements for at least twelve months from the date of this prospectus. Our estimate as to how long we expect the net proceeds from this offering, together with our existing cash and cash equivalents, to be able to continue to fund our operations is based on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect. Further, changing circumstances, some of which may be beyond our control, could cause us to consume capital significantly faster than we currently anticipate, and we may need to seek additional funds sooner than planned. Our future funding requirements, both short-term and long-term, will depend on many factors, including:

- the scope, progress, timing, costs and results of clinical trials of BXCL501, BXCL701 and our other product candidates;
- our ability to enter into and the terms and timing of any collaborations, licensing agreements or other arrangements;
- the costs, timing and outcome of seeking regulatory approvals;
- the costs of commercialization activities for any of our product candidates that receive marketing approval to the extent such costs are not the responsibility of any future collaborators, including the costs and timing of establishing product sales, marketing, distribution and manufacturing capabilities;
- our headcount growth and associated costs as we expand our research and development as well as potentially establish a commercial infrastructure;
- revenue received from commercial sales, if any, of our current and future product candidates;
- the costs of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights and defending against intellectual property related claims;
- the number of future product candidates that we pursue and their development requirements;
- changes in regulatory policies or laws that may affect our operations;
- changes in physician acceptance or medical society recommendations that may affect commercial efforts;
- the costs of acquiring potential new product candidates or technology; and
- the costs of operating as a public company.

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Risks Related to the Discovery and Development of Product Candidates

We have limited experience in drug discovery and drug development, and we have never had a drug approved.

Prior to the acquisition of our product candidates, we were not involved in and had no control over their preclinical and clinical development. In addition, we are relying upon the parties we have acquired our product candidates from to have conducted such research and development in accordance with the applicable protocol, legal, regulatory and scientific standards, having accurately reported the results of all clinical trials conducted prior to our acquisition of the applicable product candidate, and having correctly collected and interpreted the data from these studies and trials. To the extent any of these has not occurred, our expected development time and costs may be increased, which could adversely affect our prospects for marketing approval of, and receiving any future revenue from, these product candidates.

In the near term, we are dependent on the success of BXCL501 and BXCL701. If we are unable to initiate or complete the clinical development of, obtain marketing approval for or successfully commercialize BXCL501, BXCL701 and our other product candidates, either alone or with a collaborator, or if we experience significant delays in doing so, our business could be substantially harmed.

We currently do not have any products that have received regulatory approval and may never be able to develop marketable product candidates. We are investing a significant portion of our efforts and financial resources in the development of BXCL501, BXCL701 and our other product candidates. Our prospects are substantially dependent on our ability, or that of any future collaborator, to develop, obtain marketing approval for and successfully commercialize product candidates in one or more disease indications.

The success of BXCL501, BXCL701 and our other product candidates will depend on several factors, including the following:

- acceptance of an Investigational New Drug, or IND, for the conduct of clinical trials of product candidates and proposed design of future clinical trials;
- initiation, progress, timing, costs and results of clinical trials of our product candidates and potential product candidates;
- establishment of a safety, tolerability and efficacy profile that is satisfactory to the FDA or any comparable foreign regulatory authority for marketing approval;
- the performance of our future collaborators, if any;
- the extent of any required post-marketing approval commitments to applicable regulatory authorities;
- establishment of supply arrangements with third-party raw materials suppliers and manufacturers;
- establishment of arrangements with third-party manufacturers to obtain finished drug product that is appropriately packaged for sale;
- adequate ongoing availability of raw materials and drug product for clinical development and any commercial sales;
- obtaining and maintaining patent, trade secret protection and regulatory exclusivity, both in the United States and internationally;
- protection of our rights in our intellectual property portfolio;
- successful launch of commercial sales following any marketing approval;

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- a continued acceptable safety profile following any marketing approval;
- commercial acceptance by patients, the medical community and third-party payors; and
- our ability to compete with other therapies.

Many of these factors are beyond our control, including the results of clinical trials, the time required for the FDA or any comparable foreign regulatory authorities to review any regulatory submissions we may make, potential threats to our intellectual property rights and the manufacturing, marketing and sales efforts of any future collaborator. If we are unable to develop, receive marketing approval for and successfully commercialize BXCL501, BXCL701 and our other product candidates, on our own or with any future collaborator, or experience delays as a result of any of these factors or otherwise, our business could be substantially harmed.

The regulatory approval processes of the FDA and comparable foreign authorities are lengthy, time consuming, expensive and inherently unpredictable, and if we are ultimately unable to obtain regulatory approval for our product candidates, our business will be substantially harmed.

The time required to obtain approval by the FDA and comparable foreign authorities is unpredictable but typically takes many years following the commencement of clinical trials and depends upon numerous factors, including the substantial discretion of the regulatory authorities. The results of preclinical studies and early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy traits despite having progressed through preclinical studies and initial clinical trials. It is not uncommon for companies in the biopharmaceutical industry to suffer significant setbacks in advanced clinical trials due to nonclinical findings made while clinical studies were underway and safety or efficacy observations made in clinical studies, including previously unreported adverse events. Our future clinical trial results may not be successful, and notwithstanding any potential promising results in earlier studies, we cannot be certain that we will not face similar setbacks. The historical failure rate for product candidates in our industry is high. In addition, approval policies, regulations, or the type and amount of clinical data necessary to gain approval may change during the course of a product candidate's clinical development and may vary among jurisdictions. We have not obtained regulatory approval for any product candidate and it is possible that none of our existing product candidates or any product candidates we may seek to develop in the future will ever obtain regulatory approval.

Our product candidates could fail to receive regulatory approval for many reasons, including the following:

- the FDA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials;
- we may be unable to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that a product candidate is safe and effective for its proposed indication;
- the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable foreign regulatory authorities for approval;
- the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical studies or clinical trials;
- the data collected from clinical trials of our product candidates may not be sufficient to support the submission of an New Drug Application, or NDA, or other submission or to obtain regulatory approval in the United States or elsewhere; the FDA or comparable foreign regulatory authorities may disagree that our changes to branded reference drugs meet the criteria for the 505(b)(2) regulatory pathway or foreign regulatory pathways;

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- the FDA or comparable foreign regulatory authorities may fail to approve the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and

- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

We have not previously completed a clinical trial of any of our product candidates. Consequently, we may not have the necessary capabilities, including adequate staffing, to successfully manage the execution and completion of any clinical trials we initiate in a way that leads to our obtaining marketing approval for our product candidates in a timely manner, or at all. This lengthy approval process as well as the unpredictability of future clinical trial results may result in our failing to obtain regulatory approval to market our product candidates, which would significantly harm our business, results of operations and prospects.

In addition, even if we were to obtain approval, regulatory authorities may approve any of our product candidates for fewer or more limited indications than we request, may not approve the price we intend to charge for our products, may grant approval contingent on the performance of costly post-marketing clinical trials, may approve a product candidate with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that product candidate or may restrict its distribution. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates.

We have not previously submitted an NDA to the FDA or similar drug approval filings to comparable foreign authorities, for any product candidate, and we cannot be certain that any of our product candidates will be successful in clinical trials or receive regulatory approval. Further, our product candidates may not receive regulatory approval even if they are successful in clinical trials. If we do not receive regulatory approvals for our product candidates, we may not be able to continue our operations. Even if we successfully obtain regulatory approvals to market one or more of our product candidates, our revenues will be dependent, in part, upon the size of the markets in the territories for which we gain regulatory approval and have commercial rights. If the markets for patients that we are targeting for our product candidates are not as significant as we estimate, we may not generate significant revenues from sales of such products, if approved.

We plan to seek regulatory approval to commercialize our product candidates both in the United States and the European Union and in additional foreign countries. While the scope of regulatory approval is similar in other countries, to obtain separate regulatory approval in many other countries we must comply with numerous and varying regulatory requirements of such countries regarding safety and efficacy and governing, among other things, clinical trials and commercial sales, pricing and distribution of our product candidates, and we cannot predict success in these jurisdictions.

We depend on enrollment of patients in our clinical trials in order for us to continue development of our product candidates. If we are unable to enroll patients in our clinical trials, our research and development efforts could be adversely affected.

The timely completion of clinical trials in accordance with their protocols depends, among other things, on our ability to enroll a sufficient number of patients who remain in the study until its conclusion. We may experience difficulties in patient enrollment in our clinical trials for a variety of reasons. Patient enrollment is affected by many factors including the size and nature of the patient population, the proximity of patients to clinical sites, the eligibility criteria for the trial, the design of the clinical trial, the size of the patient population required for analysis of the trial's primary endpoints, the proximity of patients to study sites, our ability to recruit clinical trial investigators with the appropriate competencies and experience, our ability to obtain and maintain patient consents, the risk that patients enrolled in clinical trials will drop out of the trials before completion, and competing

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clinical trials and clinicians' and patients' perceptions as to the potential advantages of the drug being studied in relation to other available therapies, including any new drugs that may be approved for the indications we are investigating. Many pharmaceutical companies are conducting clinical trials in patients with the disease indications that our potential drug products target. As a result, we must compete with them for clinical sites, physicians and the limited number of patients who fulfill the stringent requirements for participation in clinical trials. Also, due to the confidential nature of clinical trials, we do not know how many of the eligible patients may be enrolled in competing studies and who are consequently not available to us for our clinical trials. Our clinical trials may be delayed or terminated due to the inability to enroll enough patients. The delay or inability to meet planned patient enrollment may result in increased costs and delay or termination of our trials, which could have a harmful effect on our ability to develop products.

Delays in clinical testing could result in increased costs to us and delay our ability to generate revenue.

Although we are planning for certain clinical trials relating to BXCL501, BXCL701 and our other product candidates, there can be no assurance that the FDA will accept our proposed trial designs. We may experience delays in our clinical trials and we do not know whether planned clinical trials will begin on time, need to be redesigned, enroll patients on time or be completed on schedule, if at all. Clinical trials can be delayed for a variety of reasons, including delays related to:

- obtaining regulatory approval to commence a trial;
- reaching agreement on acceptable terms with prospective contract research organizations, or CROs, and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- obtaining institutional review board, or IRB, approval at each site;
- recruiting suitable patients to participate in a trial;
- clinical sites deviating from trial protocol or dropping out of a trial;
- addressing patient safety concerns that arise during the course of a trial;
- having patients complete a trial or return for post-treatment follow-up;
- adding a sufficient number of clinical trial sites; or
- manufacturing sufficient quantities of a product candidate for use in clinical trials.

We may also experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent our ability to receive marketing approval or commercialize our product candidates, including:

- we may receive feedback from regulatory authorities that requires us to modify the design of our clinical trials;
- clinical trials of our product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon drug development programs;
- the number of patients required for clinical trials of our product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate or participants may drop out of these clinical trials at a higher rate than we anticipate;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- the cost of clinical trials of our product candidates may be greater than we anticipate;

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- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate;
- regulators may revise the requirements for approving our product candidates, or such requirements may not be as we anticipate; and
- any future collaborators that conduct clinical trials may face any of the above issues, and may conduct clinical trials in ways they view as advantageous to themselves but that are suboptimal for us.

For example, we believe that we will be able to proceed directly to Phase 3 registration trials of BXCL501 if we successfully complete our planned Phase 1b/2 open-label PoC and bridging BA/BE studies. However, the FDA may not agree with our development plans and could require us to perform additional clinical trials or preclinical studies, including additional Phase 1 and/or Phase 2 clinical trials, before permitting us to conduct our planned registration trials.

If we are required to conduct additional clinical trials or other testing of our product candidates beyond those that we currently contemplate, if we are unable to successfully complete clinical trials of our product candidates or other testing, if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we may:

- incur unplanned costs;
- be delayed in obtaining marketing approval for our product candidates or not obtain marketing approval at all;
- obtain marketing approval in some countries and not in others;
- obtain marketing approval for indications or patient populations that are not as broad as intended or desired;
- obtain marketing approval with labeling that includes significant use or distribution restrictions or safety warnings, including boxed warnings;
- be subject to additional post-marketing testing requirements; or
- have the drug removed from the market after obtaining marketing approval.

Furthermore, we intend to rely on CROs and clinical trial sites to ensure the proper and timely conduct of our clinical trials and we intend to have agreements governing their committed activities. They may not perform as required.

We could encounter delays if a clinical trial is suspended or terminated by us, by the IRBs of the institutions in which such trials are being conducted, by the Data Safety Monitoring Board, or DSMB, for such trial or by the FDA or other regulatory authorities. Such authorities may impose such a suspension or termination due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial.

Further, conducting clinical trials in foreign countries, as we may do for our current and future product candidates, presents additional risks that may delay completion of our clinical trials. These risks include the failure of enrolled patients in foreign countries to adhere to clinical protocol as a result of differences in healthcare services or cultural customs, managing additional administrative burdens associated with foreign regulatory schemes, as well as political and economic risks relevant to such foreign countries.

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If we experience delays in the completion of, or termination of, any clinical trial of our product candidates, the commercial prospects of our product candidates will be harmed, and our ability to generate product revenues from any of these product candidates will be delayed. In addition, any delays in completing our clinical trials will increase our costs, slow down our product candidate development and approval process and jeopardize our ability to commence product sales and generate revenues. Any of these occurrences may harm our business, financial condition and prospects significantly. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates.

Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit the commercial profile of an approved label, or result in significant negative consequences following marketing approval, if any.

Undesirable side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or other comparable foreign authorities. The clinical evaluation of BXCL501, BXCL701 and our other product candidates in patients is still in the early stages and it is possible that there may be side effects associated with their use. To date, based on information available in the package insert for Dex, patients treated with Dex have experienced drug-related side effects including hypotension, transient hypertension, bradycardia, dry mouth, acute respiratory distress syndrome, respiratory failure and agitation with hypotension, bradycardia and dry mouth considered serious adverse events. In addition, based on the investigator brochure for Talabostat, patients treated with Talabostat have experienced edema/peripheral swelling, hypotension, dizziness, hypovolemia fatigue, nausea, vomiting, pyrexia rigors and rash with edema and fatigue representing the most frequently observed serious adverse events. Results of our trials could reveal a high and unacceptable severity and prevalence of these or other side effects. In such an event, we, the FDA, the IRBs at the institutions in which our studies are conducted, or the DSMB could suspend or terminate our clinical trials or the FDA or comparable foreign regulatory authorities could order us to cease clinical trials or deny approval of our product candidates for any or all targeted indications. For example, the FDA placed Point Therapeutics, Inc.'s IND for BXCL701 on clinical hold following an increase in observed mortality in patients receiving BXCL701 in a Phase 3 trial in patients with non-small cell lung cancer. Though we believe that this result was caused by, among other things, an imbalance in the disease severity of patients enrolled in the active arm of the clinical trial, there is no guarantee that excess mortality will not be observed in future clinical studies. Treatment-related side effects could also affect patient recruitment or the ability of enrolled patients to complete the clinical trial or result in potential product liability claims. In addition, these side effects may not be appropriately recognized or managed by the treating medical staff. We expect to have to train medical personnel using our product candidates to understand the side effect profiles for our clinical trials and upon any commercialization of any of our product candidates. Inadequate training in recognizing or managing the potential side effects of our product candidates could result in patient injury or death. Any of these occurrences may harm our business, financial condition and prospects significantly.

Additionally, if one or more of our product candidates receives marketing approval, and we or others later identify undesirable side effects caused by such products, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw approvals of such products;
- we may be required to recall a product or change the way such a product is administered to patients;
- additional restrictions may be imposed on the marketing or distribution of the particular product or the manufacturing processes for the product or any component thereof;

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- regulatory authorities may require additional warnings on the label, such as a "black box" warning or contraindication;
- we may be required to implement Risk Evaluation and Mitigation Strategies, or REMS, or create a medication guide outlining the risks of such side effects for distribution to patients;
- we could be sued and held liable for harm caused to patients;
- our product may become less competitive; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of the particular product candidate or for particular indications of a product candidate, if approved, and could significantly harm our business, results of operations and prospects.

BioXcel's approach to the discovery and development of product candidates based on EvolverAI is novel and unproven, and we do not know whether we will be able to develop any products of commercial value.

We are leveraging EvolverAI to create a pipeline of neuroscience and immuno-oncology product candidates for patients whose diseases have not been adequately addressed to date by other approaches and to design and conduct efficient clinical trials with a higher likelihood of success. While we believe that applying EvolverAI to create medicines for defined patient populations may potentially enable drug research and clinical development that is more efficient than conventional drug research and development, our approach is both novel and unproven. Because our approach is both novel and unproven, the cost and time needed to develop our product candidates is difficult to predict, and our efforts may not result in the discovery and development of commercially viable medicines. We may also be incorrect about the effects of our product candidates on the diseases of our defined patient populations, which may limit the utility of our approach or the perception of the utility of our approach. Furthermore, our estimates of our defined patient populations available for study and treatment may be lower than expected, which could adversely affect our ability to conduct clinical trials and may also adversely affect the size of any market for medicines we may successfully commercialize. Our approach may not result in time savings, higher success rates or reduced costs as we expect it to, and if not, we may not attract collaborators or develop new drugs as quickly or cost effectively as expected and therefore we may not be able to commercialize our approach as originally expected.

EvolverAI may fail to help us discover and develop additional potential product candidates.

Any drug discovery that we are conducting using EvolverAI may not be successful in identifying compounds that have commercial value or therapeutic utility. EvolverAI may initially show promise in identifying potential product candidates, yet fail to yield viable product candidates for clinical development or commercialization for a number of reasons, including:

- research programs to identify new product candidates will require substantial technical, financial and human resources, and we may be unsuccessful in our efforts to identify new product candidates. If we are unable to identify suitable additional compounds for preclinical and clinical development, our ability to develop product candidates and obtain product revenues in future periods could be compromised, which could result in significant harm to our financial position and adversely impact our stock price;
- compounds found through EvolverAI may not demonstrate efficacy, safety or tolerability;
- potential product candidates may, on further study, be shown to have harmful side effects or other characteristics that indicate that they are unlikely to receive marketing approval and achieve market acceptance;

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- competitors may develop alternative therapies that render our potential product candidates non-competitive or less attractive; or
- a potential product candidate may not be capable of being produced at an acceptable cost.

An NDA submitted under Section 505(b)(2) subjects us to the risk that we may be subject to a patent infringement lawsuit that would delay or prevent the review or approval of our product candidate.

Our product candidates will be submitted to the FDA for approval under Section 505(b)(2) of the Federal Food, Drug and Cosmetic Act, or FDCA. Section 505(b)(2) permits the submission of an NDA where at least some of the information required for approval comes from studies that were not conducted by, or for, the applicant and on which the applicant has not obtained a right of reference. The 505(b)(2) application would enable us to reference published literature and/or the FDA's previous findings of safety and effectiveness for a branded reference drug with the same active ingredient. For NDAs submitted under Section 505(b)(2) of the FDCA, the patent certification and related provisions of the Drug Price Competition and Patent Term Restoration Act of 1984, also known as the Hatch-Waxman Act, apply. In accordance with the Hatch-Waxman Act, such NDAs may be required to include certifications, known as paragraph IV certifications, that certify that any patents listed in the Patent and Exclusivity Information Addendum of the FDA's publication, Approved Drug Products with Therapeutic Equivalence Evaluations, commonly known as the Orange Book, with respect to any product referenced in the 505(b)(2) application, are invalid, unenforceable or will not be infringed by the manufacture, use or sale of the product that is the subject of the 505(b)(2) NDA.

Under the Hatch-Waxman Act, the holder of patents that the 505(b)(2) application references may file a patent infringement lawsuit after receiving notice of the paragraph IV certification. Filing of a patent infringement lawsuit against the filer of the 505(b)(2) applicant within 45 days of the patent owner's receipt of notice triggers a one-time, automatic, 30-month stay of the FDA's ability to approve the 505(b)(2) NDA, unless patent litigation is resolved in the favor of the paragraph IV filer or the patent expires before that time. Accordingly, we may invest a significant amount of time and expense in the development of one or more product candidates only to be subject to significant delay and patent litigation before such product candidates may be commercialized, if at all. In addition, a 505(b)(2) application will not be approved until any non-patent exclusivity, such as exclusivity for obtaining approval of a new chemical entity, or NCE, listed in the Orange Book for the branded reference drug product has expired. The FDA may also require us to perform one or more additional clinical studies or measurements to support the change from the branded reference drug, which could be time consuming and could substantially delay our achievement of regulatory approvals for such product candidates. The FDA may also reject our future 505(b)(2) submissions and require us to file such submissions under Section 505(b)(1) of the FDCA, which would require us to provide extensive data to establish safety and effectiveness of the drug product for the proposed use and could cause delay and be considerably more expensive and time consuming. These factors, among others, may limit our ability to successfully commercialize our product candidates.

The FDA and other regulatory agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses.

If we are found to have improperly promoted off-label uses of our products or product candidates, if approved, we may become subject to significant liability. Such enforcement has become more common in the industry. The FDA and other regulatory agencies strictly regulate the promotional claims that may be made about prescription drug products, such as our product candidates, if approved. In particular, a product may not be promoted for uses that are not approved by the FDA or such other regulatory agencies as reflected in the product's approved labeling. If we receive marketing approval for our product candidates for our proposed indications, physicians may nevertheless use our products for their patients in a manner that is inconsistent with the approved label, if the physicians personally

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believe in their professional medical judgment it could be used in such manner. However, if we are found to have promoted our products for any off-label uses, the federal government could levy civil, criminal and/or administrative penalties, and seek fines against us. The FDA or other regulatory authorities could also request that we enter into a consent decree or a corporate integrity agreement, or seek a permanent injunction against us under which specified promotional conduct is monitored, changed or curtailed. If we cannot successfully manage the promotion of our product candidates, if approved, we could become subject to significant liability, which would materially adversely affect our business and financial condition.

We may seek Fast Track designation for one or more of our product candidates, but we might not receive such designation, and even if we do, such designation may not actually lead to a faster development or regulatory review or approval process.

If a product is intended for the treatment of a serious condition and nonclinical or clinical data demonstrate the potential to address unmet medical need for this condition, a product sponsor may apply for FDA Fast Track designation. If we seek Fast Track designation for a product candidate, we may not receive it from the FDA. However, even if we receive Fast Track designation, Fast Track designation does not ensure that we will receive marketing approval or that approval will be granted within any particular timeframe. We may not experience a faster development or regulatory review or approval process with Fast Track designation compared to conventional FDA procedures. In addition, the FDA may withdraw Fast Track designation if it believes that the designation is no longer supported by data from our clinical development program. Fast Track designation alone does not guarantee qualification for the FDA's priority review procedures.

Even if our product candidates receive regulatory approval, we will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense, and we may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with our product candidates.

Any regulatory approvals that we receive for our product candidates may be subject to limitations on the approved indicated uses for which the product may be marketed or the conditions of approval, or contain requirements for potentially costly post-market testing and surveillance to monitor the safety and efficacy of the product candidate. The FDA may also require a REMS as a condition of approval of our product candidates, which could include requirements for a medication guide, physician communication plans or additional elements to ensure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. In addition, if the FDA or a comparable foreign regulatory authority approves our product candidates, our product candidates will remain subject to ongoing requirements governing the manufacturing process, labeling, packaging, storage, advertising, distribution, import, export, promotion, recordkeeping and adverse event reporting. These requirements include submissions of safety and other post-marketing information and reports, registration, as well as continued compliance with good clinical practice, or GCP, requirements for any clinical trials that we conduct post-approval. In addition, manufacturers of drug products and their facilities are subject to continual review and periodic inspections by the FDA and other regulatory authorities for compliance with Good Manufacturing Practices, or GMP, regulations. If we or a regulatory agency discovers previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, a regulatory agency may impose restrictions on that product or the manufacturer, including requiring voluntary or mandatory recalls, additional restrictions on manufacturing or withdrawal of the product from the market or suspension of manufacturing. If we, our product candidates or the manufacturing facilities for our product candidates fail to comply with applicable regulatory requirements, a regulatory agency may:

- issue warning letters;

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- impose civil or criminal penalties;
- suspend regulatory approval;
- suspend any ongoing clinical trials;
- refuse to approve pending applications or supplements to applications filed by us;
- impose restrictions on operations, including costly new manufacturing requirements;
- seize or detain products or request us to initiate a product recall; or
- pursue and obtain an injunction.

Any failure by us to comply with existing regulations could harm our reputation and operating results.

We will be subject to extensive regulation by U.S. federal and state and foreign governments in each of the markets where we intend to sell BXCL501 and BXCL701 if and after they are approved. For example, we will have to adhere to all regulatory requirements including the FDA's current GCPs, Good Laboratory Practice, or GLP, and GMP requirements. If we fail to comply with applicable regulations, including FDA pre-or post- approval cGMP requirements, then the FDA or other foreign regulatory authorities could sanction us. Even if a drug is FDA-approved, regulatory authorities may impose significant restrictions on a product's indicated uses or marketing or impose ongoing requirements for potentially costly post-marketing studies.

Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses, divert our management's attention from the operation of our business and damage our reputation. We expend significant resources on compliance efforts and such expenses are unpredictable and might adversely affect our results.

The FDA's and other regulatory authorities' policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. For example, in December 2016, the 21st Century Cures Act, or Cures Act, was signed into law. The Cures Act, among other things, is intended to modernize the regulation of drugs and spur innovation, but its ultimate implementation is unclear. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability, which would adversely affect our business, prospects, financial condition and results of operations.

In addition, we cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or executive action, either in the United States or abroad. For example, certain policies of the Trump administration may impact our business and industry. Namely, the Trump administration has taken several executive actions, including the issuance of a number of Executive Orders, that could impose significant burdens on, or otherwise materially delay, FDA's ability to engage in routine oversight activities such as implementing statutes through rulemaking, issuance of guidance, and review and approval of marketing applications. Notably, on January 30, 2017, President Trump issued an Executive Order directing all executive agencies, including the FDA, that, for each notice of proposed rulemaking or final regulation to be issued in fiscal year 2017, the agency shall identify at least two existing regulations to be repealed, unless prohibited by law. These requirements are referred to as the "two-for-one" provisions. This Executive Order includes a budget neutrality provision that requires the total incremental cost of all new regulations in the 2017 fiscal year, including repealed regulations, to be no greater than zero, except in limited circumstances. For fiscal years 2018 and beyond, the Executive Order requires agencies to identify regulations to offset any incremental cost of a new regulation. In interim guidance issued by the Office of Information and Regulatory Affairs within OMB on February 2, 2017, the administration indicates that the

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"two-for-one" provisions may apply not only to agency regulations, but also to significant agency guidance documents., and on September 8, 2017, the FDA published notices in the Federal Register soliciting broad public comment to identify regulations that could be modified in compliance with these Executive Orders. It is difficult to predict how these requirements will be implemented, and the extent to which they will impact the FDA's ability to exercise its regulatory authority. If these executive actions impose restrictions on FDA's ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted. In addition, if we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability.

We may be subject to extensive regulations outside the United States and may not obtain marketing approvals for products in Europe and other jurisdictions.

In addition to regulations in the United States, should we or our collaborators pursue marketing approvals for BXCL501, BXCL701 and our other product candidates internationally, we and our collaborators will be subject to a variety of regulations in other jurisdictions governing, among other things, clinical trials and any commercial sales and distribution of our products. Whether or not we, or our collaborators, obtain FDA approval for a product, we must obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical trials or marketing of the product in those countries. The requirements and process governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country.

We expect to pursue marketing approvals for BXCL501, BXCL701 and our other product candidates in Europe and other jurisdictions outside the United States with collaborative partners. The time and process required to obtain regulatory approvals and reimbursement in Europe and other jurisdictions may be different from those in the United States regulatory and approval in one jurisdiction does not ensure approvals in any other jurisdiction; however, negative regulatory decisions in any jurisdiction may have a negative impact on the regulatory process in other jurisdictions.

Additionally, on June 23, 2016, the electorate in the United Kingdom voted in favor of leaving the European Union, commonly referred to as Brexit. On March 29, 2017, the country formally notified the European Union of its intention to withdraw pursuant to Article 50 of the Lisbon Treaty. Since a significant proportion of the regulatory framework in the United Kingdom is derived from European Union directives and regulations, the referendum could materially impact the regulatory regime with respect to the approval of our product candidates in the United Kingdom or the European Union. Any delay in obtaining, or an inability to obtain, any marketing approvals, as a result of Brexit or otherwise, would prevent us from commercializing our product candidates in the United Kingdom and/or the European Union and restrict our ability to generate revenue and achieve and sustain profitability. If any of these outcomes occur, we may be forced to restrict or delay efforts to seek regulatory approval in the United Kingdom and/or European Union for our product candidates, which could significantly and materially harm our business.

If we are found in violation of federal or state "fraud and abuse" laws, we may be required to pay a penalty and/or be suspended from participation in federal or state health care programs, which may adversely affect our business, financial condition and results of operations.

In the United States, we will be subject to various federal and state health care "fraud and abuse" laws, including anti-kickback laws, false claims laws and other laws intended to reduce fraud and abuse in federal and state health care programs, which could affect us, particularly upon successful commercialization of our products in the United States. The federal Anti-Kickback Statute makes it illegal for any person, including a prescription drug manufacturer (or a party acting on its behalf), to knowingly and willfully solicit, receive, offer or pay any remuneration that is intended to induce the

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referral of business, including the purchase, order or prescription of a particular drug for which payment may be made under a federal health care program, such as Medicare or Medicaid. Under federal government regulations, some arrangements, known as safe harbors, are deemed not to violate the federal Anti-Kickback Statute. Although we seek to structure our business arrangements in compliance with all applicable requirements, these laws are broadly written, and it is often difficult to determine precisely how the law will be applied in specific circumstances. Accordingly, it is possible that our practices may be challenged under the federal Anti-Kickback Statute. False claims laws prohibit anyone from knowingly and willfully presenting or causing to be presented for payment to third-party payers, including government payers, claims for reimbursed drugs or services that are false or fraudulent, claims for items or services that were not provided as claimed, or claims for medically unnecessary items or services. Cases have been brought under false claims laws alleging that off-label promotion of pharmaceutical products or the provision of kickbacks has resulted in the submission of false claims to governmental health care programs. Under the Health Insurance Portability and Accountability Act of 1996, we are prohibited from knowingly and willfully executing a scheme to defraud any health care benefit program, including private payers, or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for health care benefits, items or services. Violations of fraud and abuse laws may be punishable by criminal and/or civil sanctions, including fines and/or exclusion or suspension from federal and state health care programs such as Medicare and Medicaid and debarment from contracting with the U.S. government. In addition, private individuals have the ability to bring actions on behalf of the government under the federal False Claims Act as well as under the false claims laws of several states.

Many states have adopted laws similar to the federal anti-kickback statute, some of which apply to the referral of patients for health care services reimbursed by any source, not just governmental payers. Neither the government nor the courts have provided definitive guidance on the application of fraud and abuse laws to our business. Law enforcement authorities are increasingly focused on enforcing these laws, and if we are found in violation of one of these laws, we could be required to pay a penalty and could be suspended or excluded from participation in federal or state health care programs, and our business, results of operations and financial condition may be adversely affected.

We may be unable to maintain sufficient clinical trial liability insurance.

Our inability to obtain and retain sufficient clinical trial liability insurance at an acceptable cost to protect against potential liability claims could prevent or inhibit our ability to conduct clinical trials for product candidates we develop. We are currently a 93% owned subsidiary of BioXcel and until the closing of this offering, we will be operated as a majority-owned subsidiary of BioXcel, and we are covered under BioXcel's insurance policies. We currently do not have clinical trial liability insurance and would need to secure coverage before commencing patient enrollment for our clinical trials in the United States, which we currently expect to occur in 2018. Any claim that may be brought against us could result in a court judgment or settlement in an amount that is not covered, in whole or in part, by our insurance or that is in excess of the limits of our insurance coverage. We expect we will supplement our clinical trial coverage with product liability coverage in connection with the commercial launch of BXCL501, BXCL701 or other product candidates we develop in the future; however, we may be unable to obtain such increased coverage on acceptable terms or at all. If we are found liable in a clinical trial lawsuit or a product liability lawsuit in the future, we will have to pay any amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be able to obtain, sufficient capital to pay such amounts.

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Risks Related to Commercialization of Our Product Candidates

If our products do not gain market acceptance, our business will suffer because we might not be able to fund future operations.

A number of factors may affect the market acceptance of our products or any other products we develop or acquire, including, among others:

- the price of our products relative to other products for the same or similar treatments;
- the perception by patients, physicians and other members of the health care community of the effectiveness and safety of our products for their indicated applications and treatments;
- our ability to fund our sales and marketing efforts; and
- the effectiveness of our sales and marketing efforts.

If our products do not gain market acceptance, we may not be able to fund future operations, including developing, testing and obtaining regulatory approval for new product candidates and expanding our sales and marketing efforts for our approved products, which would cause our business to suffer.

If the FDA does not conclude that our product candidates satisfy the requirements for the 505(b)(2) regulatory approval pathway, or if the requirements for approval of any of our product candidates under Section 505(b)(2) are not as we expect, the approval pathway for our product candidates will likely take significantly longer, cost significantly more and encounter significantly greater complications and risks than anticipated, and in any case may not be successful.

We intend to seek FDA approval through the 505(b)(2) regulatory pathway for certain of our product candidates, including BXCL501. The Hatch-Waxman Act, added Section 505(b)(2) to the FDCA. Section 505(b)(2) permits the filing of an NDA where at least some of the information required for approval comes from studies that were not conducted by or for the applicant. If the FDA does not allow us to pursue the 505(b)(2) regulatory pathway for our product candidates as anticipated, we may need to conduct additional clinical trials, provide additional data and information and meet additional standards for regulatory approval. If this were to occur, the time and financial resources required to obtain FDA approval for our product candidates would likely substantially increase. Moreover, the inability to pursue the 505(b)(2) regulatory pathway could result in new competitive products reaching the market faster than our product candidates, which could materially adversely impact our competitive position and prospects. Even if we are allowed to pursue the 505(b)(2) regulatory pathway for a product candidate, we cannot assure you that we will receive the requisite or timely approvals for commercialization of such product candidate. In addition, we expect that our competitors will file citizens' petitions with the FDA in an attempt to persuade the FDA that our product candidates, or the clinical studies that support their approval, contain deficiencies. Such actions by our competitors could delay or even prevent the FDA from approving any NDA that we submit under Section 505(b)(2).

We expect to rely heavily on orphan drug status to commercialize some of our product candidates, if approved, but any orphan drug designations we recieve may not confer marketing exclusivity or other expected commercial benefits.

We expect to rely heavily on orphan drug exclusivity for our product candidates. In the United States, orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages and user-fee waivers. In addition, if a product that has orphan drug designation subsequently receives the first FDA approval for the disease for which it has such designation, the product is entitled to orphan drug exclusivity. Orphan drug exclusivity in the

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United States provides that the FDA may not approve any other applications, including a full NDA, to market the same drug for the same indication for seven years, except in limited circumstances the applicable exclusivity period is ten years in Europe. The European exclusivity period can be reduced to six years if a drug no longer meets the criteria for orphan drug designation or if the drug is sufficiently profitable so that market exclusivity is no longer justified. Although we have received orphan designation for BXCL701 for the treatment of pancreatic cancer, BXCL701 has not been granted orphan designation as of the date of this prospectus for the treatment of NEPC.

Even if we, or any future collaborators, obtain orphan drug designation for a product candidate, we, or they, may not be able to obtain or maintain orphan drug exclusivity for that product candidate. We may not be the first to obtain marketing approval of any product candidate for which we have obtained orphan drug designation for the orphan-designated indication due to the uncertainties associated with developing pharmaceutical products, and it is possible that another company also holding orphan drug designation for the same product candidate will receive marketing approval for the same indication before we do. If that were to happen, our applications for that indication may not be approved until the competing company's period of exclusivity expires. In addition, exclusive marketing rights in the United States may be limited if we seek approval for an indication broader than the orphan-designated indication or may be lost if the FDA later determines that the request for designation was materially defective or if we are unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition. Further, even if we, or any future collaborators, obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs with different active moieties may be approved for the same condition. Even after an orphan drug is approved, the FDA can subsequently approve the same drug with the same active moiety for the same condition if the FDA concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care or the manufacturer of the product with orphan exclusivity is unable to maintain sufficient product quantity. Orphan drug designation neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process, nor does it prevent competitors from obtaining approval of the same product candidate as ours for indications other than those in which we have been granted orphan drug designation.

We may seek a breakthrough therapy designation for BXCL701 or one or more of our other product candidates, we might not receive such designation, and even if we do, such designation may not lead to a faster development or regulatory review or approval process.

We may seek a breakthrough therapy designation for BXCL701 or one or more of our other product candidates. A breakthrough therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For drugs and biologics that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Drugs designated as breakthrough therapies by the FDA may also be eligible for priority review if supported by clinical data at the time the NDA is submitted to the FDA.

Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe that one of our product candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. Even if we receive breakthrough therapy designation, the receipt of such designation for a product candidate may not result in a faster development or regulatory review or approval process compared to drugs considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA.

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In addition, even if one or more of our product candidates qualify as breakthrough therapies, the FDA may later decide that the product candidates no longer meet the conditions for qualification or decide that the time period for FDA review or approval will not be shortened.

We may seek priority review designation for BXCL701 or one or more of our other product candidates, but we might not receive such designation, and even if we do, such designation may not lead to a faster development or regulatory review or approval process.

If the FDA determines that a product candidate offers a treatment for a serious condition and, if approved, the product would provide a significant improvement in safety or effectiveness, the FDA may designate the product candidate for priority review. A priority review designation means that the goal for the FDA to review an application is six months, rather than the standard review period of ten months. We may request priority review for our product candidates. The FDA has broad discretion with respect to whether or not to grant priority review status to a product candidate, so even if we believe a particular product candidate is eligible for such designation or status, the FDA may decide not to grant it. Moreover, a priority review designation does not necessarily mean a faster development or regulatory review or approval process or necessarily confer any advantage with respect to approval compared to conventional FDA procedures. Receiving priority review from the FDA does not guarantee approval within the six-month review cycle or at all.

If we are unable to develop satisfactory sales and marketing capabilities, we may not succeed in commercializing BXCL501, BXCL701 or any other product candidate.

We have no experience in marketing and selling drug products. We have not entered into arrangements for the sale and marketing of BXCL501, BXCL701 or any other product candidate. Typically, pharmaceutical companies would employ groups of sales representatives and associated sales and marketing staff numbering in the hundreds to thousands of individuals to call on this large number of physicians and hospitals. We may seek to collaborate with a third party to market our drugs or may seek to market and sell our drugs by ourselves. If we seek to collaborate with a third party, we cannot be sure that a collaborative agreement can be reached on terms acceptable to us. If we seek to market and sell our drugs directly, we will need to hire additional personnel skilled in marketing and sales. We cannot be sure that we will be able to acquire, or establish third party relationships to provide, any or all of these marketing and sales capabilities. The establishment of a direct sales force or a contract sales force or a combination direct and contract sales force to market our products will be expensive and time-consuming and could delay any product launch. Further, we can give no assurances that we may be able to maintain a direct and/or contract sales force for any period of time or that our sales efforts will be sufficient to grow our revenues or that our sales efforts will ever lead to profits.

We operate in a highly competitive and rapidly changing industry.

Biopharmaceutical product development is highly competitive and subject to rapid and significant technological advancements. Our success is highly dependent upon our ability to in-license, acquire, develop and obtain regulatory approval for new and innovative products on a cost-effective basis and to market them successfully. In doing so, we face and will continue to face intense competition from a variety of businesses, including large, fully integrated, well-established pharmaceutical companies who already possess a large share of the market, specialty pharmaceutical and biopharmaceutical companies, academic institutions, government agencies and other private and public research institutions in the United States, the European Union and other jurisdictions.

Many of the companies against which we are competing or against which we may compete in the future have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved drugs than we do. These third parties compete with us in recruiting and retaining

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qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Mergers and acquisitions in the biopharmaceutical industry could result in even more resources being concentrated among a small number of our competitors.

Competition may further increase as a result of advances in the commercial applicability of technologies and greater availability of capital for investment in these industries. Our competitors may succeed in developing, acquiring or licensing, on an exclusive basis, products that are more effective or less costly than any product candidate that we may develop.

Established biopharmaceutical companies may invest heavily to accelerate discovery and development of novel compounds or to in-license novel compounds that could make our product candidates less competitive. In addition, any new product that competes with an approved product must demonstrate compelling advantages in efficacy, convenience, tolerability and safety in order to overcome price competition and to be commercially successful. Accordingly, our competitors may succeed in obtaining patent protection, discovering, developing, receiving FDA approval for or commercializing drugs before we do, which would have an adverse impact on our business and results of operations.

The availability of our competitors' products could limit the demand and the price we are able to charge for any product candidate we commercialize, if any. The inability to compete with existing or subsequently introduced drugs would harm our business, financial condition and results of operations.

Even if we obtain regulatory approvals to commercialize BXCL501, BXCL701 or our other product candidates, our product candidates may not be accepted by physicians or the medical community in general.

There can be no assurance that BXCL501, BXCL701 and our other product candidates or any other product candidate successfully developed by us, independently or with partners, will be accepted by physicians, hospitals and other health care facilities. BXCL501, BXCL701 and any future product candidates we develop will compete with a number of products manufactured and marketed by major pharmaceutical and biotech companies. The degree of market acceptance of any drugs we develop depends on a number of factors, including:

- our demonstration of the clinical efficacy and safety of BXCL501, BXCL701 and our other product candidates;
- timing of market approval and commercial launch of BXCL501, BXCL701 and our other product candidates;
- the clinical indication(s) for which BXCL501, BXCL701 and our other product candidates are approved;
- product label and package insert requirements;
- advantages and disadvantages of our product candidates compared to existing therapies;
- continued interest in and growth of the market for anti-cancer or anti-agitation drugs;
- strength of sales, marketing, and distribution support;
- product pricing in absolute terms and relative to alternative treatments;
- future changes in health care laws, regulations, and medical policies; and
- availability of reimbursement codes and coverage in select jurisdictions, and future changes to reimbursement policies of government and third-party payors.

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Significant uncertainty exists as to the coverage and reimbursement status of any product candidate for which we obtain regulatory approval. In the United States and markets in other countries, sales of any products for which we receive regulatory approval for commercial sale will depend in part on the availability of reimbursement from third-party payors. Third-party payors include government health administrative authorities, managed care providers, private health insurers and other organizations.

Healthcare reform measures could hinder or prevent our product candidates' commercial success.

The U.S. government and other governments have shown significant interest in pursuing healthcare reform. Any government-adopted reform measures could adversely impact the pricing of healthcare products and services in the United States or internationally and the amount of reimbursement available from governmental agencies or other third-party payors. The continuing efforts of the U.S. and foreign governments, insurance companies, managed care organizations and other payors of health care services to contain or reduce health care costs may adversely affect our ability to set prices for our products which we believe are fair, and our ability to generate revenues and achieve and maintain profitability.

New laws, regulations and judicial decisions, or new interpretations of existing laws, regulations and decisions, that relate to healthcare availability, methods of delivery or payment for products and services, or sales, marketing or pricing, may limit our potential revenue, and we may need to revise our research and development programs. The pricing and reimbursement environment may change in the future and become more challenging due to several reasons, including policies advanced by the current executive administration in the United States, new healthcare legislation or fiscal challenges faced by government health administration authorities. Specifically, in both the United States and some foreign jurisdictions, there have been a number of legislative and regulatory proposals to change the health care system in ways that could affect our ability to sell our products profitably.

For example, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or the PPACA has substantially changed the way healthcare is financed by both government health plans and private insurers, and significantly impacts the pharmaceutical industry. The PPACA contains a number of provisions that are expected to impact our business and operations in ways that may negatively affect our potential revenues in the future. For example, the PPACA imposes a non-deductible excise tax on pharmaceutical manufacturers or importers that sell branded prescription drugs to government programs which we believe will increase the cost of our products. In addition, as part of the PPACA's provisions closing a funding gap that currently exists in the Medicare Part D prescription drug program, we will be required to provide a discount on branded prescription drugs equal to 50% of the government-negotiated price, for drugs provided to certain beneficiaries who fall within the donut hole. Similarly, PPACA increases the level of Medicaid rebates payable by manufacturers of brand-name drugs from 15.1% to 23.1% and requires collection of rebates for drugs paid by Medicaid managed care organizations. The PPACA also includes significant changes to the 340B drug discount program including expansion of the list of eligible covered entities that may purchase drugs under the program. At the same time, the expansion in eligibility for health insurance benefits created under PPACA is expected to increase the number of patients with insurance coverage who may receive our products. While it is too early to predict all the specific effects the PPACA or any future healthcare reform legislation will have on our business, they could have a material adverse effect on our business and financial condition.

Congress periodically adopts legislation like the PPACA and the Medicare Prescription Drug, Improvement and Modernization Act of 2003, that modifies Medicare reimbursement and coverage policies pertaining to prescription drugs. Implementation of these laws is subject to ongoing revision through regulatory and sub regulatory policies. Congress also may consider additional changes to Medicare policies, potentially including Medicare prescription drug policies, as part of ongoing budget negotiations. While the scope of any such legislation is uncertain at this time, there can be no

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assurances that future legislation or regulations will not decrease the coverage and price that we may receive for our proposed products. Other third-party payors are increasingly challenging the prices charged for medical products and services. It will be time consuming and expensive for us to go through the process of seeking coverage and reimbursement from Medicare and private payors. Our proposed products may not be considered cost-effective, and coverage and reimbursement may not be available or sufficient to allow us to sell our proposed products on a profitable basis. Further federal and state proposals and health care reforms are likely which could limit the prices that can be charged for the product candidates that we develop and may further limit our commercial opportunities. Our results of operations could be materially adversely affected by proposed healthcare reforms, by the Medicare prescription drug coverage legislation, by the possible effect of such current or future legislation on amounts that private insurers will pay and by other health care reforms that may be enacted or adopted in the future.

In September 2007, the Food and Drug Administration Amendments Act of 2007 was enacted, giving the FDA enhanced post-marketing authority, including the authority to require post-marketing studies and clinical trials, labeling changes based on new safety information, and compliance with risk evaluations and mitigation strategies approved by the FDA. The FDA's exercise of this authority could result in delays or increased costs during product development, clinical trials and regulatory review, increased costs to assure compliance with post-approval regulatory requirements, and potential restrictions on the sale and/or distribution of approved products.

Risks Related to Our Relationship with BioXcel

BioXcel controls the direction of our business, and the concentrated ownership of our common stock will prevent you and other stockholders from influencing significant decisions.

After this offering, BioXcel will own approximately 60.6% of the economic interest and voting power of our outstanding common stock, or approximately 57.6% of the economic interest and voting power of our outstanding common stock if the underwriters exercise their option to purchase additional shares in full. As long as BioXcel beneficially controls a majority of the voting power of our outstanding common stock, it will generally be able to determine the outcome of all corporate actions requiring stockholder approval, including the election and removal of directors. Even if BioXcel were to control less than a majority of the voting power of our outstanding common stock, it may influence the outcome of such corporate actions so long as it owns a significant portion of our common stock. If BioXcel continues to hold its shares of our common stock, it could remain our controlling stockholder for an extended period of time or indefinitely.

Approval of commercial terms between us and BioXcel does not preclude the possibility of stockholder litigation, including but not limited to derivative litigation nominally against BioXcel and against its directors and officers and also against us and our directors and officers.

The commercial terms of the Services Agreement, the grid note, dated June 30, 2017, or Grid Note, and the Contribution Agreement that we have entered into with BioXcel have been not been negotiated on behalf of BioXcel by persons consisting solely of disinterested BioXcel directors. Notwithstanding the foregoing, we have no basis for believing that the terms of these agreements will not be in the best interests of both BioXcel and its stockholders and also us and our stockholders. Nonetheless, no assurance can be given that any stockholder of BioXcel will not claim in a lawsuit that such terms in fact are not in the best interests of BioXcel and its stockholders, that the directors and officers of BioXcel breached their fiduciary duties in connection with such agreements and that any disclosures by BioXcel to its stockholders regarding these agreements and the relationship between BioXcel and us did not satisfy applicable requirements. In any such instance, we and our directors and officers may also be named as defendants and we would have to defend ourselves and our directors and officers. While we will seek indemnification from BioXcel under the terms of these agreements

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against any damages or other costs, which could be substantial, no such indemnification has yet been agreed to or may be agreed to and be in effect. Further, any such litigation would be time-consuming and would divert focus and resources from the development of our product candidates and our business, including but not limited to possibly delaying our clinical trials due to our management having to spend time and attention on such litigation.

The Distribution may not occur and your investment in our securities may be adversely affected if BioXcel does not distribute the shares of our common stock owned by BioXcel.

BioXcel has advised us that, following the completion of this offering and subject to the expiration of any applicable lock-up periods or other agreements we have or may have with BioXcel, it does not have any near-term plans to distribute the shares of BTI common stock held by BioXcel to the BioXcel stockholders. It is expected that any potential distribution will be taxable to BioXcel and its stockholders. Whether a Distribution is conducted in the future will depend on many factors, including BioXcel's cash position, market capitalization, BioXcel's investment opportunities, taxation to BioXcel and BioXcel's stockholders and the our status and prospects. In addition, the liquidity of the market for our common stock may be constrained for as long as BioXcel continues to hold a significant position in our common stock. Additionally, without a Distribution, there will be limited liquidity in the market for our common stock, which will impact our stockholders and our stock price. A lack of liquidity in the market for our common stock may adversely affect our stock price and therefore, our ability to raise additional funds in the public markets, which may have a material adverse effect on our ability to grow our business.

Following this offering, we will continue to depend on BioXcel to provide us with certain services for our business.

We have operated as a 93% owned subsidiary of BioXcel. Certain administrative services required by us for the operation of our business are currently provided by BioXcel, including services related to insurance and risk management, accounting and human resources. Under the Services Agreement, BioXcel will continue to provide us with various services following the closing of the offering until we are able to build our own capabilities in the transition areas. We believe it is most efficient for BioXcel to provide these services for us to facilitate the efficient operation of our business as we transition to becoming an independent, public company. At our election, or if BioXcel does not or is unable to perform its obligations under the Services Agreement, we will be required to provide these services ourselves or to obtain substitute arrangements with other third parties. We may be unable to provide these services because of financial or other constraints or be unable to implement substitute arrangements on a timely basis on terms that are favorable to us, or at all.

We exercise no control over the activities of BioXcel other than the contractual rights we have pursuant to our Services Agreement and Contribution Agreement. Because of our historical relationship with our parent, our reputation is also tied to BioXcel. We may be subject to reputational harm, or our relationships with existing and potential clients, third-party research organizations, consultants and other business partners could be harmed if BioXcel or any of its affiliates, previously, or in the future, among other things, engages in poor business practices, restructures or files for bankruptcy, becomes subject to litigation or otherwise damages its reputation or business prospects. Any of these events might in turn adversely affect our reputation, revenues and/or business prospects, and may also adversely affect our access to EvolverAI and BioXcel's collaborative services.

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We also rely, in part, on BioXcel and access to EvolverAI, a research and development engine created and owned by BioXcel, to identify, research and develop potential product candidates in neuroscience and immuno-oncology. We have the option to enter into a collaborative services agreement with BioXcel, pursuant to which BioXcel shall perform product identification and related services for us utilizing EvolverAI. We have agreed that such agreement will be negotiated in good faith and that such agreement will incorporate reasonable market based terms, including royalty payments on net sales and reasonable development and commercialization milestone payments. In addition, BioXcel has granted us, upon completion of this offering, a first right to negotiate exclusive rights to any additional product candidates in the fields of neuroscience and immuno-oncology that BioXcel may identify on its own and not in connection with BioXcel's provision of services to us under the Services Agreement. This option for first negotiation shall be valid for a period of five years from the date of this offering. If our rights and access to BioXcel's collaborative services and to EvolverAI were to become limited, terminated, or if we were otherwise precluded from conducting research and development using EvolverAI, or if BioXcel is unable to fulfill its obligations under the agreements, such development could materially adversely affect our future operating results, financial condition and prospects. Furthermore, certain individuals conducting services on our behalf are not our employees, and except for remedies available to us under our agreements with BioXcel, we cannot control whether or not they devote sufficient time, skill and resources to our ongoing development programs. We also cannot ensure that BioXcel retains sufficient resources of personnel or otherwise to conduct its operations. BioXcel may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting research and development activities, which could impede their ability to devote appropriate time to our research and development programs. In addition, if we fail to comply with our diligence, payment or other obligations under the agreements, any such collaboration may terminate or we may not be able to successfully negotiate agreements for future product candidates or collaborations with BioXcel.

The ownership by our executive officers and our directors of shares of BioXcel common stock and rights to purchase BioXcel common stock may create, or may create the appearance of, conflicts of interest.

The ownership by our executive officers and our directors of shares of BioXcel common stock, options to purchase shares of BioXcel common stock, or other equity awards of BioXcel may create, or may create the appearance of, conflicts of interest. Our Chief Executive Officer and Vice President—Finance will continue to serve in the same respective roles at BioXcel until the consummation of this offering. Three of our four directors currently serve on both our board of directors and the board of directors of BioXcel. Upon completion of this offering, Sandeep Laumas, M.D. has agreed to step down from BioXcel's board of directors and plans to continue his service on our board of directors. Because of the current (and former, upon the closing) positions of our executive officers and our directors with BioXcel, they own shares of BioXcel common stock, options to purchase shares of BioXcel common stock or other equity awards of BioXcel. Our Chief Executive Officer, Vimal Mehta, Ph.D. and one of our directors, Krishnan Nandabalan, Ph.D., each own approximately 43% and 43%, respectively, of outstanding BioXcel voting stock. Ownership by our executive officers and directors of common stock or options to purchase common stock of BioXcel, or any other equity awards, whether prior to, or following the consummation of this offering, creates, or, may create the appearance of, conflicts of interest when these individuals are faced with decisions that could have different implications for BioXcel than the decisions have for us, including decisions that relate to our Services Agreement, Contribution Agreement, as well as potential agreements relating to future product candidates and AI-related services or collaborations. In connection with the Separation, our chief executive officer has agreed to recuse himself with respect to voting on any matter coming before either BioXcel's or our board of directors related to our relationship with BioXcel, although he will still be permitted to participate in discussions and negotiations. Any perceived conflicts of interest resulting

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from investors questioning the independence of our management or the integrity of corporate governance procedures may materially affect our stock price.

Any disputes that arise between us and BioXcel with respect to our past and ongoing relationships could harm our business operations.

Disputes may arise between BioXcel and us in a number of areas relating to our past and ongoing relationships, including:

- intellectual property, technology and business matters, including failure to make required technology transfers and failure to comply with non-compete provisions applicable to BioXcel and us;
- labor, tax, employee benefit, indemnification and other matters arising from the Separation;
- distribution and supply obligations;
- employee retention and recruiting;
- business combinations involving us;
- sales or distributions by BioXcel of all or any portion of its ownership interest in us;
- the nature, quality and pricing of services BioXcel has agreed to provide us; and
- business opportunities that may be attractive to both BioXcel and us.

We have entered into the Services Agreement with BioXcel related to the separation of our business operations from those of BioXcel that contains certain limitations on BioXcel's ability to control various aspects of our business and operations, notwithstanding BioXcel's substantial ownership position following the offering. This agreement may be amended upon agreement between us and BioXcel.

We and our stockholders may not achieve some or all of the expected benefits of the Separation.

Drug development is an expensive and time-consuming process, but we believe the knowledge we have gained while operating as a subsidiary of BioXcel has helped expedite this process. However, in order to realize the value proposition of BTI as a drug development company, we intend to target early stage healthcare and pharmaceutical focused investors, who are interested in investing in drug development companies and who appreciate the risks, rewards and typically longer investment timelines associated with such investments. In order to successfully attract this type of new investment, we believe it is critical that we separate from BioXcel, because we believe that doing so will provide us with some or all of the following benefits:

- improving strategic and operational flexibility, increasing management focus and streamlining decision-making by providing the flexibility to implement our strategic plan and to respond more effectively to different customer needs and the changing economic environment;
- allowing us to adopt the capital structure, investment policy and dividend policy best suited to our financial profile and business needs, without competing for capital with BioXcel's other businesses;
- creating an independent equity structure that will facilitate our ability to affect future acquisitions utilizing our common stock; and
- facilitating incentive compensation arrangements for employees more directly tied to the performance of our business, and enhancing employee hiring and retention by, among other

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things, improving the alignment of management and employee incentives with performance and growth objectives of our business.

If we are not successful implementing the Separation, we may not be able to achieve the full strategic and financial benefits we expect to receive, or the benefits may be delayed or not occur at all. Even if we are able to achieve stand-alone, independent status as a drug development company, there can be no assurance that investors and analysts will place a greater value on us as a stand-alone drug development company than as a wholly- or substantially-owned subsidiary of BioXcel.

We may be a "controlled company" within the meaning of the Nasdaq rules and, as a result, may qualify for, and may rely on, exemptions from certain corporate governance requirements that provide protection to stockholders of other companies.

Upon completion of this offering, BioXcel will continue to control a majority of the voting power of our outstanding common stock. As a result, we will be a "controlled company" within the meaning of the corporate governance standards of the Nasdaq rules. Under these rules, a listed company of which more than 50% of the voting power is held by an individual, group or another company is a "controlled company" and may elect not to comply with certain corporate governance requirements.

As a controlled company, we will rely on certain exemptions from the Nasdaq standards that may enable us not to comply with certain Nasdaq corporate governance requirements if BioXcel continues to control a majority of the voting power of our outstanding common stock. Accordingly, you may not have the same protections afforded to stockholders of companies that are subject to all of the corporate governance requirements of The Nasdaq Capital Market.

The assets and resources that we acquire from BioXcel in the Separation may not be sufficient for us to operate as a stand-alone company, and we may experience difficulty in separating our assets and resources from BioXcel.

Because we have not operated as a stand-alone company in the past, we may have difficulty doing so. We may need to acquire assets and resources in addition to those provided by BioXcel to us, and in connection with the Separation, may also face difficulty in separating our resources from BioXcel's and integrating newly acquired assets into our business. For example, we may need to hire additional personnel to assist with administrative and technical functions, and acquire other office and laboratory equipment for use in the ordinary course operations of our business. If we have difficulty operating as a stand-alone company, fail to acquire assets that we need to run our operations, or incur unexpected costs in separating our business from BioXcel's business or in integrating newly acquired assets into our business, our financial condition and results of operations will be adversely affected.

You may have difficulty evaluating our business because we have no history as a separate company and our historical financial information may not be representative of our results as a separate company.

The historical financial information included in this prospectus does not necessarily reflect the financial condition, results of operations or cash flows that we would have achieved as a separate company during the periods presented or those that we will achieve in the future. Prior to the contribution of our assets from BioXcel, our research and development activities were conducted by BioXcel as part of its broader operations, rather than as an independent division or subsidiary. BioXcel also performed various corporate functions relating to our business. Our historical financial information reflects allocations of corporate expenses from BioXcel for these and similar functions. We believe that these allocations are comparable to the expenses we would have incurred had we operated as a separate company, although we may incur higher expenses as a separate company.

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BioXcel may experience challenges with the acquisition, development, enhancement or deployment of technology necessary for EvolverAI.

BioXcel operates in businesses that require sophisticated computer systems and software for data collection, data processing, cloud-based platforms, analytics, statistical projections and forecasting, mobile computing, social media analytics and other applications and technologies. BioXcel seeks to address its technology risks by increasing its reliance on the use of innovations by cross-industry technology leaders and adapt these for their pharmaceutical, specialty-pharma, biotech, biopharmaceutical, diagnostic, medical device and contract research and manufacturing clients. Some of the technologies supporting the industries they serve are changing rapidly and we must continue to adapt to these changes in a timely and effective manner at an acceptable cost. They also must continue to deliver data to its clients in forms that are easy to use while simultaneously providing clear answers to complex questions. There can be no guarantee that we or BioXcel will be able to develop, acquire or integrate new technologies, that these new technologies will meet our and BioXcel's needs or achieve our expected goals, or that we will be able to do so as quickly or cost-effectively as our competitors. Significant technological change could render EvolverAI obsolete. BioXcel's continued success will depend on its ability to adapt to changing technologies, manage and process ever-increasing amounts of data and information and improve the performance, features and reliability of its services in response to changing client and industry demands. BioXcel may experience difficulties that could delay or prevent the successful design, development, testing, and introduction of advanced versions of EvolverAI, limiting our ability to identify new product candidates. New services, or enhancements to existing EvolverAI services, may not adequately meet our requirements. Any of these failures could have a material adverse effect on our operating results and financial condition.

Risks Related to Our Reliance on Third Parties

We are substantially dependent on third parties for the manufacture of our clinical supplies of our product candidates, and we intend to rely on third parties to produce commercial supplies of any approved product candidate. Therefore, our development of our products could be stopped or delayed, and our commercialization of any future product could be stopped or delayed or made less profitable if third party manufacturers fail to obtain approval of the FDA or comparable regulatory authorities or fail to provide us with drug product in sufficient quantities or at acceptable prices.

The manufacture of biotechnology and pharmaceutical products is complex and requires significant expertise, capital investment, process controls and know-how. Common difficulties in biotechnology and pharmaceutical manufacturing may include: sourcing and producing raw materials, transferring technology from chemistry and development activities to production activities, validating initial production designs, scaling manufacturing techniques, improving costs and yields, establishing and maintaining quality controls and stability requirements, eliminating contaminations and operator errors, and maintaining compliance with regulatory requirements. We do not currently have nor do we plan to acquire the infrastructure or capability internally in accordance with cGMP prescribed by the FDA or to produce an adequate supply of compounds to meet future requirements for clinical trials and commercialization of our products. Drug manufacturing facilities are subject to inspection before the FDA will issue an approval to market a new drug product, and all of the manufacturers that we intend to use must adhere to the cGMP regulations prescribed by the FDA.

We expect therefore to rely on third-party manufacturers for clinical supplies of our product candidates that we may develop. These third-party manufacturers will be required to comply with current good manufacturing practices, or GMPs, and other applicable laws and regulations. We will have no control over the ability of these third parties to comply with these requirements, or to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or any other applicable regulatory authorities do not approve the facilities of these third parties for the manufacture of our other product candidates or any products that we may successfully develop, or if it withdraws any such

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approval, or if our suppliers or contract manufacturers decide they no longer want to supply or manufacture for us, we may need to find alternative manufacturing facilities, in which case we might not be able to identify manufacturers for clinical or commercial supply on acceptable terms, or at all. Any of these factors would significantly impact our ability to develop, obtain regulatory approval for or market our product candidates and adversely affect our business.

We and/or our third-party manufacturers may be adversely affected by developments outside of our control, and these developments may delay or prevent further manufacturing of our products. Adverse developments may include labor disputes, resource constraints, shipment delays, inventory shortages, lot failures, unexpected sources of contamination, lawsuits related to our manufacturing techniques, equipment used during manufacturing, or composition of matter, unstable political environments, acts of terrorism, war, natural disasters, and other natural and man-made disasters. If BioXcel, we or our third-party manufacturers were to encounter any of the above difficulties, or otherwise fail to comply with contractual obligations, our ability to provide any product for clinical trial or commercial purposes would be jeopardized. This may increase the costs associated with completing our clinical trials and commercial production. Further, production disruptions may cause us to terminate ongoing clinical trials and/or commence new clinical trials at additional expense. We may also have to take inventory write-offs and incur other charges and expenses for products that fail to meet specifications or pass safety inspections. If production difficulties cannot be solved with acceptable costs, expenses, and timeframes, we may be forced to abandon our clinical development and commercialization plans, which could have a material adverse effect on our business, prospects, financial condition, and the value of our securities.

We, or third-party manufacturers on whom we rely, may be unable to successfully scale-up manufacturing of our product candidates in sufficient quality and quantity, which would delay or prevent us from developing our product candidates and commercializing approved products, if any.

In order to conduct clinical trials of our product candidates and commercialize any approved product candidates, we, or our manufacturers, will need to manufacture them in large quantities. We, or our manufacturers, may be unable to successfully increase the manufacturing capacity for any of our product candidates in a timely or cost-effective manner, or at all. In addition, quality issues may arise during scale-up activities. If we, or any of our manufacturers, are unable to successfully scale up the manufacture of our product candidates in sufficient quality and quantity, the development, testing, and clinical trials of that product candidate may be delayed or infeasible, and regulatory approval or commercial launch of any resulting product may be delayed or not obtained, which could significantly harm our business. If we are unable to obtain or maintain third-party manufacturing for commercial supply of our product candidates, or to do so on commercially reasonable terms, we may not be able to develop and commercialize our product candidates successfully.

Our failure to find third party collaborators to assist or share in the costs of product development could materially harm our business, financial condition and results of operations.

Our strategy for the development and commercialization of our proprietary product candidates may include the formation of collaborative arrangements with third parties. We are party to a collaboration research agreement with Nektar Therapeutics, Inc., or Nektar, relating to Nektar's NKTR-214 compound and BXCL 701. Existing and future collaborators have significant discretion in determining the efforts and resources they apply and may not perform their obligations as expected. Potential third party collaborators include biopharmaceutical, pharmaceutical and biotechnology companies, academic institutions and other entities. Third-party collaborators may assist us in:

- funding research, preclinical development, clinical trials and manufacturing;

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- seeking and obtaining regulatory approvals; and
- successfully commercializing any future product candidates.

If we are not able to establish further collaboration agreements, we may be required to undertake product development and commercialization at our own expense. Such an undertaking may limit the number of product candidates that we will be able to develop, significantly increase our capital requirements and place additional strain on our internal resources. Our failure to enter into additional collaborations could materially harm our business, financial condition and results of operations.

In addition, our dependence on licensing, collaboration and other agreements with third parties may subject us to a number of risks. These agreements may not be on terms that prove favorable to us and may require us to relinquish certain rights in our product candidates. To the extent we agree to work exclusively with one collaborator in a given area, our opportunities to collaborate with other entities could be curtailed. Lengthy negotiations with potential new collaborators may lead to delays in the research, development or commercialization of product candidates. The decision by our collaborators to pursue alternative technologies or the failure of our collaborators to develop or commercialize successfully any product candidate to which they have obtained rights from us could materially harm our business, financial condition and results of operations.

We rely on third parties to conduct our preclinical and clinical trials. If these third parties do not successfully perform their contractual legal and regulatory duties or meet expected deadlines, we may not be able to obtain regulatory approval for or commercialize our product candidates and our business could be substantially harmed.

We have relied upon and plan to continue to rely upon third-party medical institutions, clinical investigators, contract laboratories and other third party CROs to monitor and manage data for our ongoing preclinical and clinical programs. We rely on these parties for execution of our preclinical and clinical trials, and control only certain aspects of their activities. Nevertheless, we are responsible for ensuring that each of our studies is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards, and our reliance on the CROs does not relieve us of our regulatory responsibilities. We and our CROs are required to comply with cGCPs, which are regulations and guidelines enforced by the FDA, the Competent Authorities of the Member States of the European Economic Area, or EEA, and comparable foreign regulatory authorities for all of our products in clinical development.

Regulatory authorities enforce these cGCPs through periodic inspections of trial sponsors, principal investigators and trial sites. If we or any of our CROs fail to comply with applicable cGCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA, the EMA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical trials comply with cGCP regulations. In addition, our clinical trials must be conducted with product produced under current good manufacturing practices, or cGMP, regulations. Our failure to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process.

If any of our relationships with these third-party CROs terminate, we may not be able to enter into arrangements with alternative CROs or to do so on commercially reasonable terms. In addition, our CROs are not our employees, and except for remedies available to us under our agreements with such CROs, we cannot control whether or not they devote sufficient time and resources to our on-going clinical, nonclinical and preclinical programs. If CROs do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols, regulatory requirements or for other reasons, our clinical trials may be extended,

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delayed or terminated and we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. As a result, our results of operations and the commercial prospects for our product candidates would be harmed, our costs could increase and our ability to generate revenues could be delayed.

Many of the third parties with whom we contract may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other drug development activities that could harm our competitive position. If the third parties conducting our GLP preclinical studies or our clinical trials do not perform their contractual duties or obligations, experience work stoppages, do not meet expected deadlines, terminate their agreements with us or need to be replaced, or if the quality or accuracy of the clinical data they obtain is compromised due to their failure to adhere to our clinical trial protocols or to GCPs, or for any other reason, we may need to enter into new arrangements with alternative third parties. Switching or adding additional CROs involves additional cost and requires management time and focus. In addition, there is a natural transition period when a new CRO commences work. As a result, delays occur, which can materially impact our ability to meet our desired clinical development timelines. Though we carefully manage our relationships with our CROs, there can be no assurance that we will not encounter similar challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition and prospects.

Risks Related to Our Business and Industry

We will need to increase the size of our organization and the scope of our outside vendor relationships, and we may experience difficulties in managing growth.

As of December 31, 2017, we employed a total of four full-time employees and our parent, BioXcel, has two employees who are leased to us pursuant to the Services Agreement. In addition, we will have access to certain of BioXcel's employees and resources through the various agreements we have entered into with BioXcel. Our current internal departments include finance, research and development and administration. We intend to expand our management team to include an operation ramp up of additional technical staff required to achieve our business objectives. We will need to expand our managerial, operational, technical and scientific, financial and other resources in order to manage our operations and clinical trials, establish independent manufacturing, continue our research and development activities, and commercialize our product candidate. Our management and scientific personnel, systems and facilities currently in place may not be adequate to support our future growth.

Our need to effectively manage our operations, growth and various projects requires that we:

- manage our clinical trials effectively, including our planned clinical trials of BXCL501, BXCL701 and our other product candidates;
- manage our internal development efforts effectively while carrying out our contractual obligations to licensors, contractors and other third parties;
- continue to improve our operational, financial and management controls and reporting systems and procedures; and
- attract and retain sufficient numbers of talented employees.

We may utilize the services of third party vendors to perform tasks including pre-clinical and clinical trial management, statistics and analysis, regulatory affairs, medical advisory, market research, formulation development, chemistry, manufacturing and control activities, other drug development functions, legal, auditing, financial advisory, and investor relations. Our growth strategy may also entail expanding our group of contractors or consultants to implement these and other tasks going forward. Because we rely on numerous consultants, to outsource many key functions of our business, we will

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need to be able to effectively manage these consultants to ensure that they successfully carry out their contractual obligations and meet expected deadlines. However, if we are unable to effectively manage our outsourced activities or if the quality or accuracy of the services provided by consultants is compromised for any reason, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for our product candidate or otherwise advance our business. There can be no assurance that we will be able to manage our existing consultants or find other competent outside contractors and consultants on economically reasonable terms, or at all. If we are not able to effectively expand our organization by hiring new employees and expanding our groups of consultants and contractors, we may be unable to successfully implement the tasks necessary to further develop and commercialize our product candidate and, accordingly, may not achieve our research, development and commercialization goals.

We depend on our senior management team, and the loss of one or more of our executive officers or key employees or an inability to attract and retain highly skilled employees could adversely affect our business.

Our success depends largely upon the continued services of our key executive officers, Vimal Mehta, our Chief Executive Officer, President, Secretary and Director and Frank Yocca, our Chief Scientific Officer. We do not maintain "key person" insurance for any of these executive officers or any of our other key employees. We also rely on our leadership team in the areas of research and development, marketing, services and general and administrative functions. From time to time, there may be changes in our executive management and leadership teams resulting from the hiring or departure of executives or other key employees, which could disrupt our business. The replacement of one or more of our executive officers or other key employees would likely involve significant time and costs and may significantly delay or prevent the achievement of our business objectives.

To continue to execute our growth strategy, we also must attract and retain highly skilled personnel. We might not be successful in maintaining our unique culture and continuing to attract and retain qualified personnel. We have from time to time in the past experienced, and we expect to continue to experience in the future, difficulty in hiring and retaining highly skilled personnel with appropriate qualifications. The pool of qualified personnel with SaaS, or experience working with the pharma market is limited overall. In addition, many of the companies with which we compete for experienced personnel have greater resources than we have.

In addition, in making employment decisions, particularly in the internet, biotechnology and high-technology industries, job candidates often consider the value of the stock options or other equity instruments they are to receive in connection with their employment. Volatility in the price of our stock might, therefore, adversely affect our ability to attract or retain highly skilled personnel. Furthermore, the requirement to expense stock options and other equity instruments might discourage us from granting the size or type of stock option or equity awards that job candidates require to join our company. If we fail to attract new personnel or fail to retain and motivate our current personnel, our business and future growth prospects could be severely harmed.

Our employees may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements and insider trading.

We are exposed to the risk of employee fraud or other misconduct. Misconduct by employees could include intentional failures to comply with any regulations applicable to us, to provide accurate information to regulatory authorities, to comply with manufacturing standards we have established, to comply with federal and state healthcare fraud and abuse laws and regulations, or to report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion,

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sales commission, customer incentive programs and other business arrangements. Employee misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. We have adopted a Code of Business Conduct, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risk.

Business interruptions could adversely affect future operations, revenues, and financial conditions, and may increase our costs and expenses.

Our operations, and those of our directors, advisors, contractors, consultants, CROs, and collaborators, could be adversely affected by earthquakes, floods, hurricanes, typhoons, extreme weather conditions, fires, water shortages, power failures, business systems failures, medical epidemics and other natural and man-made disaster or business interruptions. Our phones, electronic devices and computer systems and those of our directors, advisors, contractors, consultants, CROs, and collaborators are vulnerable to damages, theft and accidental loss, negligence, unauthorized access, terrorism, war, electronic and telecommunications failures, and other natural and man-made disasters. Operating as a virtual company, our employees conduct business outside of our headquarters and leased or owned facilities. These locations may be subject to additional security and other risk factors due to the limited control of our employees. If such an event as described above were to occur in the future, it may cause interruptions in our operations, delay research and development programs, clinical trials, regulatory activities, manufacturing and quality assurance activities, sales and marketing activities, hiring, training of employees and persons within associated third parties, and other business activities. For example, the loss of clinical trial data from completed or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data.

Likewise, we will rely on third parties to manufacture BXCL501 and BXCL701 and conduct clinical trials, and similar events as those described in the prior paragraph relating to their business systems, equipment and facilities could also have a material adverse effect on our business. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development and commercialization of our product candidate could be delayed or altogether terminated.

Our independent registered public accounting firm has included an explanatory paragraph relating to our ability to continue as a going concern in its report on our audited financial statements included in this prospectus.

Our audited financial statements at December 31, 2017 and 2016 and for the years then ended were prepared assuming that we will continue as a going concern.

Primarily as a result of our losses and limited cash balances, the report of our independent registered public accounting firm included elsewhere in this prospectus contains an explanatory paragraph on our financial statements stating there is substantial doubt about our ability to continue as a going concern due to recurring losses from operations and deficiencies in working capital and net capital. Such an opinion could materially limit our ability to raise additional funds through the issuance of new debt or equity securities or otherwise. There is no assurance that sufficient financing will be available when needed to allow us to continue as a going concern. The perception that we may not be able to continue as a going concern may also make it more difficult to operate our business due to concerns about our ability to meet our contractual obligations. Our ability to continue as a going concern is contingent upon, among other factors, the sale of the shares of our common stock in this offering or obtaining alternate financing. We cannot provide any assurance that we will be able to raise additional capital.

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If we are unable to secure additional capital, we may be required to curtail our research and development initiatives and take additional measures to reduce costs in order to conserve our cash in amounts sufficient to sustain operations and meet our obligations. These measures could cause significant delays in our clinical and regulatory efforts, which is critical to the realization of our business plan. The accompanying financial statements do not include any adjustments that may be necessary should we be unable to continue as a going concern. It is not possible for us to predict at this time the potential success of our business. The revenue and income potential of our proposed business and operations are currently unknown. If we cannot continue as a viable entity, you may lose some or all of your investment.

Our failure to successfully acquire, develop and market additional product candidates or approved drug products could impair our ability to grow.

As part of our growth strategy, we may evaluate, acquire, license, develop and/or market additional product candidates and technologies. These investments will not constitute a significant portion of our business. However, our internal research capabilities are limited, we may be dependent upon pharmaceutical and biotechnology companies, academic scientists and other researchers to sell or license products or technology to us. The success of this strategy depends partly upon our ability to identify, select and acquire promising pharmaceutical product candidates and products. The process of proposing, negotiating and implementing a license or acquisition of a product candidate or approved product is lengthy and complex. Other companies, including some with substantially greater financial, marketing and sales resources, may compete with us for the license or acquisition of product candidates and approved products. We have limited resources to identify and execute the acquisition or in-licensing of third party products, businesses and technologies and integrate them into our current infrastructure. Moreover, we may devote resources to potential acquisitions or in-licensing opportunities that are never completed, or we may fail to realize the anticipated benefits of such efforts. We may not be able to acquire the rights to additional product candidates on terms that we find acceptable, or at all.

In addition, future acquisitions may entail numerous operational and financial risks, including:

- exposure to unknown liabilities;
- disruption of our business and diversion of our management's and technical personnel's time and attention to develop acquired products or technologies;
- incurrence of substantial debt or dilutive issuances of securities to pay for acquisitions;
- higher than expected acquisition and integration costs;
- increased amortization expenses;
- difficulty and cost in combining the operations and personnel of any acquired businesses with our operations and personnel;
- impairment of relationships with key suppliers or customers of any acquired businesses due to changes in management and ownership; and
- inability to retain key employees of any acquired businesses.

Any product candidate that we acquire may require additional development efforts prior to commercial sale, including extensive clinical testing and approval by the FDA and applicable foreign regulatory authorities. All product candidates are prone to risks of failure typical of pharmaceutical product development, including the possibility that a product candidate will not be shown to be sufficiently safe and effective for approval by regulatory authorities. In addition, we cannot provide assurance that any

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products that we develop or approved products that we acquire will be manufactured profitably or achieve market acceptance.

Risks Related to Our Intellectual Property

It is difficult and costly to protect our proprietary rights, and we may not be able to ensure their protection. If our patent position does not adequately protect our product candidates, others could compete against us more directly, which would harm our business, possibly materially.

Our commercial success will depend in part on obtaining and maintaining patent protection and trade secret protection of our current and future product candidates, the processes used to manufacture them and the methods for using them, as well as successfully defending these patents against third-party challenges. We are the owner of record of patent applications pending in the United States and in certain foreign jurisdictions. We own Patent Cooperation Treaty, or PCT, patent applications relating to our platform technologies covering methods of use and applications of the platform technologies. To date, no patents have been issued to us specifically covering our product candidates, and we cannot be certain that any patents will issue with claims that cover our product candidates. Our ability to stop third parties from making, using, selling, offering to sell or importing our product candidates is dependent upon the extent to which we have rights under valid and enforceable patents or trade secrets that cover these activities.

The patent positions of biotechnology and pharmaceutical companies can be highly uncertain and involve complex legal and factual questions for which important legal principles remain unresolved. No consistent policy regarding the breadth of claims allowed in pharmaceutical patents has emerged to date in the United States or in foreign jurisdictions outside of the United States. Changes in either the patent laws or interpretations of patent laws in the United States and other countries may diminish the value of our intellectual property. Accordingly, we cannot predict the breadth of claims that may be enforced in the patents that may be issued from the applications we currently or may in the future own or license from third parties. Further, if any patents we obtain or license are deemed invalid and unenforceable, our ability to commercialize or license our technology could be adversely affected.

Others have filed, and in the future are likely to file, patent applications covering products and technologies that are similar, identical or competitive to ours or important to our business. We cannot be certain that any patent application owned by a third party will not have priority over patent applications filed or in-licensed by us, or that we or our licensors will not be involved in interference, opposition, reexamination, review, reissue, post grant review or invalidity proceedings before U.S. or non-U.S. patent offices.

The degree of future protection for our proprietary rights is uncertain because legal means afford only limited protection and may not adequately protect our rights or permit us to gain or keep our competitive advantage. For example:

- others may be able to make compounds that are similar to our product candidates, but that are not covered by the claims of our patents;
- we might not have been the first to make the inventions covered by our pending patent applications;
- we might not have been the first to file patent applications for these inventions;
- our pending patent applications may not result in issued patents;
- the claims of our issued patents or patent applications when issued may not cover our products or product candidates;
- any patents that we obtain may not provide us with any competitive advantages;

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- any granted patents may be held invalid or unenforceable as a result of legal challenges by third parties; and
- the patents of others may have an adverse effect on our business.

If we fail to comply with our obligations in the agreements under which we may license intellectual property rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose rights that are important to our business.

We may be required to enter into intellectual property license agreements that are important to our business. These license agreements may impose various diligence, milestone payment, royalty and other obligations on us. For example, we may enter into exclusive license agreements with various universities and research institutions, we may be required to use commercially reasonable efforts to engage in various development and commercialization activities with respect to licensed products, and may need to satisfy specified milestone and royalty payment obligations. If we fail to comply with any obligations under our agreements with any of these licensors, we may be subject to termination of the license agreement in whole or in part; increased financial obligations to our licensors or loss of exclusivity in a particular field or territory, in which case our ability to develop or commercialize products covered by the license agreement will be impaired.

In addition, disputes may arise regarding intellectual property subject to a license agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- our diligence obligations under the license agreement and what activities satisfy those obligations;
- if a third-party expresses interest in an area under a license that we are not pursuing, under the terms of certain of our license agreements, we may be required to sublicense rights in that area to a third party, and that sublicense could harm our business; and
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us.

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates.

We may need to obtain licenses from third parties to advance our research or allow commercialization of our product candidates. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we would be unable to further develop and commercialize one or more of our product candidates, which could harm our business significantly.

We may incur substantial costs as a result of litigation or other proceedings relating to patents and other intellectual property rights.

If we choose to commence a proceeding or litigation to prevent another party from infringing our patents, that party will have the right to ask the examiner or court to rule that our patents are invalid or should not be enforced against them. There is a risk that the examiner or court will decide that our patents are not valid and that we do not have the right to stop the other party from using the related inventions. There is also the risk that, even if the validity of our patents is upheld, the examiner or court will refuse to stop the other party on the ground that such other party's activities do not infringe

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our rights to such patents. In addition, the U.S. Supreme Court has recently modified some tests used by the U.S. Patent and Trademark Office, or USPTO, in granting patents over the past 20 years, which may decrease the likelihood that we will be able to obtain patents and increase the likelihood of challenge to any patents we obtain or license. Any proceedings or litigation to enforce our intellectual property rights or defend ourselves against claims of infringement of third-party intellectual property rights could be costly and divert the attention of managerial and scientific personnel, regardless of whether such litigation is ultimately resolved in our favor. We may not have sufficient resources to bring these actions to a successful conclusion. Moreover, if we are unable to successfully defend against claims that we have infringed the intellectual property rights of others, we may be prevented from using certain intellectual property and may be liable for damages, which in turn could materially adversely affect our business, financial condition or results of operations.

We may infringe the intellectual property rights of others, which may prevent or delay our product development efforts and stop us from commercializing or increase the costs of commercializing our product candidates.

Our success will depend in part on our ability to operate without infringing the proprietary rights of third parties. We cannot guarantee that our products, or manufacture or use of our product candidates, will not infringe third-party patents. Furthermore, a third party may claim that we are using inventions covered by the third party's patent rights and may go to court to stop us from engaging in our normal operations and activities, including making or selling our product candidates. These lawsuits are costly and could affect our results of operations and divert the attention of managerial and scientific personnel. Some of these third parties may be better capitalized and have more resources than us. There is a risk that a court would decide that we are infringing the third party's patents and would order us to stop the activities covered by the patents. In that event, we may not have a viable way around the patent and may need to halt commercialization of the relevant product candidate. In addition, there is a risk that a court will order us to pay the other party damages for having violated the other party's patents. In addition, we may be obligated to indemnify our licensors and collaborators against certain intellectual property infringement claims brought by third parties, which could require us to expend additional resources. The pharmaceutical and biotechnology industries have produced a proliferation of patents, and it is not always clear to industry participants, including us, which patents cover various types of products or methods of use. The coverage of patents is subject to interpretation by the courts, and the interpretation is not always uniform.

If we are sued for patent infringement, we would need to demonstrate that our products or methods either do not infringe the patent claims of the relevant patent or that the patent claims are invalid, and we may not be able to do this. Proving invalidity is difficult. For example, in the United States, proving invalidity requires a showing of clear and convincing evidence to overcome the presumption of validity enjoyed by issued patents. Even if we are successful in these proceedings, we may incur substantial costs and divert management's time and attention in pursuing these proceedings, which could have a material adverse effect on us. If we are unable to avoid infringing the patent rights of others, we may be required to seek a license, which may not be available, defend an infringement action or challenge the validity of the patents in court. Patent litigation is costly and time consuming. We may not have sufficient resources to bring these actions to a successful conclusion. In addition, if we do not obtain a license, develop or obtain non-infringing technology, fail to defend an infringement action successfully or have infringed patents declared invalid, we may incur substantial monetary damages, encounter significant delays in bringing our product candidates to market and be precluded from manufacturing or selling our product candidates.

We cannot be certain that others have not filed patent applications for technology covered by our pending applications, or that we were the first to invent the technology, because:

- some patent applications in the United States may be maintained in secrecy until the patents are issued;

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- patent applications in the United States are typically not published until 18 months after the priority date; and
- publications in the scientific literature often lag behind actual discoveries.

Our competitors may have filed, and may in the future file, patent applications covering technology similar to ours. Any such patent application may have priority over our patent applications, which could further require us to obtain rights to issued patents covering such technologies. If another party has filed USpatent applications on inventions similar to ours that claims priority to any applications filed prior to the priority dates of our applications, we may have to participate in an interference proceeding declared by the USPTO to determine priority of invention in the United States. The costs of these proceedings could be substantial, and it is possible that such efforts would be unsuccessful if, unbeknownst to us, the other party had independently arrived at the same or similar inventions prior to our own inventions, resulting in a loss of our U.S. patent position with respect to such inventions. Other countries have similar laws that permit secrecy of patent applications, and may be entitled to priority over our applications in such jurisdictions.

Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise the funds necessary to continue our operations.

If we are not able to adequately prevent disclosure of trade secrets and other proprietary information, the value of our technology and products could be significantly diminished.

We also rely on trade secrets to protect our proprietary technologies, especially where we do not believe patent protection is appropriate or obtainable. However, trade secrets are difficult to protect. We rely in part on confidentiality agreements with our employees, consultants, outside scientific collaborators, sponsored researchers and other advisors to protect our trade secrets and other proprietary information. These agreements may not effectively prevent disclosure of confidential information and may not provide an adequate remedy in the event of unauthorized disclosure of confidential information. Furthermore, any license agreements we enter into in the future may require us to notify, and in some cases license back to the licensor, certain additional proprietary information or intellectual property that we developed using the rights licensed to us under these agreements. Any such licenses back to the licensor could allow our licensors to use that proprietary information or intellectual property in a manner that could harm our business. In addition, others may independently discover our trade secrets and proprietary information. For example, the FDA, as part of its transparency initiative, is currently considering whether to make additional information publicly available on a routine basis, including information that we may consider to be trade secrets or other proprietary information, and it is not clear at the present time how the FDA's disclosure policies may change in the future, if at all. Costly and time-consuming litigation could be necessary to enforce and determine the scope of our proprietary rights, and failure to obtain or maintain trade secret protection could adversely affect our competitive business position.

We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed alleged trade secrets.

As is common in the biotechnology and pharmaceutical industries, we employ individuals who were previously employed at other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and independent contractors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed trade secrets or other proprietary information of their

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former employers. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we could lose valuable intellectual property rights or personnel, which could adversely impact our business. Even if we are successful in defending against these claims, litigation could result in substantial costs and be a distraction to management.

Our intellectual property may not be sufficient to protect our products from competition, which may negatively affect our business as well as limit our partnership or acquisition appeal.

We may be subject to competition despite the existence of intellectual property we license or own. We can give no assurances that our intellectual property claims will be sufficient to prevent third parties from designing around patents we own or license and developing and commercializing competitive products. The existence of competitive products that avoid our intellectual property could materially adversely affect our operating results and financial condition. Furthermore, limitations, or perceived limitations, in our intellectual property may limit the interest of third parties to partner, collaborate or otherwise transact with us, if third parties perceive a higher than acceptable risk to commercialization of our products or future products.

Our drug re-innovation approach involves the filing of patent applications covering new methods of use and/or new formulations of previously known, studied and/or marketed drugs. Although the protection afforded by our patent applications may be significant with respect to BXCL501 and BXCL701, when looking at our patents' ability to block competition, the protection offered by our patents may be, to some extent, more limited than the protection provided by patents claiming the composition of matter of entirely new chemical structures previously unknown. If a competitor were able to successfully design around any method of use and formulation patents we may have in the future, our business and competitive advantage could be significantly affected.

We may elect to sue a third party, or otherwise make a claim, alleging infringement or other violation of patents, trademarks, trade dress, copyrights, trade secrets, domain names or other intellectual property rights that we either own or license from BioXcel. If we do not prevail in enforcing our intellectual property rights in this type of litigation, we may be subject to:

- paying monetary damages related to the legal expenses of the third party;
- facing additional competition that may have a significant adverse effect on our product pricing, market share, business operations, financial condition, and the commercial viability of our products; and
- restructuring our company or delaying or terminating select business opportunities, including, but not limited to, research and development, clinical trial, and commercialization activities, due to a potential deterioration of our financial condition or market competitiveness.

A third party may also challenge the validity, enforceability or scope of the intellectual property rights that we license or own; and, the result of these challenges may narrow the scope or claims of or invalidate patents that are integral to our product candidates in the future. There can be no assurance that we will be able to successfully defend patents we own in an action against third parties due to the unpredictability of litigation and the high costs associated with intellectual property litigation, amongst other factors.

Intellectual property rights and enforcement may be less extensive in jurisdictions outside of the United States; thus, we may not be able to protect our intellectual property and third parties may be able to market competitive products that may use some or all of our intellectual property.

Changes to patent law, including the Leahy-Smith America Invests Act, AIA or Leahy-Smith Act, of 2011 and the Patent Reform Act of 2009 and other future article of legislation, may substantially change the regulations and procedures surrounding patent applications, issuance of patents, and

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prosecution of patents. We can give no assurances that our patents and those of our licensor, BioXcel, can be defended or will protect us against future intellectual property challenges, particularly as they pertain to changes in patent law and future patent law interpretations.

In addition, enforcing and maintaining our intellectual property protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by the U.S. Patent and Trademark Office, courts and foreign government patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Risks Related to Owning our Common Stock and this Offering

An active trading market for our common stock may not develop, and you may not be able to sell your common stock at or above the initial public offering price.

Prior to the consummation of this offering, there has been no public market for our common stock. An active trading market for shares of our common stock may never develop or be sustained following this offering. If an active trading market does not develop, you may have difficulty selling your shares of common stock at an attractive price, or at all. The price for our common stock in this offering will be determined by negotiations between us and the underwriters, and it may not be indicative of prices that will prevail in the open market following this offering. Consequently, you may not be able to sell your common stock at or above the initial public offering price or at any other price or at the time that you would like to sell. An inactive market may also impair our ability to raise capital by selling our common stock, and it may impair our ability to attract and motivate our employees through equity incentive awards and our ability to acquire other companies, products or technologies by using our common stock as consideration.

The price of our common stock may fluctuate substantially.

You should consider an investment in our common stock to be risky, and you should invest in our common stock only if you can withstand a significant loss and wide fluctuations in the market value of your investment. Some factors that may cause the market price of our common stock to fluctuate, in addition to the other risks mentioned in this "Risk Factors" section and elsewhere in this prospectus, are:

- sale of our common stock by our stockholders, executives, and directors;
- volatility and limitations in trading volumes of our shares of common stock;
- our ability to obtain financings to conduct and complete research and development activities including, but not limited to, our clinical trials, and other business activities;
- possible delays in the expected recognition of revenue due to lengthy and sometimes unpredictable sales timelines;
- the timing and success of introductions of new applications and services by us or our competitors or any other change in the competitive dynamics of our industry, including consolidation among competitors, customers or strategic partners;
- network outages or security breaches;
- our ability to attract new customers;
- customer renewal rates and the timing and terms of customer renewals;
- our ability to secure resources and the necessary personnel to conduct clinical trials on our desired schedule;

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- commencement, enrollment or results of our clinical trials for our product candidates or any future clinical trials we may conduct;
- changes in the development status of our product candidates;
- any delays or adverse developments or perceived adverse developments with respect to the FDA's review of our planned preclinical and clinical trials;
- any delay in our submission for studies or product approvals or adverse regulatory decisions, including failure to receive regulatory approval for our product candidates;
- unanticipated safety concerns related to the use of our product candidates;
- failures to meet external expectations or management guidance;
- changes in our capital structure or dividend policy, future issuances of securities, sales of large blocks of common stock by our stockholders;
- our cash position;
- announcements and events surrounding financing efforts, including debt and equity securities;
- our inability to enter into new markets or develop new products;
- reputational issues;
- competition from existing technologies and products or new technologies and products that may emerge;
- announcements of acquisitions, partnerships, collaborations, joint ventures, new products, capital commitments, or other events by us or our competitors;
- changes in general economic, political and market conditions in or any of the regions in which we conduct our business;
- changes in industry conditions or perceptions;
- changes in valuations of similar companies or groups of companies;
- analyst research reports, recommendation and changes in recommendations, price targets, and withdrawals of coverage;
- departures and additions of key personnel;
- disputes and litigations related to intellectual properties, proprietary rights, and contractual obligations;
- changes in applicable laws, rules, regulations, or accounting practices and other dynamics; and
- other events or factors, many of which may be out of our control.

In addition, if the market for stocks in our industry or industries related to our industry, or the stock market in general, experiences a loss of investor confidence, the trading price of our common stock could decline for reasons unrelated to our business, financial condition and results of operations. If any of the foregoing occurs, it could cause our stock price to fall and may expose us to lawsuits that, even if unsuccessful, could be costly to defend and a distraction to management.

We have broad discretion in the use of the net proceeds from this offering and may not use them effectively.

Our management will have broad discretion in the application of the net proceeds from this initial public offering, including for any of the currently intended purposes described in the section entitled "Use of Proceeds." Because of the number and variability of factors that will determine our use of the

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net proceeds from this offering, their ultimate use may vary substantially from their currently intended use. Our management may not apply our cash from this offering in ways that ultimately increase the value of any investment in our securities or enhance shareholder value. The failure by our management to apply these funds effectively could harm our business. Pending their use, we may invest the net proceeds from this offering in short-term, investment-grade, interest-bearing securities. These investments may not yield a favorable return to our stockholders. If we do not invest or apply our cash in ways that enhance shareholder value, we may fail to achieve expected financial results, which may result in a decline in the price of our shares of common stock, and, therefore, may negatively impact our ability to raise capital, invest in or expand our business, acquire additional products or licenses, commercialize our products, or continue our operations.

We may acquire other companies or technologies, which could divert our management's attention, result in dilution to our stockholders and otherwise disrupt our operations and adversely affect our operating results.

We may in the future seek to acquire or invest in businesses, applications and services or technologies that we believe could complement or expand our services, enhance our technical capabilities or otherwise offer growth opportunities. The pursuit of potential acquisitions may divert the attention of management and cause us to incur various expenses in identifying, investigating and pursuing suitable acquisitions, whether or not they are consummated.

In addition, we do not have any experience in acquiring other businesses. If we acquire additional businesses, we may not be able to integrate the acquired personnel, operations and technologies successfully, or effectively manage the combined business following the acquisition. We also may not achieve the anticipated benefits from the acquired business due to a number of factors, including:

- inability to integrate or benefit from acquired technologies or services in a profitable manner;
- unanticipated costs or liabilities associated with the acquisition;
- difficulty integrating the accounting systems, operations and personnel of the acquired business;
- difficulties and additional expenses associated with supporting legacy products and hosting infrastructure of the acquired business;
- difficulty converting the customers of the acquired business onto our platform and contract terms, including disparities in the revenue, licensing, support or professional services model of the acquired company;
- diversion of management's attention from other business concerns;
- adverse effects to our existing business relationships with business partners and customers as a result of the acquisition;
- the potential loss of key employees;
- use of resources that are needed in other parts of our business; and
- use of substantial portions of our available cash to consummate the acquisition.

In addition, a significant portion of the purchase price of companies we acquire may be allocated to acquired goodwill and other intangible assets, which must be assessed for impairment at least annually. In the future, if our acquisitions do not yield expected returns, we may be required to take charges to our operating results based on this impairment assessment process, which could adversely affect our results of operations.

Acquisitions could also result in dilutive issuances of equity securities or the incurrence of debt, which could adversely affect our operating results. In addition, if an acquired business fails to meet our expectations, our operating results, business and financial position may suffer.

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Market and economic conditions may negatively impact our business, financial condition and share price.

Concerns over inflation, energy costs, geopolitical issues, the U.S. mortgage market and a declining real estate market, unstable global credit markets and financial conditions, and volatile oil prices have led to periods of significant economic instability, diminished liquidity and credit availability, declines in consumer confidence and discretionary spending, diminished expectations for the global economy and expectations of slower global economic growth going forward, increased unemployment rates, and increased credit defaults in recent years. Our general business strategy may be adversely affected by any such economic downturns, volatile business environments and continued unstable or unpredictable economic and market conditions. If these conditions continue to deteriorate or do not improve, it may make any necessary debt or equity financing more difficult to complete, more costly, and more dilutive. Failure to secure any necessary financing in a timely manner and on favorable terms could have a material adverse effect on our growth strategy, financial performance, and share price and could require us to delay or abandon development or commercialization plans.

If securities or industry analysts do not publish research or reports, or publish unfavorable research or reports about our business, our stock price and trading volume may decline.

The trading market for our common stock will rely in part on the research and reports that industry or financial analysts publish about us, our business, our markets and our competitors. We do not control these analysts. If securities analysts do not cover our common stock after the closing of this offering, the lack of research coverage may adversely affect the market price of our common stock. Furthermore, if one or more of the analysts who do cover us downgrade our stock or if those analysts issue other unfavorable commentary about us or our business, our stock price would likely decline. If one or more of these analysts cease coverage of us or fails to regularly publish reports on us, we could lose visibility in the market and interest in our stock could decrease, which in turn could cause our stock price or trading volume to decline and may also impair our ability to expand our business with existing customers and attract new customers.

Because certain of our stockholders control a significant number of shares of our common stock, they may have effective control over actions requiring stockholder approval.

Following this offering, our directors, executive officers and principal stockholders, and their respective affiliates, will beneficially own approximately 63% of our outstanding shares of common stock. As a result, these stockholders, acting together, would have the ability to control the outcome of matters submitted to our stockholders for approval, including the election of directors and any merger, consolidation or sale of all or substantially all of our assets. In addition, these stockholders, acting together, would have the ability to control the management and affairs of our company. Accordingly, this concentration of ownership might harm the market price of our common stock by:

- delaying, deferring or preventing a change in corporate control;
- impeding a merger, consolidation, takeover or other business combination involving us; or
- discouraging a potential acquirer from making a tender offer or otherwise attempting to obtain control of us.

You will incur immediate dilution as a result of this offering.

If you purchase common stock in this offering, you will pay more for your shares than the net tangible book value of your shares. As a result, you will incur immediate dilution of $7.47 per share, representing the difference between the initial public offering price of $11.00 per share and our estimated pro forma net tangible book value per share as of December 31, 2017 of $3.53. Accordingly, should we be liquidated at our book value, you would not receive the full amount of your investment.

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Future sales and issuances of our common stock could result in additional dilution of the percentage ownership of our stockholders and could cause our share price to fall.

We expect that significant additional capital will be needed in the future to continue our planned operations, including increased marketing, hiring new personnel, commercializing our products, and continuing activities as an operating public company. To the extent we raise additional capital by issuing equity securities, our stockholders may experience substantial dilution. We may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time. If we sell common stock, convertible securities or other equity securities in more than one transaction, investors may be materially diluted by subsequent sales. Such sales may also result in material dilution to our existing stockholders, and new investors could gain rights superior to our existing stockholders.

We do not intend to pay cash dividends on our shares of common stock so any returns will be limited to the value of our shares.

We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. Any return to stockholders will therefore be limited to the increase, if any, of our share price.

We are an "emerging growth company" and will be able to avail ourselves of reduced disclosure requirements applicable to emerging growth companies, which could make our common stock less attractive to investors.

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and we intend to take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are not "emerging growth companies" including not being required to comply with the auditor attestation requirements of Section 404(b) of the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. In addition, Section 107 of the JOBS Act also provides that an "emerging growth company" can take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act, for complying with new or revised accounting standards. In other words, an "emerging growth company" can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We are not electing to delay such adoption of new or revised accounting standards, and as a result, we will comply with new or revised accounting standards on the relevant dates on which adoption of such standards is required for non-emerging growth companies. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile. We may take advantage of these reporting exemptions until we are no longer an "emerging growth company." We will remain an "emerging growth company" until the earliest of (i) the last day of the fiscal year in which we have total annual gross revenues of $1.07 billion or more; (ii) the last day of our fiscal year following the fifth anniversary of the date of the completion of this offering; (iii) the date on which we have issued more than $1 billion in nonconvertible debt during the previous three years; or (iv) the date on which we are deemed to be a large accelerated filer under the rules of the Securities and Exchange Commission.

We may be at risk of securities class action litigation.

We may be at risk of securities class action litigation. In the past, biotechnology and pharmaceutical companies have experienced significant stock price volatility, particularly when associated with binary events such as clinical trials and product approvals. If we face such litigation, it could result in substantial costs and a diversion of management's attention and resources, which could harm our business and results in a decline in the market price of our common stock.

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There is no assurance that an active and liquid trading market in our common stock will develop.

We have been approved, subject to notice of issuance, to list our shares of common stock on The Nasdaq Capital Market. There can be no assurance any broker will be interested in trading our common stock. Therefore, it may be difficult to sell any shares you purchase in this offering if you desire or need to sell them. Our lead underwriter, Barclays, is not obligated to make a market in our common stock, and even after making a market, can discontinue market making at any time without notice. Neither we nor the underwriters can provide any assurance that an active and liquid trading market in our common stock will develop or, if developed, that the market will continue.

Our certificate of incorporation and our bylaws, and Delaware law may have anti-takeover effects that could discourage, delay or prevent a change in control, which may cause our stock price to decline.

Our amended and restated certificate of incorporation and our amended and restated bylaws, to be effective upon completion of the offering, and Delaware law could make it more difficult for a third party to acquire us, even if closing such a transaction would be beneficial to our stockholders. Upon consummation of this offering, we will be authorized to issue up to 10,000,000 shares of preferred stock. This preferred stock may be issued in one or more series, the terms of which may be determined at the time of issuance by our board of directors without further action by stockholders. The terms of any series of preferred stock may include voting rights (including the right to vote as a series on particular matters), preferences as to dividend, liquidation, conversion and redemption rights and sinking fund provisions. No preferred stock is currently outstanding. The issuance of any preferred stock could materially adversely affect the rights of the holders of our common stock, and therefore, reduce the value of our common stock and the Notes. In particular, specific rights granted to future holders of preferred stock could be used to restrict our ability to merge with, or sell our assets to, a third party and thereby preserve control by the present management.

Provisions of our amended and restated certificate of incorporation and our amended and restated bylaws and Delaware law also could have the effect of discouraging potential acquisition proposals or making a tender offer or delaying or preventing a change in control, including changes a stockholder might consider favorable. Such provisions may also prevent or frustrate attempts by our stockholders to replace or remove our management. In particular, the certificate of incorporation and bylaws and Delaware law, as applicable, among other things:

- provide the board of directors with the ability to alter the bylaws without stockholder approval;
- place limitations on the removal of directors;
- establishing advance notice requirements for nominations for election to the board of directors or for proposing matters that can be acted upon at stockholder meetings; and
- provide that vacancies on the board of directors may be filled by a majority of directors in office, although less than a quorum.

Financial reporting obligations of being a public company in the United States are expensive and time-consuming, and our management will be required to devote substantial time to compliance matters.

As a publicly traded company that is separate from BioXcel, we will incur significant additional legal, accounting and other expenses that we did not incur as a privately held subsidiary of BioXcel. The obligations of being a public company in the United States require significant expenditures and will place significant demands on our management and other personnel, including costs resulting from public company reporting obligations under the Securities Exchange Act of 1934, as amended, or the Exchange Act, and the rules and regulations regarding corporate governance practices, including those under the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act, the Dodd-Frank Wall Street Reform and Consumer Protection Act, or the Dodd-Frank Act, and the listing requirements of the stock exchange on which our securities are listed. These rules require the establishment and maintenance of effective disclosure and financial controls and procedures, internal control over financial reporting and changes in corporate governance practices, among many other complex rules that are often difficult to

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implement, monitor and maintain compliance with. Moreover, despite recent reforms made possible by the JOBS Act, the reporting requirements, rules, and regulations will make some activities more time-consuming and costly, particularly after we are no longer an "emerging growth company." In addition, we expect these rules and regulations to make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to maintain the same or similar coverage that we had through BioXcel. Our management and other personnel will need to devote a substantial amount of time to ensure that we comply with all of these requirements and to keep pace with new regulations, otherwise we may fall out of compliance and risk becoming subject to litigation or being delisted, among other potential problems.

If we fail to comply with the rules under the Sarbanes-Oxley Act of 2002 related to accounting controls and procedures in the future, or, if we discover additional material weaknesses and other deficiencies in our internal control and accounting procedures, our stock price could decline significantly and raising capital could be more difficult. Our management determined that our disclosure controls and procedures and internal controls were ineffective as of December 31, 2017 and 2016 and if they continue to be ineffective could result in material misstatements in our financial statements.

Section 404 of the Sarbanes-Oxley Act requires annual management assessments of the effectiveness of our internal control over financial reporting. If we fail to comply with the rules under the Sarbanes-Oxley Act related to disclosure controls and procedures in the future, or, if we discover material weaknesses and other deficiencies in our internal control and accounting procedures, our stock price could decline significantly and raising capital could be more difficult. In connection with the audit of our consolidated financial statements for the years ended December 31, 2017 and 2016, our management concluded that the Company had material weaknesses in its internal controls because we did not have adequately designed internal controls to ensure the timely preparation and review of the accounting for certain complex, non-routine transactions by those with appropriate technical expertise, which was necessary to provide reasonable assurance that the Company's consolidated financial statements and related disclosures would be prepared in accordance with generally accepted accounting principles in the United States of America. In addition, we did not have adequately designed and documented financial close and management review controls to properly detect and prevent certain accounting errors and omitted disclosures in the financial statements and related footnotes. Upon completion of this offering, we intend to invest as soon as practicable in resources to create a larger finance function with additional personnel to comply with evolving laws, regulations and standards, and this investment may result in increased general and administrative expenses and a diversion of management's time and attention from revenue-generating activities to compliance activities. If additional material weaknesses or significant deficiencies are discovered or if we otherwise fail to achieve and maintain the adequacy of our internal control, we may not be able to ensure that we can conclude on an ongoing basis that we have effective internal controls over financial reporting in accordance with Section 404 of the Sarbanes-Oxley Act. Moreover, effective internal controls are necessary for us to produce reliable financial reports and are important to helping prevent financial fraud. If we cannot provide reliable financial reports or prevent fraud, our business and operating results could be harmed, investors could lose confidence in our reported financial information, and the trading price of our common stock could drop significantly.

Comprehensive tax reform bills could adversely affect our business and financial condition.

The U.S. government recently enacted comprehensive federal income tax legislation that includes significant changes to the taxation of business entities. These changes include, among others, a permanent reduction to the corporate income tax rate. Notwithstanding the reduction in the corporate income tax rate, the overall impact of this tax reform is uncertain, and our business and financial condition could be adversely affected. This prospectus does not discuss any such tax legislation or the manner in which it might affect purchasers of our common stock. We urge our stockholders to consult with their legal and tax advisors with respect to any such legislation and the potential tax consequences of investing in our common stock.

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INFORMATION REGARDING FORWARD-LOOKING STATEMENTS

This prospectus contains forward-looking statements that involve risks and uncertainties. You should not place undue reliance on these forward-looking statements. All statements other than statements of historical facts contained in this prospectus are forward-looking statements. The forward-looking statements in this prospectus are only predictions. We have based these forward-looking statements largely on our current expectations and projections about future events and financial trends that we believe may affect our business, financial condition and results of operations. In some cases, you can identify these forward-looking statements by terms such as "anticipate," "believe," "continue," "could," "depends," "estimate," "expects," "intend," "may," "ongoing," "plan," "potential," "predict," "project," "should," "will," "would" or the negative of those terms or other similar expressions, although not all forward-looking statements contain those words. We have based these forward-looking statements on our current expectations and projections about future events and trends that we believe may affect our financial condition, results of operations, strategy, short- and long-term business operations and objectives, and financial needs. These forward-looking statements include, but are not limited to, statements concerning the following:

- our plans to initiate clinical trials BXCL501, BXCL701 and our other product candidates;
- our plans for 505(b)(2) regulatory path approval;
- our plans to research, develop and commercialize our current and future product candidates;
- our plans to seek to enter into collaborations for the development and commercialization of certain product candidates;
- the potential benefits of any future collaboration;
- the timing of and our ability to obtain and maintain regulatory approvals for our product candidates;
- the rate and degree of market acceptance and clinical utility of any products for which we receive marketing approval;
- our commercialization, marketing and manufacturing capabilities and strategy;
- our intellectual property position and strategy;
- our estimates regarding expenses, future revenue, capital requirements and need for additional financing;
- developments relating to our competitors and our industry;
- the impact of government laws and regulations; and
- risks associated with our relationship with BioXcel.

These forward-looking statements are subject to a number of risks, uncertainties and assumptions, including those described in "Risk Factors." Moreover, we operate in a very competitive and rapidly changing environment. New risks emerge from time to time. It is not possible for our management to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements we may make. In light of these risks, uncertainties and assumptions, the forward-looking events and circumstances discussed in this prospectus may not occur and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements.

You should not rely upon forward-looking statements as predictions of future events. Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee that the future results, levels of activity, performance or events and circumstances reflected

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in the forward-looking statements will be achieved or occur. Moreover, except as required by law, neither we nor any other person assumes responsibility for the accuracy and completeness of the forward-looking statements. We undertake no obligation to update publicly any forward-looking statements for any reason after the date of this prospectus to conform these statements to actual results or to changes in our expectations.

You should read this prospectus and the documents that we reference in this prospectus and have filed with the SEC as exhibits to the registration statement of which this prospectus is a part with the understanding that our actual future results, levels of activity, performance and events and circumstances may be materially different from what we expect.

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INDUSTRY AND MARKET DATA

This prospectus contains estimates and other statistical data made by independent parties and by us relating to market size and growth and other data about our industry. We obtained the industry and market data in this prospectus from our own research as well as from industry and general publications, surveys and studies conducted by third parties. This data involves a number of assumptions and limitations and contains projections and estimates of the future performance of the industries in which we operate that are subject to a high degree of uncertainty, including those discussed in "Risk Factors". We caution you not to give undue weight to such projections, assumptions and estimates. Further, industry and general publications, studies and surveys generally state that they have been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information. While we believe that these publications, studies and surveys are reliable, we have not independently verified the data contained in them. In addition, while we believe that the results and estimates from our internal research are reliable, such results and estimates have not been verified by any independent source.

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USE OF PROCEEDS

We estimate that the net proceeds from our issuance and sale of shares of our common stock in this offering will be approximately $54.2 million, based on the initial public offering price of $11.00 per share and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. If the underwriters exercise their option to purchase additional shares in full, we estimate that the net proceeds from this offering will be approximately $62.6 million.

We intend to use the net proceeds from this offering as follows:

- approximately $25 million to fund BXCL501 through Phase 2 clinical development and potentially one registration trial;
- approximately $17 million to fund BXCL701 through Phase 2 clinical development;
- $1 million to be reimbursed to BioXcel pursuant to the Contribution Agreement;
- $.88 million to be repaid to BioXcel purusant to the Services Agreement and Grid Note; and
- the balance for working capital and other general corporate purposes.

We believe that the net proceeds from this offering and our existing cash, cash equivalents and investments will be sufficient to fund our current operations for at least twelve months from the date of this prospectus. We have based this estimate on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect. We believe the amount of net proceeds from this offering currently allocated to BXCL501 and BXCL701 will be sufficient to fund those programs through Phase 2 clinical development. We will need to raise substantial additional funds to complete registration trials for both BXCL501 and BXCL701 and before we can expect to commercialize any products, if approved. As of the date of this prospectus, we believe we will need approximately $25 million and $40 million to complete registration trials for each indication of BXCL501 and BXCL701, respectively, assuming no accelerated approval pathways are received. We may satisfy our future cash needs through the sale of equity securities, debt financings, working capital lines of credit, corporate collaborations or license agreements, grant funding, interest income earned on invested cash balances or a combination of one or more of these sources. The amount and timing of our actual expenditures will depend upon numerous factors, including the status and results of our planned Phase 2 PoC open label clinical trials in 2018 for both BXCL501 and BXCL701. Furthermore, we anticipate that we will need to secure additional funding for the further development of BXCL501 and BXCL701, and for the development of any of our other product candidates.

This expected use of the net proceeds from this offering, our existing cash and cash equivalents and the amounts we believe we will need to complete registration trials for BXCL501 and BXCL701 represents our intentions based upon our current plans, financial condition and business conditions. Predicting the cost necessary to develop product candidates can be difficult and the amounts and timing of our actual expenditures may vary significantly depending on numerous factors, including the progress of our development and commercialization efforts, the status of and results from clinical trials, any collaborations that we may enter into with third parties for our product candidates and any unforeseen cash needs. As a result, our management will retain broad discretion over the allocation of the net proceeds from this offering and our existing cash and cash equivalents.

In the ordinary course of our business, we expect to from time to time evaluate the acquisition of, investment in or in-license of complementary products, technologies or businesses, and we could use a portion of the net proceeds from this offering for such activities. We currently do not have any agreements, arrangements or commitments with respect to any potential acquisition, investment or license.

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Pending our use of the net proceeds from this offering, we intend to invest the net proceeds in a variety of capital preservation investments, including short-term, investment-grade, interest-bearing instruments and government securities.

In connection with the Services Agreement, we entered into the Grid Note with BioXcel. As of December 31, 2017, we have drawn an amount of $371,000 under the Grid Note. The Grid Note is payable upon the earlier of (i) the completion of this offering and (ii) December 31, 2018, together with interest on the unpaid balance of each advance made under the Grid Note, which shall accrue at a rate per annum equal to the applicable federal rate for short-term loans as of the date hereof, in each case calculated based on a 365-day year and actual days elapsed. We have also agreed to reimburse BioXcel for its contributed services and support to us in connection with our organization and development prior to the date of the Grid Note in the amount of $562,000 of which $122,000 has been repaid as of December 31, 2017 which amount shall be payable upon the earlier of (i) thirty days after the completion of this offering and (ii) December 31, 2018. See the section titled "Certain Relationships and Related Person Transactions—Amended and Restated Separation and Shared Services Agreement with BioXcel" for additional information.

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DIVIDEND POLICY

We have never paid or declared any cash dividends on our common stock, and we do not anticipate paying any cash dividends on our common stock in the foreseeable future. We intend to retain all available funds and any future earnings to fund the development and expansion of our business. Any future determination to pay dividends will be at the discretion of our board of directors and will depend upon a number of factors, including our results of operations, financial condition, future prospects, contractual restrictions, restrictions imposed by applicable law and other factors our board of directors deems relevant.

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CAPITALIZATION

The following table sets forth our cash and cash equivalents and capitalization as of December 31, 2017:

- on an actual basis;
- on a pro forma basis to reflect the sale of 283,452 shares of common stock, at a price of $6.88 per share in January and February 2018 and the filing of our amended and restated certificate of incorporation upon the closing of this offering;
- on a pro forma as adjusted basis to give further effect to our issuance and sale of 5,454,545 shares of our common stock included in the shares of common stock being sold in this offering at the initial public offering price of $11.00 per share, after deducting the estimated underwriting discounts and commissions and our estimated offering expenses.

	December 31, 2017		
(in thousands, except share and per share data)	Actual	Pro Forma (unaudited)	Pro Forma, As Adjusted (unaudited)
Cash	$ 887	$ 2,836	$ 57,036
Short term note payable and amounts due to related party	878	878	878
Stockholders' equity:			
Preferred stock, par value $0.001 per share; no shares authorized, issued or outstanding, actual and pro forma; 10,000,000 shares authorized and no shares issued or outstanding, pro forma as adjusted	—	—	—
Common stock, par value $0.001 per share; 100,000 shares authorized, 41,804 shares issued and outstanding, actual; 50,000,000 shares authorized, 9,907,548 shares issued and outstanding, pro forma; 50,000,000 shares authorized, 15,191,000 shares issued and outstanding, pro forma as adjusted	10	10	15
Additional paid-in capital	3,458	5,407	59,602
Accumulated deficit	(4,450)	(4,450)	(4,450)
Total stockholders' equity/(deficit)	(982)	967	55,167
Total capitalization	$ (982)	$ 967	$ 55,167

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DILUTION

If you invest in our common stock, your ownership interest will be diluted to the extent of the difference between initial public offering price per share of our common stock and the pro forma as adjusted net tangible book value per share of our common stock immediately after this offering.

As of December 31, 2017 we had a historical net tangible book value (deficit) of $(982,000), or $(0.10) per share of common stock, based on shares of common stock outstanding at December 31, 2017. Our historical net tangible book value per share is the amount of our total tangible assets less our total liabilities at December 31, 2017, divided by the number of shares of common stock outstanding at December 31, 2017.

Our pro forma net tangible book value as of December 31, 2017 was $967,000, or $0.10 per share of common stock. Pro forma net tangible book value represents the amount of our total tangible assets less our total liabilities, after giving effect to the sale of 283,452 shares of common stock, at a price of $6.88 per share in January and February 2018.

After giving further effect to the sale of 5,454,545 shares of common stock in this offering at the initial public offering price of $11.00 per share and after deducting the underwriting discounts and commissions and estimated offering expenses payable by us, our pro forma as adjusted net tangible book value at December 31, 2017 would have been $55.2 million, or $3.53 per share of common stock. This represents an immediate increase in pro forma as adjusted net tangible book value of $3.43 per share to existing stockholders and immediate dilution of $7.47 per share to new investors purchasing shares of common stock in this offering.

The following table illustrates this dilution on a per share basis:

Initial public offering price per share		$ 11.00
Pro forma net tangible book value per share as of December 31, 2017	$ 0.10	
Increase in pro forma as adjusted net tangible book value per share attributable to new investors in this offering	3.43	
Pro forma as adjusted net tangible book value per share immediately after this offering		3.53
Dilution per share to new investors in this offering		$ 7.47

If the underwriters exercise their option to purchase additional shares in full, the pro forma as adjusted net tangible book value per share after giving effect to the offering would be $3.86 per share. This represents an increase in pro forma as adjusted net tangible book value of $3.76 per share to existing stockholders and dilution in pro forma as adjusted net tangible book value of $7.14 per share to new investors.

The following table summarizes, on the pro forma as adjusted basis described above, the total number of shares of common stock purchased from us, the total consideration paid or to be paid, and the average price per share paid or to be paid by existing shareholders and by new investors in this offering at the initial public offering price of $11.00 per share, before deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us:

	Shares Purchased		Total Consideration		Average Price
	Number	Percentage	Amount	Percentage	Per Share
Existing shareholders	12,494,877	69.61%	$ 4,010,541	6.3%	$ 0.32
New investors	5,454,545	30.39	59,999,995	93.7	$ 11.00
Total	17,949,422	100.0%	$ 64,010,536	100.0%	

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The table above assumes no exercise of the underwriters' over-allotment option in this offering. If the underwriters' over-allotment option is exercised in full, the number of common shares held by new investors purchasing common stock in this offering would be increased to 33% of the total number of shares of common stock outstanding after this offering, and the number of shares held by existing shareholders would be reduced to 67% of the total number of shares of common stock outstanding after this offering.

To the extent that stock options or warrants are exercised, new stock options are issued under our equity incentive plan, or we issue additional common stock in the future, there will be further dilution to investors participating in this offering. In addition, we may choose to raise additional capital because of market conditions or strategic considerations, even if we believe that we have sufficient funds for our current or future operating plans. If we raise additional capital through the sale of equity or convertible debt securities, the issuance of these securities could result in further dilution to our shareholders.

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SELECTED FINANCIAL DATA

The following table sets forth our selected financial data as of the dates and for the periods indicated. We have derived the statement of operations data for the years ended December 31, 2017 and 2016 from our audited financial statements included elsewhere in this prospectus. The following summary financial data should be read with "Management's Discussion and Analysis of Financial Condition and Results of Operations" and our financial statements and related notes and other information included elsewhere in this prospectus. Our historical results are not necessarily indicative of the results to be expected in future periods.

Our historical results of operations presented below may not be reflective of our financial position, results of operations and cash flows had we operated as a stand-alone public company during all periods presented. Prior to June 30, 2017, BTI operated as part of BioXcel and not as a separate stand-alone entity. Our financial statement prior to June 30, 2017 have been prepared on a "carve-out" basis from the financial statements of BioXcel to represent our financial position and performance as if we had existed on a stand-alone basis during each of the fiscal years presented in the financial statements. The financial information for the period beginning January 1, 2017 through June 30, 2017 have been carved out of the financial statements of BioXcel. Our financial information for the period beginning July 1, 2017 through December 31, 2017 have been prepared as if we are standalone entity. These results reflect amounts specifically attributable to our business, including the costs BioXcel incurred for the assets that were contributed to us by our parent under the Contribution Agreement and the Services Agreement. The agreements provide us with certain general and administrative and development support services that became effective June 30, 2017. However, during the carve-out period, consistent with accounting regulations, we have assumed that we were a separate business within BioXcel and we have reflected the related assets, liabilities and expenses in our results for periods prior to and post incorporation. We believe that such allocations have been made on a reasonable basis, but may not necessarily be indicative of all of the costs that would have been incurred if we had operated on a standalone basis.

Statement of Operations Data:

(in thousands, except share and per share data)

	Years Ended December 31,	
	2017	2016
Revenues	$ —	$ —
Operating costs and expenses		
Research and development	2,690	1,399
General and administrative	1,847	721
Total operating expenses	4,537	2,120
Loss from operations	(4,537)	(2,120)
Other expense		
Interest expense	(2)	—
Net loss	$ (4,539)	$ (2,120)
Net loss per share—basic and diluted	$ (0.47)	$ (0.22)
Weighted average shares outstanding—basic and diluted[1]	9,686,005	9,480,000

[1] See Note 3 to our financial statements for an explanation of the method used to compute basic and diluted net loss per share.

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Balance Sheet Data:

(in thousands)

	December 31,	
	2017	2016
Cash	$ 887	$ —
Working capital deficit	(1,447)	(329)
Total assets	1,355	7
Total liabilities	2,337	331
Additional paid-in-capital	3,468	—
Total net Parent investment	—	(324)
Accumulated deficit	(4,450)	—
Total liabilities and stockholders' deficit/net Parent investment	$ 1,355	$ 7

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MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND PLAN OF OPERATIONS

You should read the following discussion and analysis of our financial condition and plan of operations together with "Selected Financial Data" and our financial statements and the related notes appearing elsewhere in this prospectus. In addition to historical information, this discussion and analysis contains forward-looking statements that involve risks, uncertainties and assumptions. Our actual results may differ materially from those discussed below. Factors that could cause or contribute to such differences include, but are not limited to, those identified below, and those discussed in the section titled "Risk Factors" included elsewhere in this prospectus. All amounts in this report are in U.S. dollars, unless otherwise noted.

Overview

We are a clinical stage biopharmaceutical company focused on drug development that utilizes novel artificial intelligence, or AI, to identify the next wave of medicines across neuroscience and immuno-oncology. Our drug re-innovation approach leverages existing approved drugs and/or clinically validated product candidates together with big data and proprietary machine learning algorithms to identify new therapeutic indices. We believe that this differentiated approach has the potential to reduce the cost and time of drug development in diseases with substantial unmet medical need. Our two most advanced clinical development programs are BXCL501, a sublingual thin film formulation of the α_{2a} adrenergic receptor agonist dexmedetomidine, or Dex, for acute treatment of agitation resulting from neurological and psychiatric disorders, and BXCL701, an immuno-oncology agent for treatment of a rare form of prostate cancer and pancreatic cancer. We initiated a Phase 1b pharmacokinetic/pharmacodynamic, or PK/PD, safety study using the IV formulation of Dex in mild probable AD in December 2017 and we plan to initiate a Phase 1b PK/PD safety study using the IV formulation of Dex in schizophrenia patients in the first half of 2018. We expect to report data from both studies by the second half of 2018. We also intend to commence Phase 2 proof of concept, or PoC, open label clinical trials in 2018 for both programs. We expect that a data readout from the planned Phase 2 PoC open label clinical trials for the BXCL501 program will be available by the end of 2018. We intend to initiate a bridging bioavailability, or BA, and bioequivalence, or BE, study for the sublingual thin film formulation in the second half of 2018 that, if successful, could potentially lead to the start of a registration trial in the first half of 2019. Preliminary data from the planned Phase 2 PoC clinical trials of BXCL701 will be available in the first half of 2019. We also acquired the rights to two other product candidates, BXCL502 and BXCL702, which together with BXCL501 and BXCL701 collectively represent the "BTI Business."

We were formed to develop first-in-class, high value therapeutics by leveraging EvolverAI, a research and development engine created and owned by our parent, BioXcel Corporation, or BioXcel. We believe the combination of our therapeutic area expertise, our ability to generate product candidates through our exclusive collaborative relationship with BioXcel in the areas of neuroscience and immuno-oncology gives us a significant competitive advantage. EvolverAI was developed over the last decade and integrates millions of fragmented data points using artificial intelligence and proprietary machine learning algorithms. After evaluating multiple product candidates using EvolverAI, we selected our lead programs because our analysis indicated these drugs may have utility in new therapeutic indices where there is substantial unmet medical needs and limited competition. By focusing on clinical candidates with relevant human data, we believe our approach will help us design more efficient clinical trials, thereby accelerating our product candidates time to market. We retain global development and commercialization rights to these two programs.

To date, we have not generated any revenue, we have incurred net losses and all of our operations have been financed by BioXcel and sales of our common stock. Our net losses were approximately $4.5 million and $2.1 million for the years ended December 31, 2017 and 2016, respectively.

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Our net losses have resulted from costs incurred in developing the drugs in our pipeline, planning, preparing and conducting clinical trials and general and administrative activities associated with our operations. We expect to continue to incur significant expenses and corresponding increased operating losses for the foreseeable future as we continue to develop our pipeline. Our costs may further increase as we conduct clinical trials and seek regulatory approval for and prepare to commercialize our candidates. We expect to incur significant expenses to continue to build the infrastructure necessary to support our expanded operations, clinical trials, commercialization, including manufacturing, marketing, sales and distribution functions. We will also experience increased costs associated with operating as an independent entity and a public company.

We were incorporated on March 29, 2017 as a wholly-owned subsidiary of BioXcel and our operating activities have been funded by BioXcel since January 1, 2015. We have adopted a calendar year-end for reporting purposes.

Relationship with BioXcel

We have entered into an asset contribution agreement, effective June 30, 2017, with BioXcel, as amended and restated on November 7, 2017, or the Contribution Agreement, pursuant to which BioXcel agreed to contribute to us, and we agreed to acquire from BioXcel, all of BioXcel's rights, title and interest in and to BXCL501, BXCL701, BXCL502 and BXCL702, collectively, the Candidates, and all of the assets and liabilities associated with the Candidates, in consideration for (i) 9,480,000 shares of our common stock, (ii) $1 million upon completion of this offering, (iii) $500,000 upon the later of the 12 month anniversary of this offering and the first dosing of a patient in the bridging bioavailability/bioequivalence study for the BXCL501 program, (iv) $500,000 upon the later of the 12 month anniversary of this offering and the first dosing of a patient in the Phase 2 PoC open label monotherapy or combination trial with Keytruda for the BXCL701 program and (v) a one-time payment of $5 million within 60 days after the achievement of $50 million in cumulative net sales of any product or combination of products resulting from the development and commercialization of any one of the Candidates or a product derived therefrom. In addition, pursuant to the Contribution Agreement, upon completion of this offering, BioXcel will grant us a first right to negotiate exclusive rights to any additional product candidates in the fields of neuroscience and immuno-oncology that BioXcel may identify on its own, excluding the Candidates, and not in connection with BioXcel's provision of services to us under the Services Agreement as defined and described below. This option for first negotiation shall be valid for a period of five years from the date of this offering. See the section titled "Certain Relationships and Related Person Transactions—Amended and Restated Asset Contribution Agreement with BioXcel" for additional information.

We have entered into a separation and shared services agreement with BioXcel that took effect on June 30, 2017, as amended and restated on November 7, 2017, or the Services Agreement, pursuant to which BioXcel will allow us to continue to use the office space, equipment, services and leased employees based on the agreed upon terms and conditions for a payment of defined monthly and/or hourly fees. The parties have agreed that the services and office space provided under the Services Agreement shall decrease over time until the 12 month anniversary of the date of the Services Agreement, except for services to be provided by BioXcel through its subsidiary in India, which shall decrease until the 24 to 36 month anniversary of the date of the Services Agreement, provided such dates may be extended upon mutual agreement between the parties. On or before December 31, 2019, we shall have the option to enter into a collaborative services agreement with BioXcel pursuant to which BioXcel shall perform product identification and related services for us utilizing EvolverAI. We have agreed that this agreement will be negotiated in good faith and that such agreement will incorporate reasonable market-based terms, including consideration for BioXcel reflecting a low, single-digit royalty on net sales and reasonable development and commercialization milestone payments, provided that (i) development milestones shall not exceed $10 million in the aggregate and not be

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payable prior to proof of concept in humans and (ii) commercialization milestones shall be based on reaching annual net sales levels, be limited to 3% of the applicable net sales level, and not exceed $30 million in the aggregate. BioXcel shall continue to make such product identification and related services available to us for at least five years from June 30, 2017. See the section titled "Certain Relationships and Related Person Transactions—Amended and Restated Separation and Shared Services Agreement with BioXcel" for additional information.

In connection with the Services Agreement, BioXcel agreed to provide us a line of credit, which shall be capped at $1 million, or the Total Funding Amount, pursuant to the terms of a grid note, or the Grid Note. The Grid Note shall be payable upon the earlier of (i) the completion of this offering and (ii) December 31, 2018, together with interest on the unpaid balance of each advance made under the Grid Note, which shall accrue at a rate per annum equal to the applicable federal rate for short-term loans as of the date hereof, in each case calculated based on a 365-day year and actual days elapsed. As of December 31, 2017, we have drawn $371,000 under the Grid Note. See the section titled "Certain Relationships and Related Person Transactions—Amended and Restated Separation and Shared Services Agreement with BioXcel" for additional information.

For the period March 29, 2017 through June 30, 2017 BioXcel paid for expenses on our behalf totaling approximately $562,000 of which $122,000 has been repaid as of December 31, 2017. We have agreed to reimburse BioXcel for this amount upon the earlier of (i) 30 days after the completion of this offering and (ii) December 31, 2018. See the section titled "Certain Relationships and Related Person Transactions—Amended and Restated Separation and Shared Services Agreement with BioXcel" for additional information.

Basis of Presentation

For periods prior to incorporation and through June 30, 2017, our financial statements are presented on a carve-out basis from the financial records of BioXcel. The carve-out includes reasonable allocations of assets and liabilities and expenses attributable to our business. For all periods after June 30, 2017, the allocations of assets, liabilities and expenses attributable to our business shall be made at prevailing prices pursuant to the terms of the Services Agreement, as described below.

These results reflect amounts specifically attributable to the BTI Business, which include expenses, assets and liabilities of BioXcel relating to the Candidates that were contributed to us by BioXcel under the Contribution Agreement for the period from January 1, 2015 until March 29, 2017 (date of incorporation) and further until June 30, 2017. The Services Agreement provides us with certain general and administrative and development support services that became effective June 30, 2017. However, consistent with accounting regulations, we have assumed that we were a separate business within BioXcel and we have reflected the related assets, liabilities and expenses in our results for periods prior to and post incorporation. These financial statements are presented on a carve-out basis and have been derived from the financial statements and accounting records of BioXcel and include reasonable allocations for assets and liabilities and expenses attributable to the business of the product candidates that were contributed.

Management believes the assumptions underlying the allocations of indirect expenses in the carve-out financial information are reasonable, however, our financial position, results of operations and cash flows may have been materially different if it had operated as a stand-alone entity as of and for the fiscal years ended December 31, 2017 and 2016. For the year ended December 31, 2017 results include carve-out amounts from our parent for the period January 1, 2017 through June 30, 2017 and as a standalone entity for the period July 1, 2017 through December 31, 2017.

We have calculated our income tax amounts using a separate return methodology and we have presented these amounts as if we were a separate taxpayer from BioXcel for the period since the date of incorporation (March 29, 2017). BioXcel is a standalone S corporation and its tax obligations were

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passed through to its shareholders and were not a liability of the S corporation. As a result, BioXcel did not require a tax provision for federal or state purposes. Therefore no taxes have been allocated to the financials of the Company which is derived from a carve-out process from the financials of BioXcel. Pursuant to our incorporation as a C corporation, BioXcel became our sole owner and contributed the BTI Business in a tax free transaction. From the date of incorporation, we have been a standalone C corporation subject to corporate income tax and the deferred tax and assets have been calculated accordingly.

We consider our expense methodology and results to be reasonable for all periods we present. However, our allocations may not be indicative of the actual expenses we would have incurred had we operated as an independent, publicly traded company for the periods we present.

Components of Our Results of Operations

Revenues

We have not recognized any revenue since inception.

Operating Costs and Expenses

Research and Development

Research and development expenses consist primarily of costs incurred for the research and development of our preclinical and clinical candidates, which include:

- employee-related expenses, including salaries, benefits and stock-based compensation expense;
- expenses incurred towards consultants, laboratories and investigators that conduct our preclinical or clinical research activities;
- the cost of acquiring, developing and manufacturing pre-clinical trial materials and lab supplies; and
- depreciation and other expenses.

We expense research and development costs to operations as incurred. Historically we have not segmented costs associated with our various development programs. The carve-out financials represent the business involving the BTI Business. However, beginning January 1, 2018, we will assign costs to our individual development candidates.

As of December 31, 2017, we had incurred an aggregate of approximately $4.3 million in research and development expenses related to the development of BXCL501 and BXCL701. We expect that our research and development expenses will increase as we plan for and commence our clinical trials of BXCL501, which we expect to accelerate in the first half of 2018, and BXCL701, which we also expect to commence in the first half of 2018.

Because of the numerous risks and uncertainties associated with product development, we cannot determine with certainty the duration and completion costs of these or other current or future clinical trials of BXCL501, BXCL701 or our other product candidates. We may never succeed in achieving regulatory approval for BXCL501, BXCL701 or any of our other product candidates. The duration, costs and timing of clinical trials and development of our product candidates will depend on a variety of factors, including the uncertainties of future clinical and preclinical studies, uncertainties in clinical trial enrollment rate and significant and changing government regulation. In addition, the probability of success for each product candidate will depend on numerous factors, including competition, manufacturing capability and commercial viability.

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General and Administrative

General and administrative expenses consist primarily of salaries and related costs for employees in executive, finance and administration, corporate development and administrative support functions, including stock-based compensation expenses and benefits. Other significant general and administrative expenses include accounting and legal services, the cost of various consultants, occupancy costs and information systems costs.

We expect that our general and administrative expenses will increase as we operate both as an independent entity and as a public company. We expect increased administrative costs resulting from our anticipated clinical trials and the potential commercialization of our product candidates. We believe that these increases will likely include increased costs for director and officer liability insurance, hiring additional personnel to support future market research and future product commercialization efforts and increased fees for outside consultants, attorneys and accountants. We also expect to incur increased costs to comply with corporate governance, internal controls, investor relations and disclosures and similar requirements applicable to public companies.

Financial Operations Overview and Analysis for the Years Ended December 31, 2017 and 2016

(in thousands, except percentages)	Years Ended December 31, 2017	2016	Increase (Decrease) $	%
Revenues	$ —	$ —	$ —	
Operating costs and expenses				
Research and development	2,690	1,399	1,291	92%
General and administrative	1,847	721	1,126	156%
Total operating expenses	4,537	2,120	2,417	
Loss from operations	(4,537)	(2,120)	(2,417)	
Other expense				
Interest expense	(2)	—	(2)	
Net loss	$ (4,539)	$ (2,120)	$ (2,419)	

Research and Development Expense

Research and development expenses increased approximately $1.3 million, or 92%, from $1.4 million for the year ended December 31, 2016 to $2.7 million for the year ended December 31, 2017. The increase was primarily due to the increase in drug development expenses of $840,000, from $357,000 for the year ended December 31, 2016 to $1.2 million for the year ended December 31, 2017, which included material costs, clinical trial expenses and consulting fees for therapeutic area experts. Non-cash stock-based compensation charges increased by $416,000, from $528,000 to $944,000 mainly from the charges pertaining to our 2017 Equity Incentive Plan over and above the stock-based compensation costs transferred to us by our Parent. Compensation expenses also increased by $35,000 from $511,000 for the year ended December 31, 2016 to $546,000 for the year ended December 31, 2017.

General and Administrative Expense

General and administrative expenses increased approximately $1.1 million or 156%, from $721,000 for the year ended December 31, 2016 to $1.8 million for the year ended December 31, 2017. The increase was primarily attributable to an increase in employee compensation of $198,000 mainly from

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additional administrative time allocated to us by BioXcel to support our increased business activity in the first half of the year and salary costs for our executives during the second half of the year. There was also an increase in non-cash stock-based compensation of $520,000, from $143,000 for the year ended December 31, 2016 to $663,000 for the year ended December 31, 2017, which was mainly from the charges pertaining to our 2017 Equity Incentive Plan over and above the stock-based compensation costs transferred to us by our Parent. In addition, we incurred increases in travel, professional and consultants fees and other expenses totaling $408,000.

Liquidity and Capital Resources

We reported losses of approximately $4.5 and $2.1 million for the years ended December 31, 2017 and 2016 respectively. At December 31, 2017, our accumulated deficit amounted to approximately $4.5 million. We had a working capital deficit of approximately $1.4 million as of December 31, 2017.

As of December 31, 2017, we had cash and cash equivalents of $887,000.

We have not yet generated any revenues and we have not yet achieved profitability. These conditions raise substantial doubt about our ability to continue as a going concern. We expect that our research and development and general and administrative expenses will continue to increase and, as a result, we will need to generate significant product revenues to achieve profitability. We may never achieve profitability.

Sources of Liquidity

Since our inception, until the date of incorporation, all our operations have been financed by our Parent, BioXcel, in the form of net Parent investment. For the period from inception (March 29, 2017) until June 30, 2017 (effective date of the Services Agreement), our operations have been financed through $562,000 (of which $122,000 has been repaid as of December 31, 2017) in advances from BioXcel. Such advances are payable to BioXcel upon the earlier of (i) 30 days after the completion of this offering, (ii) ten days after receiving funding of at least $5,000,000 other than through an IPO and (iii) December 31, 2018. On June 30, 2017, BioXcel agreed to provide us a line of credit of $1 million, pursuant to the terms of the Grid Note. The Grid Note shall be payable upon the earlier of (i) the completion of this offering and (ii) December 31, 2018, together with interest on the unpaid balance of each advance made under the Grid Note, which shall accrue at a rate per annum equal to the applicable federal rate for short-term loans as of the date hereof, in each case calculated based on a 365-day year and actual days elapsed. As of December 31, 2017, we have drawn an amount of $371,000 under the Grid Note.

Our cash and cash equivalents as of December 31, 2017 do not reflect proceeds from the issuance of common shares amounting to $1.95 million in January 2018 and February 2018.

Cash Flows

	Years Ended December 31,	
(in thousands)	**2017**	**2016**
Cash provided by (used in) in thousands		
Operating activities	$ (2,196)	$ (1,294)
Investing activities	—	(4)
Financing activities	3,083	1,298

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Operating Activities

For the year ended December 31, 2017, net cash used in operating activities was approximately $2.2 million, which consisted of a net loss of $4.5 million partially offset by an increase of $1.6 million in stock-based compensation and an increase in accounts payable and accrued expenses of $737,000.

For the year ended December 31, 2016, net cash used in operating activities was approximately $1.3 million, which consisted of a net loss of $2.1 million partially offset by an increase of $671,000 in stock-based compensation and an increase in accounts payables and accrued expenses of $156,000.

Investing Activities

There were no investing activities in the year ended December 31, 2017 compared to cash used in the purchase of computer equipment for $4,000 during the year ended December 31, 2016.

Financing Activities

The net cash provided by financing activities was approximately $3.1 million during the year ended December 31, 2017 which was attributable to the investment made by BioXcel prior to our incorporation of $214,000, a loan due to BioXcel of $67,000 for expenses from the date of incorporation to December 31, 2017, $440,000 of services provided by BioXcel for the six months ending December 31, 2017 and $371,000 drawn by us from the line of credit from BioXcel. In addition, we sold 1,804 shares of common stock for approximately $2.1 million in proceeds. This was partially offset by an increase of deferred offering expenses of $70,000.

Net cash provided by financing activities for the year ended December 31, 2016 was approximately $1.3 million, which was attributable to investments made by BioXcel.

Operating Capital and Capital Expenditure Requirements

We believe that the net proceeds of this offering, together with our existing cash, will be sufficient to fund our operations for at least twelve months from the date of this prospectus. We are required to repay the amounts due to BioXcel from the proceeds of this offering for the amounts borrowed under the Grid Note and the amounts due to BioXcel pursuant to the Services Agreement.

We expect to continue to incur significant and increasing operating losses at least for the next several years as we commence our clinical trials of BXCL501 and BXCL701, seek marketing approval for our product candidates and pursue development of our other product candidates. We do not expect to generate revenue unless and until we successfully complete development and obtain regulatory approval for our product candidates. Our net losses may fluctuate significantly from quarter-to-quarter and year-to-year, depending on the timing of our planned clinical trials and our expenditures on other research and development activities.

We have based our projections of operating capital requirements on assumptions that may prove to be incorrect and we may use all of our available capital resources sooner than we expect. Because of the numerous risks and uncertainties associated with research, development and commercialization of pharmaceutical products, we are unable to estimate the exact amount of our operating capital requirements. We anticipate that our expenses will increase substantially as we:

- commence our clinical development of BXCL501 and BXCL701;
- conduct additional research and development with our product candidates;
- seek to identify, acquire, develop and commercialize additional product candidates;
- integrate acquired technologies into a comprehensive regulatory and product development strategy;

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- maintain, expand and protect our intellectual property portfolio;
- hire scientific, clinical, quality control and administrative personnel;
- add operational, financial and management information systems and personnel, including personnel to support our drug development efforts;
- seek regulatory approvals for any product candidates that successfully complete clinical trials;
- ultimately establish a sales, marketing and distribution infrastructure and scale up external manufacturing capabilities to commercialize any product candidates for which we may obtain regulatory approval; and
- begin to operate as a public company.

We expect that we will need to obtain substantial additional funding in order to complete our clinical trials. To the extent that we raise additional capital through the sale of common stock, convertible securities or other equity securities, the ownership interests of our existing stockholders may be materially diluted and the terms of these securities could include liquidation or other preferences that could adversely affect the rights of our existing stockholders. In addition, debt financing, if available, would result in increased fixed payment obligations and may involve agreements that include restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends, that could adversely impact our ability to conduct our business. If we are unable to raise capital when needed or on attractive terms, we could be forced to significantly delay, scale back or discontinue the development or commercialization of BXCL501, BXCL701 or other product candidates, seek collaborators at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available, and relinquish or license, potentially on unfavorable terms, our rights to BXCL501, BXCL701 or other product candidates that we otherwise would seek to develop or commercialize ourselves.

Contractual Obligations

On December 7, 2017 we entered into a contract with a clinical research organization for our first human clinical trial in BXCL 501. The contract will total approximately $1 million.

Off-Balance Sheet Arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements as defined under Securities and Exchange Commission rules.

Critical Accounting Policies

The preparation of our financial statements in conformity with accounting principles generally accepted in the United States requires management to exercise its judgment. We exercise considerable judgment with respect to establishing sound accounting policies and in making estimates and assumptions that affect the reported amounts of our assets and liabilities, our recognition of revenues and expenses, and disclosure of commitments and contingencies at the date of the financial statements.

On an ongoing basis, we evaluate our estimates and judgments. We base our estimates and judgments on a variety of factors including our historical experience, knowledge of our business and industry, current and expected economic conditions, the attributes of our products, the regulatory environment, and in certain cases, the results of outside appraisals. We periodically re-evaluate our estimates and assumptions with respect to these judgments and modify our approach when circumstances indicate that modifications are necessary.

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While we believe that the factors we evaluate provide us with a meaningful basis for establishing and applying sound accounting policies, we cannot guarantee that the results will always be accurate. Since the determination of these estimates requires the exercise of judgment, actual results could differ from such estimates.

A description of significant accounting policies that require us to make estimates and assumptions in the preparation of our financial statements is as follows:

Use of Estimates

The preparation of financial statements in conformity with accounting principles generally accepted in the United States of America requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosures of contingent liabilities at the dates of the financial statements and the reported amounts of revenues and expenses during the reporting periods. Actual results could differ from these estimates.

For periods prior to and post incorporation, these financial statements are presented on a carve-out basis and include the financial statements of the Company and financial information derived from the financial statements and accounting records of BioXcel which include reasonable allocations for assets and liabilities and expenses attributable to the BTI Business of product candidates that were contributed.

Accordingly, the historical financial information for the fiscal years ended December 31, 2016 and for the six months ended June 30, 2017 have been carved-out of the financial statements of BioXcel. Such financial information is limited to our business activities, assets and liabilities only. The financial information for the period beginning July 1, 2017 through December 31, 2017 have been prepared as a standalone entity.

BioXcel recorded such product candidates at a zero-historical cost basis, and therefore they are recorded at a zero basis on our books. The historical financial statements have been presented on a basis that includes the results attributable to the business contributed from BioXcel as if we owned the business for all periods presented.

Research and Development

Research and development expenses are expensed as incurred. Patent costs and patent acquisition costs are expensed as incurred, and included in general and administrative expenses.

Stock-based Compensation

Charges from our Parent BioXcel Corporation.

The financial statements include certain expenses of our parent, BioXcel, including stock-based compensation expense that were carved-out of the historical financial statements of BioXcel based on the percentage of the expense attributable to BTI related activities.

BioXcel has granted stock options to its employees under its own equity incentive plan, or the BioXcel Plan. Stock-based compensation expense from awards granted under the BioXcel Plan is allocated to BTI over the required service period over which those stock option awards vest, and is based upon the percentage of time the award recipient spent working on our activities compared to BioXcel activities, which is the same basis used for allocation of salary costs.

The BioXcel stock option awards are valued at fair value on the date of grant and that fair value is recognized over the requisite service period. The estimated fair value of these BioXcel stock option awards was determined using the Black Scholes option pricing model on the date of grant. Stock based awards to non-employees are remeasured at fair value each financial reporting date until vesting is

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complete. Significant judgment and estimates were used to estimate the fair value of these awards, as they are not publicly traded.

Our estimation of fair value of the awards considered recent transactions entered into by BioXcel, relevant industry and comparable public company data. Since BioXcel is a non-public entity, the majority of the inputs used to estimate the fair value of the common stock option awards are considered level 3 due to their unobservable nature. Each option award is subject to specified vesting schedules and requirements (a mix of time-based, and corporate event-based, including financing events). Compensation expense is charged to us by BioXcel over the required service period to earn the award which is expected to be up to four years, subject to the achievement of time and event-based vesting requirements. For the years ended December 31, 2017 and 2016 we have incurred share-based compensation expense related to equity awards granted by BioXcel totaling $439,000 and $671,000, respectively. We have recorded these charges as research and development and general and administrative expense in our statement of operations.

BioXcel Therapeutics, Inc. 2017 Equity Incentive Plan

Our board of directors adopted the 2017 Equity Incentive Plan, or the Plan, on August 22, 2017. The Plan will expire on August 22, 2027. The purpose of the Plan is to attract and retain key personnel and to provide a means for directors, officers, managers, employees, consultants and advisors to acquire and maintain an interest in our company, which interest may be measured by reference to the value of its common stock. The details of the Plan are explained in the section titled "Executive and Director Compensation."

We account for stock-based compensation in accordance with ASC 718, "Compensation—Stock Compensation," which requires the measurement and recognition of compensation expense based on estimated fair market values for all share-based awards made to employees and directors, including stock options. Stock-based awards to non-employees are re-measured at fair value each financial reporting date until vesting is complete.

We are required to determine the fair value of equity incentive awards and recognize compensation expense for all equity incentive awards, including employee stock options. We recognize this expense over the requisite service period in the statement of operations. We have adopted FASB ASU 2016-09 and account for forfeitures as they occur, by reversing compensation cost for the unvested portion of an award when the award is forfeited. We use the graded attrition method for expense attribution.

The valuation model we used for calculating the fair value of awards for stock-based compensation expense is the Black-Scholes option-pricing model, or the Black-Scholes Model. The Black-Scholes Model requires us to make assumptions and judgments about the variables used in the calculation, including:

- *Expected term.* We do not believe we are able to rely on our historical exercise and post-vesting termination activity to provide accurate data for estimating the expected term for use in determining the fair value-based measurement of our options. Therefore, we have opted to use the "simplified method" for estimating the expected term of options granted to employees, which is the average of the weighted-average vesting period and contractual term of the option. We use the contractual term for non-employee awards.

- *Expected volatility.* Since there has been no public market for our common stock and lack of company specific historical volatility, we have determined the share price volatility for options granted based on an analysis of the volatility of a peer group of publicly traded companies. In evaluating similarity, we consider factors such as stage of development, risk profile, enterprise value and position within the industry.

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- *Risk-free interest rate.* The risk-free interest rate is based on the U.S. Treasury yield in effect at the time of the grant for zero-coupon U.S. Treasury notes with remaining terms similar to the expected term of the options.

- *Dividend rate.* We assumed the expected dividend to be zero as we have never paid dividends and have no current plans to do so.

- *Expected forfeiture rate.* We have adopted FASB ASU 2016-09 and account for forfeitures as they occur, by reversing compensation cost when the award is forfeited.

- *Service period.* We amortize all stock-based compensation over the requisite service period of the awards, which is generally the same as the vesting period of the awards. We amortize the stock-based compensation cost on graded attrition basis over the expected service periods.

- *Fair value of common stock.* As there has been no public market for our common stock to date, the estimated fair value of our common stock has been determined by our board of directors as of the date of each option grant, with input from management, considering our most recently available valuations of common stock as described below and our board of directors' assessment of additional objective and subjective factors that it believed were relevant and which may have changed from the date of the most recent valuation through the date of the grant. Three valuations were performed in accordance with the guidance outlined in the American Institute of Certified Public Accountants' Accounting and Valuation Guide, *Valuation of Privately-Held-Company Equity Securities Issued as Compensation*.

  The initial valuation of our common stock as of June 30, 2017 was prepared using the Option Pricing Method ("OPM"). The OPM treats common stock as call options on the total equity value of a company, with exercise prices based on the value thresholds at which the allocation among the various holders of a company's securities changes. Under this method, the common stock has value only if the funds available for distribution to stockholders exceeded the value of any preferred stock liquidation preferences at the time of the liquidity event, such as a strategic sale or a merger. The future value of the common stock under the OPM outcome is discounted back to the valuation date at an appropriate risk-adjusted discount rate and probability weighted to arrive at an indication of value for the common stock. The value of our common stock as of June 30, 2017 was estimated at $0.41 per share.

  We valued our common stock as of September 30, 2017 utilizing the hybrid method. The hybrid method uses a market approach to estimate our enterprise value. The hybrid method is a probability-weighed expected return method, or PWERM, where the equity value in one or more of the scenarios is calculated using OPM. The PWERM is a scenario-based methodology that estimates the fair value of common stock based upon an analysis of future values for the company, assuming various outcomes. The common stock value is based on the probability-weighted present value of expected future investment returns considering each of the possible outcomes available as well as the rights of all stock holders. The future value of the common stock under each outcome is discounted back to the valuation date at an appropriate risk-adjusted discount rate and probability weighted to arrive at an indication of value for the common stock. The value of our common stock at September 30, 2017 was estimated at $5.55 per share. Our initial clinical trial of BXCL501 for mild-probable AD was granted an IND exemption by the FDA on September 25, 2017. As a result, we believe development timeline for this product candidate will be shortened. Based this exemption, we initiated a Phase 1b PK/PD safety study using the IV formulation of Dex in mild probable Alzheimer's Disease, or AD, in December 2017. We believe this exemption was a significant event in our history and a major factor in the increase in our valuation between June 30, 2017 and September 30, 2017.

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We valued our common stock on December 31, 2017 also utilizing the hybrid method by updating the assumptions and facts used in our September 30, 2017 valuation. The value of our common stock at December 31, 2017 was estimated at $8.30 per share. During the three months ended December 31, 2017 we received Institutional Review Board ("IRB") approval and dosed our first patient in our Phase 1b PK/PD safety study using the IV formulation of Dex in mild probable AD patients. We believe the start of our human clinical trials is a significant event in our history and a factor in the increase in our valuation between September 30, 2017 and December 31, 2017.

In addition to considering the results of these valuations, our board of directors considered various objective and subjective factors to determine the fair value of our common stock as of each grant date, which may be a date later than the valuation dates, noted above including:

- sales of our common stock in any arms-length transactions;
- the progress of our research and development programs, including the status of preclinical studies and planned clinical trials for our product candidates;
- our stage of development and commercialization and our business strategy;
- external market conditions affecting the pharmaceutical and biotechnology industries, and trends within the biotechnology industry;
- our financial position, including cash on hand, and our forecasted performance and operating results;
- the lack of an active public market for our common stock;
- the likelihood of achieving a liquidity event, such as an initial public offering, or IPO, or a sale of our company in light of prevailing market conditions; and
- the analysis of IPOs and the market performance of similar companies in the biopharmaceutical industry.

The assumptions underlying these valuations represent management's best estimates, which involve inherent uncertainties and the application of management judgment. As a result, if factors or expected outcomes change and we use significantly different assumptions or estimates, our stock-based compensation expense could be materially different.

Options Granted

The following table sets forth by grant date the number of shares subject to options granted between June 30, 2017 and December 31, 2017, the per share exercise price of the options, the fair value of common stock per share on each grant date, and the per share estimated fair value of the options:

Grant Date	Number of Shares Subject to Options Granted	Per Share Exercise Price of Options	Fair Value of Common Stock Per Share on Grant Date	Per Share Estimated Fair Value of Options
August 23, 2017	2,197,227	$ 0.41	$ 0.41	$ 0.26 - 0.29
September 15, 2017	28,914	$ 0.41	$ 0.41	$ 0.27
October 2, 2017	83,898	$ 5.55	$ 5.55	$ 3.62 - $3.95

For stock awards after the completion of this offering, our board of directors intends to determine the fair value of each share of underlying common stock based on the closing price of our common stock as reported on the date of grant.

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The intrinsic value of all outstanding options as of December 31, 2017 was 26.34 million based on the estimated fair value of our common stock of $12.00 per share, which is the assumed initial public offering price per share of our common stock based on the midpoint of the price range set forth on the cover page of this prospectus.

Share based compensation charges related to our 2017 Equity Incentive Plan totaled $1.2 million for the year ending December 31, 2017. There were no corresponding charges for the year ending December 31, 2017 as the plan did not exist.

Total share based compensation charges including the charges from BioXcel's plan for the years ending December 31, 2017 and 2016 total $1,606,000 and $671,000 and respectively.

If factors change and we employ different assumptions, stock-based compensation expense may differ significantly from what we have recorded in the past. If there are any modifications or cancellations of the underlying unvested securities, we may be required to accelerate, increase or cancel any remaining unearned stock-based compensation expense. To the extent that our assumptions are incorrect, the amount of stock-based compensation recorded will change.

Recent Accounting Pronouncements

In May 2014, the Financial Accounting Standards Board, or FASB, issued *ASU 2014-09 Revenue form Contracts with Customers.* Under this guidance on the recognition of revenue from customers. Under this guidance, an entity will recognize revenue when it transfers promised goods or services to customers in an amount that reflects what the entity expects to receive in exchange for the goods or services. This new guidance also requires more detailed disclosures to enable users of the financial statements to understand the nature, amount, timing and uncertainty of revenue and cash flows arising from contracts with customers. The Company will adopt this guidance beginning on January 1, 2018. The guidance allows selection one of two methods of adoption, either the full retrospective approach, meaning the guidance would be applied to all periods presented, or modified retrospective approach, meaning the cumulative effect of applying the guidance would be recognized as an adjustment to opening accumulated deficit balance. Since the Company has no revenue to date, the Company does not believe the adoption of ASU 2014-09 will have a material impact on its financial statements.

In August 2014, the FASB issued *ASU 2014-15 Disclosures of Uncertainties around an Entity's Ability to Continue as a Going Concern.* This ASU requires management to determine whether substantial doubt exists regarding the entity's going concern presumption, which generally refers to an entity's ability to meets its obligations as they become due. If substantial doubt exists but is not alleviated by management's plan, the footnotes must specifically state that "there is substantial doubt about the entity's ability to continue as a going concern within one year after the financial statements are issued." In addition, if substantial doubt exists, regardless of whether such doubt was alleviated, entities must disclose (a) principal conditions or events that raise substantial doubt about the entity's ability to continue as a going concern (before consideration of management's plans, if any); (b) management's evaluation of the significance of those conditions or events in relation to the entity's ability to meet its obligations; and (c) management's plans that are intended to mitigate the conditions or events that raise substantial doubt, or that did alleviate substantial doubt, about the entity's ability to continue as a going concern. If substantial doubt has not been alleviated, these disclosures should become more extensive in subsequent reporting periods as additional information becomes available. In the period that substantial doubt no longer exists (before or after considering management's plans), management should disclose how the principal conditions and events that originally gave rise to substantial doubt have been resolved. The Company has adopted the provisions of ASU 2014-15 beginning January 1, 2016.

In February 2016, the FASB issued *ASU 2016-02 Lease Accounting Topic 842*. This ASU requires us to record all leases longer than one year on our balance sheet. Under the new guidance, when the

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Company records leases on its balance sheet under it will record a liability with a value equal to the present value of payments it will make over the life of the lease and an asset representing the underlying leased asset. The new accounting guidance requires the Company to determine if its leases are operating or financing leases, similar to current accounting guidance. The Company will record expense for operating type leases on a straight-line basis as an operating expense and it will record expense for finance type leases as interest expense. The new lease standard is effective for annual and interim periods beginning after December 15, 2018, with early adoption permitted. The Company must adopt the new standard on a modified retrospective basis, which requires it to reflect its leases on its balance sheet for the earliest comparative period presented. The Company is currently assessing the timing of adoption as well as the effects it will have on its financial statements and disclosures.

In March 2016, the FASB ASU 2016-09, *Compensation- Stock Compensation* simplifying certain aspects of share-based payment accounting. Under the amended guidance, the Company will recognize excess tax benefits and tax deficiencies as income tax expense or benefit in its statement of operations on a prospective basis. As the Company has a valuation allowance, this change will impact the Company's net operating loss carryforward and the valuation allowance disclosures. Additionally, the Company will classify excess tax benefits as an operating activity and classify amounts the Company withholds in shares for the payment of employee taxes as a financing activity on the statement of cash flows for each period presented. The amended guidance allows the Company to account for forfeitures when they occur or continue to estimate them. The Company will continue to estimate its forfeitures. The Company adopted this guidance on January 1, 2017. The amended guidance did not impact its financial results.

The SEC staff issued Staff Accounting Bulletin ("SAB") 118, which provides guidance on accounting for the tax effects of the U.S. tax reform announced on December 22, 2017 by the U.S. Government commonly referred to as the Tax Cuts and Jobs Act. SAB 118 provides a measurement period that should not extend beyond one year from the U.S. tax reform enactment date for companies to complete the accounting under Accounting Standards Codification ("ASC") 740. In accordance with SAB 118, a company must reflect the income tax effects of those aspects of the U.S. tax reform for which the accounting under ASC 740 is complete. Specifically, the Company will be required to revalue its U.S. deferred tax assets and liabilities due to the federal income tax rate reduction from 35 percent to 21 percent. Since the Company has provided a full valuation allowance against its deferred tax assets, the revaluation of the deferred tax assets did not have a material impact on any period presented.

Quantitative and Qualitative Disclosure About Market Risk

Our primary exposure to market risk is interest expense sensitivity, which is affected by changes in the general level of U.S. interest rates. Due to the short-term duration of Grid Note payable, we would not expect our operating results or cash flows to be affected to any significant degree by the effect of a sudden change in market interest rates on our note payable.

We do not believe that our cash has significant risk of default or illiquidity. While we believe our cash does not contain excessive risk, we cannot provide absolute assurance that in the future our investments will not be subject to adverse changes in market value. In addition, we maintain significant amounts of cash at one or more financial institutions that are in excess of federally insured limits.

Our balance sheet as of December 31, 2017 includes cash of $887,000. We do not participate in any foreign currency hedging activities and we do not have any other derivative financial instruments. We did not recognize any significant exchange rate losses during the years ended December 31, 2017 and 2016 respectively.

Inflation generally affects us by increasing our cost of labor and clinical trial costs. We do not believe that inflation has had a material effect on our results of operations during the periods presented.

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JOBS Act

On April 5, 2012, the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, was enacted. Section 107 of the JOBS Act provides that an "emerging growth company" can take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act of 1933, as amended, or the Securities Act, for complying with new or revised accounting standards. In other words, an "emerging growth company" can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies.

We have chosen to opt out of the extended transition periods available to emerging growth companies under the JOBS Act for complying with new or revised accounting standards. Section 107 of the JOBS Act provides that our decision to opt out of the extended transition periods for complying with new or revised accounting standards is irrevocable.

We are in the process of evaluating the benefits of relying on other exemptions and reduced reporting requirements provided by the JOBS Act. Subject to certain conditions set forth in the JOBS Act, as an "emerging growth company," we intend to rely on certain of these exemptions, including without limitation, (i) providing an auditor's attestation report on our system of internal controls over financial reporting pursuant to Section 404(b) of the Sarbanes-Oxley Act and (ii) complying with any requirement that may be adopted by the PCAOB regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements, known as the auditor discussion and analysis. We will remain an "emerging growth company" until the earliest of (i) the last day of the fiscal year in which we have total annual gross revenues of $1.07 billion or more; (ii) the last day of our fiscal year following the fifth anniversary of the date of the completion of this offering; (iii) the date on which we have issued more than $1 billion in nonconvertible debt during the previous three years; or (iv) the date on which we are deemed to be a large accelerated filer under the rules of the Securities and Exchange Commission.

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BUSINESS

Overview

BioXcel Therapeutics, Inc., or BTI, is a clinical stage biopharmaceutical company focused on drug development that utilizes novel artificial intelligence, or AI, to identify the next wave of medicines across neuroscience and immuno-oncology. Our drug re-innovation approach leverages existing approved drugs and/or clinically validated product candidates together with big data and proprietary machine learning algorithms to identify new therapeutic indices. We believe that this differentiated approach has the potential to reduce the cost and time of drug development in diseases with substantial unmet medical need. Our two most advanced clinical development programs are BXCL501, a sublingual thin film formulation of the α_{2a} adrenergic receptor agonist dexmedetomidine, or Dex, for acute treatment of agitation resulting from neurological and psychiatric disorders, and BXCL701, an immuno-oncology agent for treatment of a rare form of prostate cancer and pancreatic cancer. We initiated a Phase 1b pharmacokinetic/pharmacodynamic or PK/PD, safety study using the IV formulation of Dex in mild probable AD in December 2017 and we plan to initiate a Phase 1b pharmacokinetic/pharmacodynamic safety study using the IV formulation of Dex in schizophrenia patients in the first half of 2018. We expect to report data from both studies by the second half of 2018. We also intend to commence Phase 2 proof of concept, or PoC, open label clinical trials in 2018 for both programs. We expect that a data readout from the planned Phase 2 PoC open label clinical trials for the BXCL501 program will be available by the end of 2018. We intend to initiate a bridging bioavailability, or BA, and bioequivalence, or BE, study for the sublingual thin film formulation in the second half of 2018 that, if successful, could potentially lead to the start of a registration trial in the first half of 2019. Preliminary data from the planned Phase 2 PoC clinical trials of BXCL701 will be available in the first half of 2019.

We were formed to develop first-in-class, high value therapeutics by leveraging EvolverAI, a research and development engine created and owned by our parent, BioXcel Corporation, or BioXcel. We believe the combination of our therapeutic area expertise and our ability to generate product candidates through our exclusive collaborative relationship with BioXcel in the areas of neuroscience and immuno-oncology gives us a significant competitive advantage. EvolverAI was developed over the last decade and integrates millions of fragmented data points using artificial intelligence and proprietary machine learning algorithms. After evaluating multiple product candidates using EvolverAI, we selected our lead programs because our analysis indicated these drugs may have utility in new therapeutic indices where there is substantial unmet medical needs and limited competition. By focusing on clinical candidates with relevant human data, we believe our approach will help us design more efficient clinical trials, thereby accelerating our product candidates' time to market. We retain global development and commercialization rights to these two programs.

BXCL501 is a potential first-in-class sublingual thin film formulation of Dex designed for acute treatment of agitation in neurodegenerative and psychiatric disorders. Dex has been well tolerated, having been prescribed in millions of patients as the sedative and anesthetic Precedex and has been studied in over 130 clinical trials. BXCL501 is designed to be a non-invasive, easy to administer agent that has a rapid onset of action, which is critical for the acute treatment of agitation. We estimate that over 500,000 patients who suffer from Alzheimer's Disease, or AD, in the United States annually could be eligible for the acute treatment of agitation with BXCL501. In schizophrenia and bipolar disease, we estimate that over 600,000 patients in the United States annually could be eligible for the acute treatment of agitation with BXCL501. The current treatment options for agitation utilize antipsychotics and benzodiazepines, which have suboptimal safety and compliance issues. Antipsychotics have a black box warning for use in the elderly and can produce debilitating side effects when given acutely, and should only be considered for invasive intramuscular, or IM, delivery in highly aggressive patients requiring restraint. Benzodiazepines are predominantly in pill form, which require swallowing and can produce excessive sedation. We have designed a dual clinical development program that takes

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advantage of the U.S. Food and Drug Administration's, or FDA, Section 505(b)(2) regulatory pathway and leverages the existing clinical and safety dataset of intravenous, or IV, formulation of Dex. We plan to initiate two Phase 1b single ascending or descending dose studies of the IV formulation of Dex in mild probable AD by the first half of 2018 and schizophrenia patients in the first half of 2018, followed by PoC open label clinical trials, from both of which we expect to report data by the second half of 2018. We intend to initiate a bridging BA/BE study with the sublingual thin film formulation in the second half of 2018 that, if successful, could potentially lead to the start of a registration trial in the first half of 2019.

BXCL701 is a potential first-in-class, highly potent, oral small molecule immuno-modulator that is designed to stimulate both the innate and acquired immune systems by inhibiting dipeptidyl peptidase, or DPP, 8/9 and fibroblast activation protein, or FAP. DPP 8/9 have been show recently to behave as an "immuno-checkpoint" of the immune system, as their inhibition results in a potent pro-inflammatory, anti-tumor activity by way of the induction of cell death in the macrophages and the downstream stimulation of multiple tumor-killing immune cells. BXCL701 is differentiated among DPP inhibitors because it is designed to inhibit DPP 8/9 and FAP, whereas most other clinical stage DPP inhibitors, which have been developed to treat diabetes, are selective for DPP 4. BXCL701 has been tested in more than 700 healthy subjects and cancer patients across multiple clinical trials, providing evidence of being well tolerated, proof of mechanism, and single agent anti-tumor activity in patients with melanoma, an immuno-sensitive tumor. We believe that we can leverage this clinical data to determine the dose to use in future clinical trials and support accelerated clinical development. BXCL701 is a potential novel therapy for treatment-emergent neuroendocrine prostate cancer, or tNEPC, a segment of prostate cancer patients that have progressed on second-generation androgen inhibitors (Zytiga and Xtandi), and is also a potential treatment for pancreatic cancer, both of which are rare diseases. We selected tNEPC and pancreatic cancer as our lead indications after evaluating more than 100 different tumor types because they are two of the top three cancers that overexpressed or amplified DPP 8/9 and FAP. Additional data points to a functional role of DPP 8/9 in the biology of tNEPC. Approximately one in three patients treated with Zytiga and Xtandi are expected to develop tNEPC based on information in an article published in the Journal of the National Comprehensive Cancer Network in 2014 by Agarwal et. al. and an article published by the Journal of Clinical Oncology in 2014 by Wang et. al. and become eligible for treatment with BXCL701, which we believe can be an available treatment option that can be used after these patients are not responding to further treatment with these two drugs. The combined global sales of Zytiga and Xtandi, which are only approved for prostate cancer treatment, were over $4.5 billion in 2016 and management believes such sales number gives a perspective of the potential market for BXCL701 in this indication, which would be comprised of the approximate one-third of the patients treated with Zytiga and Xtandi. In pancreatic cancer, we estimate that approximately 20,000 patients will be eligible for treatment with BXCL701 annually as about 50% of pancreatic cancer patients can receive 2nd line therapy based on information in an article published in the Annals of Oncology in 2013 by Rahma et al. Based on our analysis, we believe that BXCL701 may establish a differentiated immuno-oncology platform by modulating multiple steps in the cancer immunity cycle, and in combination with checkpoint inhibitors can convert immuno-resistant tumors to immuno-sensitive tumors ("cold" to "hot" tumors). We plan to initiate two Phase 2 PoC open label clinical trials in the second half of 2018, as a single agent and in combination with Keytruda in patients with tNEPC, and in combination with Keytruda in pancreatic cancer. We expect to receive preliminary data in the first half of 2019 and intend to pursue breakthrough therapy designation and accelerated approval pathways for both indications. BXCL701 has received orphan drug designation by the FDA for the treatment of pancreatic cancer.

Furthermore, we are growing our pipeline with additional development candidates by leveraging our management team's therapeutic area expertise with EvolverAI. We are also exploring development of BXCL502, a novel approach to the treatment of symptoms resulting from neurological disorders, and BXCL702, an immuno-oncology agent targeting hematological malignancies for which we have received

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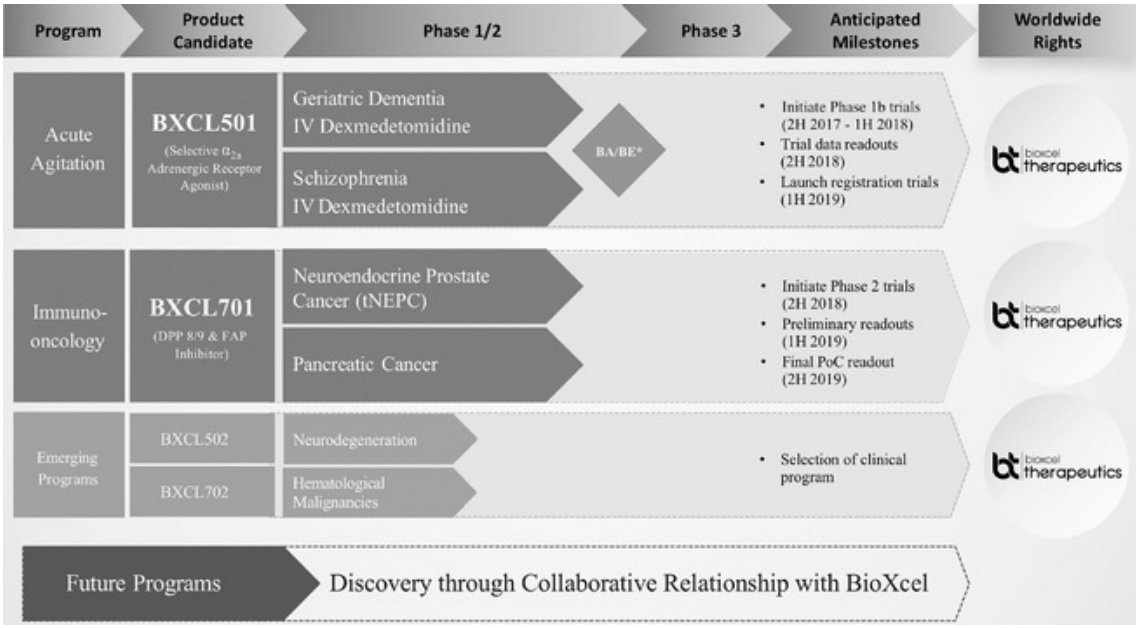
orphan drug designation from the FDA for the treatment of acute myeloid leukemia, or AML. We retain global development and commercialization rights to these two programs. We intend to select our next clinical program in 2018 from our emerging or future programs.

We have assembled a management team with extensive experience in the discovery, development and approval of more than 10 drugs and who have held senior executive roles at leading pharmaceutical companies. We are supported by our experienced board of directors and advisory board, which includes Drs. Peter Mueller (Vertex, Boehringer Ingelheim), Steven Paul (Voyager Therapeutics, Sage Therapeutics, Eli Lilly) and Sheila Gujrathi (Receptos, Bristol-Myers Squibb, Roche), who contribute to our strategy with their expertise in building public companies. We believe that our team is ideally positioned to leverage our highly differentiated platform to develop the next wave of innovative medicines.

Our Clinical Programs

The following table summarizes our lead development programs:



* Bridging bioavailability/bioequivalence (BA/BE) study for optimizing BXCL501 sublingual thin film dose for Phase 3 registration trials

There is currently no active IND for any of our product candidates in the United States, however, our initial clinical trial of the IV formulation of Dex for mild-probable AD was granted an IND exemption by the FDA on September 25, 2017. There has there been no authorization received from any other drug regulatory authority.

Our Strategy

Our goal is to become a leader in the field of neuroscience and immuno-oncology. The key elements to achieving this goal are to:

- **Advance BXCL501, a sublingual thin film formulation of Dex, a selective α_{2a} adrenergic receptor agonist, designed for acute treatment of agitation, to approval through an accelerated FDA Section 505(b)(2) pathway.**

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- **Neurological Disorders.** We believe that BXCL501 has the potential to become the standard of care for the acute treatment of agitation arising from diseases such as AD. Dex has been shown to significantly reduce agitation in elderly patients experiencing anesthetic-induced delirium who did not respond to treatment with haloperidol, a potent antipsychotic that is used to treat symptoms for schizophrenia. We initiated a Phase 1b single ascending and descending dose study of the IV formulation of Dex for evaluating PK/PD and safety in mild probable AD patients in December 2017, followed by a PoC open label clinical trial, both of which we expect to report data in the second half of 2018. We also intend to initiate a bridging BA/BE study in the second half of 2018 and potentially initiate a registration trial in the first half of 2019.

- **Psychiatric Disorders.** We intend to follow a similar development strategy for the acute treatment of agitation in schizophrenia. We plan to conduct a Phase 1b single ascending and descending dose study of the IV formulation of Dex for evaluating PK/PD and safety in schizophrenia patients being treated with atypical antipsychotics. We expect these studies to begin in the first half of 2018, and will commence a PoC open label clinical trial in agitated schizophrenia patients in the second half of 2018. We intend to initiate a bridging BA/BE study in the second half of 2018 that, if successful, could potentially lead to the start of a registration trial in the first half of 2019.

- **Additional Indications.** We also plan to expand into additional indications for acute treatment of agitation resulting from delirium, alcohol or opiate withdrawal, and post-traumatic stress disorder, or PTSD, as well as explore the use of BXCL501 in patients who are claustrophobic and anxious awaiting an MRI.

- **Advance BXCL701 into Phase 2 trials to assess its potential to be the first approved therapy for tNEPC and for the treatment of pancreatic cancer.**

  - **tNEPC (Orphan Segment of Prostate Cancer).** BXCL701 was previously studied in multiple clinical trials and demonstrated single agent anti-tumor activity in melanoma, an immuno-sensitive tumor. In our preclinical studies, BXCL701 has demonstrated the ability to synergistically increase the anti-tumor activity of checkpoint inhibitors. We believe the existing preclinical and clinical data for BXCL701 may significantly reduce our development time for this compound. We plan to initiate a Phase 2 PoC open label clinical trial in the second half of 2018, as a single agent and in combination with Keytruda in patients with tNEPC.

  - **Pancreatic Cancer.** Data indicates that fibroblast activation protein positive, or FAP+, cells contribute to checkpoint inhibitor resistance in pancreatic cancer, which we believe provides a strong rationale for combining BXCL701 with Keytruda. BXCL701 has been granted orphan drug designation by the FDA for the treatment of pancreatic cancer. We believe the existing clinical and preclinical data for BXCL701 in pancreatic cancer may reduce our development time for this compound. We are planning to initiate clinical development of BXCL701 in pancreatic cancer in the second half of 2018 in collaboration with the Lombardi Cancer Center, starting with a mechanistic study in the neoadjuvant setting (before surgery) followed by an efficacy study in pretreated metastatic patients in combination with Keytruda.

  - **Potential for Accelerated Clinical and Regulatory Approval.** Given that both indications have high unmet medical needs and limited or no treatment options, we intend to pursue breakthrough therapy designation and accelerated approval pathways for both indications.

  - **Additional Indications.** We believe BXCL701 is active at multiple stages of the cancer immunity cycle. As such, we believe BXCL701 offers a "pipeline in a product" platform

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given its potential application across other solid tumor types. We believe existing preclinical and clinical evidence support BXCL701's combination potential with checkpoint inhibitors, programmed cell death protein 1, or PD1, or programmed cell death-ligand 1, or PD-L1, inhibitors, antibody-dependent cell-mediated cytotoxicity, or ADCC, antibodies, and cellular therapies such as chimeric antigen receptor T-cell therapy, or CAR-T, for solid tumors and therapeutic vaccines.

- **Identify biomarkers to select patients who have the highest likelihood to respond to our product candidates.** Predicting optimal drug responses in patients requires the identification and validation of predictive biomarkers, specifically in cancer. We believe that our ability to identify patient subsets most likely to respond to our product candidates will increase the clinical benefit to patients and improve the probability of success of our clinical trials. The indications for our lead product candidate BXCL701 were chosen in part because they are known to overexpress DPP 8/9 and FAP. Our planned PoC clinical trial of BXCL701 will examine biomarkers related to its molecular and cellular targets to identify those that may correlate with clinical efficacy and increase our likelihood of success. We are planning to use a similar biomarker-driven approach for future product candidates, including BXCL702.

- **Enhance our R&D pipeline by leveraging our therapeutic area expertise with EvolverAI to identify, develop and commercialize new product candidates in neuroscience and immuno-oncology.** In addition to our leading clinical programs and our emerging and future pipeline, we intend to select our next clinical program during 2018. We have established translational and development expertise, which we believe will help us advance the present and future product candidates in these fields. We may also opportunistically in-license additional product candidates identified through our AI platform approach within our core areas of expertise.

- **Maximize the commercial potential of our product candidates.** We have worldwide development and commercialization rights to our BXCL501, BXCL701, BXCL502 and BXCL702 product candidates. If BXCL501 and BXCL701 are approved in the United States, we would consider building a specialty sales force in the United States and/or collaborate with third parties to maximize the potential of our product candidates. Furthermore, we intend to commercialize BXCL501 and BXCL701 outside the United States through collaborations with third parties.

Management, Board and Advisors Experience

Our management team, board members and advisors are industry veterans having combined experience of more than 150 years in drug discovery, development, business development and commercial leadership in neuroscience and oncology and they have been responsible for the development and approval of more than 10 drugs.

Our co-founder and Chief Executive Officer, Vimal Mehta, Ph.D., is a serial entrepreneur who brings over two decades of experience in launching new ventures, corporate strategy and financing, and global partnering including licensing and M&A transactions. Our Chief Scientific Officer, Frank Yocca, Ph.D., brings over three decades of experience in strategy, discovery and development focused on psychiatry, central nervous system, or CNS, and pain at AstraZeneca and Bristol-Myers Squibb where he played a key role in the development of commercialized products including Abilify, BuSpar and Serzone. Our Chief Medical Officer, Vince O'Neill, M.D., brings over two decades of oncology therapeutic and diagnostic product development experience at Sanofi, Genentech and GlaxoSmithKline where he was instrumental in the expanded approval of Genentech's Avastin and Tarceva and the approval of GSK's Mekinist. Our Vice President—Oncology R&D, Luca Rastelli, Ph.D., brings over two decades of drug discovery and development experience in oncology at several companies including CuraGen and EMD Serono, where he played a key role in the novel immuno-oncology anti-PD-L1 Bavencio and discovery of the anti-transmembrane glycoprotein NMB antibody Glembatumumab vedotin. Our Chief Financial Officer, Richard Steinhart, brings over three decades of financial experience at a number of public and private companies in the healthcare industry including Remedy Pharmaceuticals, Inc., MELA Sciences, Inc. and Emisphere Technologies, Inc.

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Our Chairman, Dr. Peter Mueller, has a career spanning more than 30 years in executive leadership roles at Vertex Pharmaceuticals and Boehringer Ingelheim where he played a key role in the development of several approved drugs, including Orkambi, Kalydeco, Incivek, Spiriva and Atrovent. Dr. Mueller and his development teams were awarded the prestigious Galenus Preis (Kalydeco—Europe) and Prix Galien (Incivek—US) industry awards recognizing their contributions in cystic fibrosis and Hepatitis C, among others. Our advisor Dr. Steven Paul is President and CEO of Voyager Therapeutics and brings more than three decades in CNS drug discovery and development to support our neuroscience program. Our advisor Dr. Sheila Gujrathi most recently served as Chief Medical Officer of Receptos and brings over two decades of experience in drug discovery, clinical development and commercial leadership in oncology and immunology to our oncology program.

Our Novel Drug Re-Innovation Approach

Our AI-based discovery and development process is the foundation of our drug re-innovation model for identifying the next wave of medicines. Our therapeutic area experts have over 60 years of experience across the drug discovery and development value chain. We believe EvolverAI is a novel method of finding potential product candidates because it combines the comprehensiveness and efficiency of machine learning and big data analytics with the expertise and intuition of human experience in drug development. We believe the combination of our therapeutic area expertise and our ability to generate therapeutic candidates in neuroscience and immuno-oncology through our exclusive collaborative relationship in those areas with BioXcel gives us a significant competitive advantage.

The pharmacological space spans more than 27,000 active pharmaceutical agents and only around 4,000 are approved and marketed drugs benefiting patients. These marketed drugs may be applied to other indications, including rare diseases, and represent an untapped potential for meeting significant unmet medical need and recoupment of research and development investments. A large number of the remaining agents are clinical candidates that are active, shelved or have failed for reasons other than toxicity and can potentially be re-engineered for different indications or patient segments. They potentially represent an unrealized investment of billions of research and development dollars by the private and public sectors, resulting in an immeasurable amount of patient suffering and sacrificing during clinical development.

Traditional drug development is plagued with low success rates (11.3%, according to Tufts Center for the Study of Drug Development White Paper, 2015), long drug development cycles (10-15 years, according to PhRMA Key Facts 2016) and exorbitant development costs ($2.6 billion per drug, according to PhRMA Key Facts 2016). Furthermore, many serious diseases continue to go unaddressed due to limitations of the current drug discovery paradigm. The recent advent of numerous 'omics' technologies (genomics, proteomics) and rapid advances in science and medicine are generating terabytes of valuable unexploited knowledge that is widely distributed in multiple big data lakes with several orders of complexity and variety. Much of this data is not being systematically applied to the development of next-generation therapeutics, thus preventing the optimization of drug development utilizing the understanding of technology, science, medicine, markets and commercial opportunities. The efficient and intuitive use of big data remains a bottleneck and a challenge to the pharmaceutical industry. Taken together, these factors underscore the need for fundamental new approaches to drug discovery and development. The market opportunity to identify new uses for existing pharmacological agents remains substantial, due to the lack of technology-driven insights. Our parent, BioXcel, has created a proprietary R&D engine, EvolverAI, for drug re-innovation that provides a proprietary systems-based approach designed to unlock the hidden value in drugs. The combination of our therapeutic area expertise and our exclusive collaborative relationship with BioXcel enables us to screen, analyze, and identify the product candidates that we believe have a high likelihood of benefiting patients. The compounds in our pipeline have been identified using this proprietary platform.

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EvolverAI is designed to eliminate human bias by scanning millions of data points from disparate data sources to create network maps. The nodes and connections in the network map are weighted and ranked based on the validity of supporting evidence using disease specific algorithms. They are then further analyzed using artificial intelligence and machine learning approaches supplemented by human domain-based expertise to uncover novel connections between disease parameters, molecular targets, mechanisms of actions and product candidates.

This drug re-innovation model is exemplified by the successful development and commercialization of drugs such as Tecfidera (Biogen, Inc.), Thalomid (Celgene Corporation) and Viagra (Pfizer, Inc.). All of these drugs were identified by insights in biology and disease pathophysiology. The successful business models of biotech companies like Puma Biotechnology, Inc. and Corvus Pharmaceuticals, Inc. are based on the re-innovation of existing clinical candidates or marketed drugs to provide novel solutions for patients. Unfortunately, such discoveries have been severely limited in scope due to the lack of a genuinely integrated big data analytics based approach.

We believe that only EvolverAI allows a comprehensive and unbiased evaluation of the complete pharmacological space. Our drug portfolio was identified using EvolverAI and the lead programs were chosen among more than 20 compounds selected using this approach. We believe our drug re-innovation model and exclusive collaborative relationship with BioXcel has the potential to reduce the cost and time of drug development, help us design more efficient trials and accelerate our product candidates' time to market. This assumption is based on capitalizing product candidates with substantial clinical data and mitigated risk due to well-defined safety profiles, known PK/PD properties, and an established manufacturing and regulatory path.

BXCL501, Potential First-in-Class Sublingual Thin Film, α_{2a}Adrenergic Receptor Agonist, for Acute Treatment of Agitation

Agitation Overview and Market Opportunity

Agitation is a common symptom of neurological and psychiatric disorders that currently can only be addressed with invasive treatments in institutional facilities. Agitation is characterized by feelings of unease, excessive talking and/or unintentional and purposeless motions, such as wringing of the hands or pacing. People experiencing agitation may also express excitement, hostility, poor impulse control, tension, uncooperativeness and sometimes disruptive behavior, which could lead to aggression and violence. Often, symptoms of agitation are observed with anxiety or aggressive behavior. In many cases, people develop agitation when treatment for their underlying disorder is not working well. Stressful situations or traumatic events can also trigger agitation. Agitation can occur suddenly or slowly and vary in length, lasting for a few minutes or for an extended period of time.

With the agitation issues associated with schizophrenia and bipolar disease coupled with a fast-growing elderly population, the difficulties and expenses of acute treatment of agitation are expected going to grow significantly. Based on our market research, we estimate that in 2016 the total direct financial cost of all aspects of care for agitation in AD was approximately $40 billion. Management believes that in the near future, the total direct financial cost of all aspects of care for agitation across schizophrenia and biopolar disorder will exceed the costs associated with agitation in

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AD. Below are estimated statistics associated with BTI's initial indications targeting agitation in AD, schizophrenia and bipolar disease.

U.S. Market for Treating Agitation		
	Alzheimer's Disease	***Schizophrenia/Bipolar Disease***
Total Patient Population	5,100,000	8,000,000
Diagnosed Agitated Patients	~1,000,000 (30%)	~4,000,000 (50%)
Agitated Patients Receiving Treatment	~525,000 (35%)	~2,000,000 (50%)
Percent Treatable by BXCL501	100%	33%
BXCL501 Addressable Market	525,000	660,000
Estimated Annual Usage Per Patient	24	12
Potential Addressable Annual Usage	12,840,000	7,920,000

Figure 1. Statistics for U.S. market for treating agitation.

Limitations of Current Treatments for Agitation

Despite observed suboptimal safety and side effect profile, antipsychotics are currently used off-label to treat agitation in dementia as well as delirium and are currently the standard of care for the acute treatment of agitation in schizophrenia and bipolar disease. IM delivered antipsychotics, such as haloperidol and risperidone, are used extensively in this setting but are invasive and require patient restraint. Furthermore, these treatments include a black box warning for use in elderly patients. While sublingual tablet formulations utilizing antipsychotics have been developed, these sublingual formulations have long half-lives (21-24 hours) and significant side effects when given either acutely or chronically. Oral agents such as benzodiazepines are also used, but have a slower onset of action and are consequently not effective in the acute treatment of agitation. Side effects of these agents include sedation, amnesia, confusion and a paradoxical response. They can intensify cognitive slowing, cause dependence and can contribute to increased risk of falls and fractures. In addition, long-term use of benzodiazepines has been found to be habit-forming and can cause addiction. Non-adherence with oral agents can also be problematic as patients may attempt to spit out these medications. We believe that based on the current method of administration of oral medicine for agitation, the sublingual thin film offers compliance advantages as it will prevent patients from avoiding treatment.

There is precedent for FDA approval of a non-invasive therapy for the acute treatment of agitation. In 2012, Adasuve, an inhaled version of the antipsychotic loxapine, became the first approved non-invasive acute treatment for agitation in patients with schizophrenia and bipolar disease. The number of hospitals and pharmacies that can administer Adasuve is limited due to a risk of management program, and Adasuve also has a high incidence of side effects. Upon launch, Adasuve was priced at $145 per dose.

The sublingual route of administration is becoming an accepted alternative to oral administration of drug delivery to the CNS when rapid onset or more controlled delivery is required. Currently, there are six products that are approved for sublingual thin film administration. For example, Cynapsus

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Therapeutics, Inc. (acquired by Sunovion Pharmaceuticals, Inc.), is a specialty CNS pharmaceutical company that developed a fast-acting, easy-to-use, apomorphine sublingual thin film for the on-demand management of debilitating episodes of tremor associated with Parkinson's Disease. We are in the process of developing a differentiated sublingual thin film dosage form of Dex, which, if approved, may offer benefits such as ease of use and quick absorption for rapid therapeutic effects.

Figure 2. Visual representation of BXCL501 sublingual thin film administration.

Our Solution: BXCL501 Potential First-in-Class Sublingual Thin Film for the Acute Treatment of Agitation

BXCL501, a sublingual thin film formulation of the sedative and anesthetic agent Dex, is designed to be easily administered and have a rapid onset of action. We believe that BXCL501, with its differentiated pharmacology and ease of administration, if approved, could potentially be a first-in-class, non-invasive acute treatment for agitation that can be rapidly administered by physicians and caregivers. Dex is approved in the United States for the sedation of initially intubated and mechanically ventilated patients during treatment in the Intensive Care Unit, or ICU. It is also used in the intensive care setting and sedation of non-intubated patients prior to and/or during surgical and other invasive procedures. Dex, launched in the United States as Precedex in 1999, is a selective α_{2a} adrenergic receptor agonist that has a strong safety record and has been studied in over 130 clinical trials to date. It has also been launched in the European Union and multiple other countries under the trade name Dexdor as a sedative for intensive care patients. Dex gained approval by the European Medicines Agency, or EMA, for sedation of adult ICU patients (requiring a sedation level no deeper than arousal in response to verbal stimulation). It has been used to prevent or treat hyperactive delirium resulting from anesthesia in the ICU. Given these uses of the IV formulation of Dex, we believe Dex formulated in a sublingual thin film will allow for ease of administration in settings where rapid acute treatment of agitation is needed.

Mechanism of Action: α_{2a} Adrenergic Receptor and NE Role in Acute Agitation

BXCL501, with its potential ease of administration and mechanism of action, targets brain agitation mechanisms. Agitation is prevalent in numerous indications, including AD, schizophrenia and bipolar disease and follows a similar causal mechanism. Norepinephrine, or NE, levels are elevated when dementia or schizophrenia patients experience agitation. An α_{2a} receptor agonist, such as Dex, would act to reduce these levels, which would produce a calming effect in patients. It has been well documented that the α_{2a} adrenergic receptors regulate NE in the central nervous system. They are predominantly involved in the control of brain cell communication. Therefore, agents which interact with the α_{2a} adrenergic receptor can selectively regulate the NE system, unlike antipsychotics. Dex is

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highly selective for the α_{2a} adrenergic receptor, which results in fewer side effects. The figure below illustrates its mechanism of action.

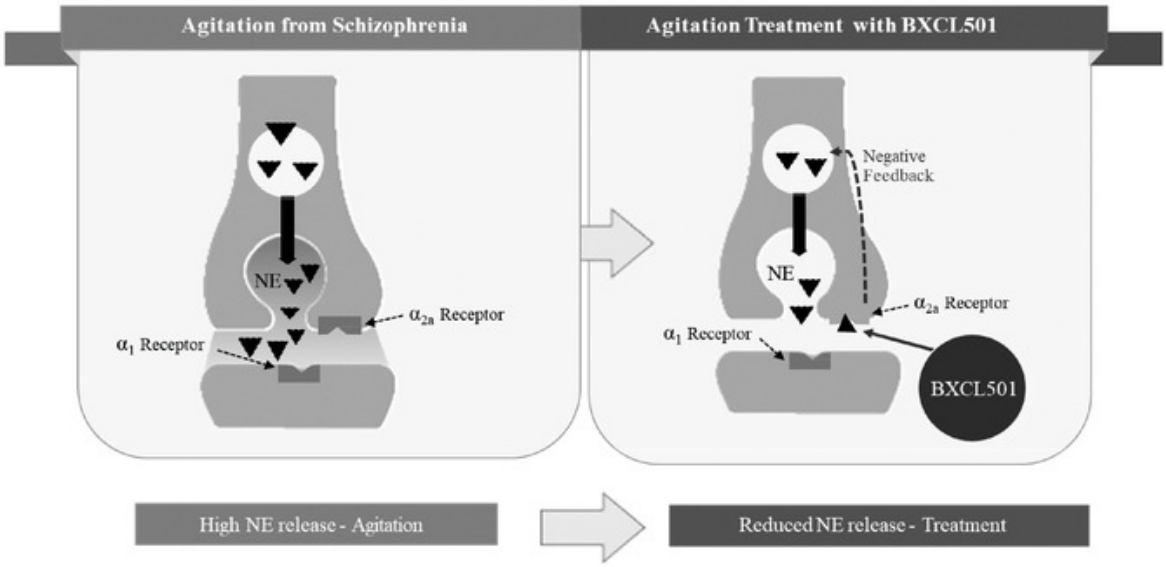

Figure 3. BXCL501 mechanism of action. High norepinephrine, or NE, levels are responsible for agitation. BXCL501 reduces agitation by selectively targeting the α_{2a}adrenergic receptor to reduce NE release.

Summary of Existing Dex Clinical Data

Dex has demonstrated efficacy in acute treatment of agitation from delirium and managing pain in patient populations. Approximately 130 trials have been conducted with Dex as an anesthetic agent in patients with diseases and disorders in a variety of patient segments. To date, based on information available in the package insert for Dex, patients treated with Dex have experienced drug-related side effects including hypotension, transient hypertension, bradycardia, dry mouth, acute respiratory distress syndrome, respiratory failure and agitation with hypotension, bradycardia and dry mouth considered serious adverse events. It has the potential to exhibit strong sedative, analgesic and anxiolytic properties. Furthermore, it demonstrates activity in reducing agitation associated with delirium, suggesting that it may have the ability to control agitation in neurological and psychiatric diseases.

Clinical studies have provided evidence of Dex's activity in reducing agitation associated with delirium, which we believe suggests that Dex may have the ability to control agitation in psychiatric diseases.

- In a non-randomized Phase 2 clinical trial based on information in an article published in Critical Care Medicine in 2015 by Carrasco et al., patients received an IV bolus of haloperidol and additional doses at intervals of 10-30 minutes until agitation was controlled (Richmond Agitation Sedation Scale, or RASS, score of 0 to –2) or until reaching the maximum total dose of 30 mg. Patients served as their own control. For those patients whose agitation was not controlled by haloperidol, Dex was infused to attain a target RASS score of 0. The haloperidol infusion was then gradually tapered and discontinued, with patients continuing on Dex alone.

- Dex demonstrated significant reduction in agitation associated with delirium in non-intubated patients who did not respond to haloperidol. Dex alone was more effective than haloperidol alone in its ability to achieve and maintain low agitation scores, as seen in Figure 4 below. There

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were multiple instances where administration of haloperidol was suspended due to over-sedation (these patients were excluded from the study and are not reflected in the figure below). In contrast, Dex administration did not result in any instances of over-sedation. These results demonstrate that Dex could be a useful treatment for treating agitation without inducing over-sedation. Further, these results suggest that Dex could be useful in treating agitation caused by different diseases, such as AD, schizophrenia and bipolar disease.

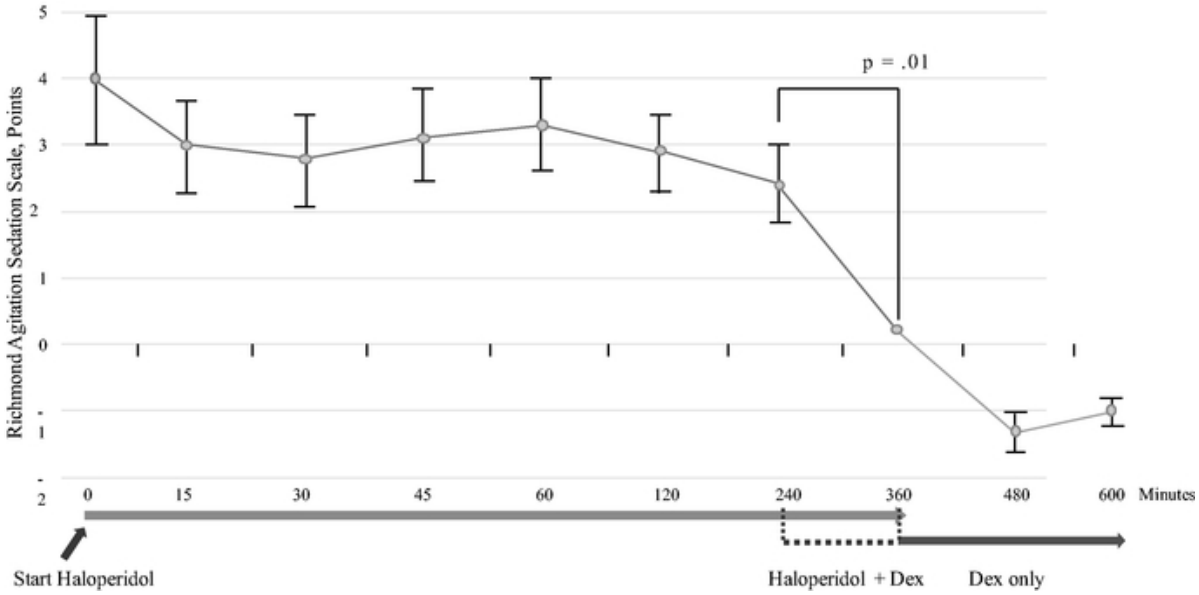

Figure 4. In an article published in Critical Care Medicine in 2015 by Carrasco et al., Dex was shown to significantly reduce agitation due to delirium in non-intubated patients that had failed on haloperidol treatment and had better effectiveness and safety than haloperidol. Significant reductions in agitation were produced by Dex in non-responsive patients who were treated with haloperidol and rescued by Dex (p=0.01). Reductions in agitation continued when Dex was given alone.

- Patients treated with Dex prior to surgery demonstrated a significant reduction in the incidence of ICU based agitation compared to patients that received propofol or midazolam. Several clinical studies conducted in this manner suggest that Dex reduces delirium and agitation, without respiratory depression. Based on information in an article published in the International Journal of Scientific Reports in 2017 by Zhang et al., patients who experienced emergent agitation and/or delirium were successfully managed with a Dex regimen with adverse events similar to those reported in the package insert.

Preclinical Studies Performed by BTI with Dex

We sponsored and conducted two animal studies of Dex. In the first study, we tested sublingual administration of a liquid form of Dex in rats to demonstrate that Dex can be absorbed sublingually and that activity (mimicking arousable sedation) could be achieved in the absence of significant heart rate and blood pressure changes. Additionally, we demonstrated in a rat model of aggression that an IV formulation of Dex inhibited behaviors associated with agitation and aggression in a dose dependent manner without over-sedation.

We also examined the acute effect of sublingual administration of Dex in rats to determine its ability to reduce activity. Hyperactivity in rats represents a preclinical translational behavioral marker for agitation. In the preclinical study, rats were given a sublingual administration of Dex at varying

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doses (5 - 40 mcg/kg). Parameters such as behavioral assessment (video monitoring of home cage activity (*e.g.,* sleep/wake)), sleep onset latency, total sleep time, motor activity (Rota rod), respiration (tidal volume and frequency), and cardiac activity (heart rate and blood pressure) were measured. Drug plasma concentrations were also measured. Sublingual administration of Dex induced a dose-dependent increase in total sleep time and a significant reduction of latency to sleep. Furthermore, no significant reduction in blood pressure, heart rate or respiratory parameters were observed at doses below 40 mcg. We believe these changes in behavior indicate that Dex was absorbed via the sublingual route and that Dex had an anti-arousal action on rats.

We have also observed the effect of IV administration of Dex in aggressive animals. We used the resident intruder rat model to evaluate the anti-agitation and/or aggression properties of Dex at varying doses. This model was used to study defensive behavior and aggression in mice and rats. When rodents are exposed to a new male in their home cage environment, they perceive the novel male animal as an "intruder" and demonstrate a repertoire of defensive behaviors. By recording the frequencies, durations, latencies and patterns of the observed behavioral acts as well as postures during these confrontations, a detailed quantitative picture (ethogram) of aggression behavior can be evaluated.

Resident animals were administered the IV formulation of Dex at doses of either 0.3, 0.5, 1.0 or 1.5 mcg/kg 15 minutes prior to testing and the response to the intruder rat was examined for agitation for 15 minutes. Parameters such as ano-genital sniffing, chasing, attacking, biting and latency to attack (both frequency and duration of events) were noted along with the estimation of terminal drug plasma concentrations. Administration of Dex resulted in a dose-dependent, significant reduction in the frequency and duration of several behavioral indices of aggression. A significant increase in the latency to attack was also observed at increasing doses of Dex compared to the control group indicating a reduction in aggression. In summary, this preliminary data for Dex dosed intravenously shows a reduction in aggressive behavior of rats in a dose dependent fashion. We believe the reduction in the overall aggression parameters demonstrates the anxiety/anti-aggression potential of the drug. Future studies are planned with sublingual thin film formulation using the same animal model for aggression.

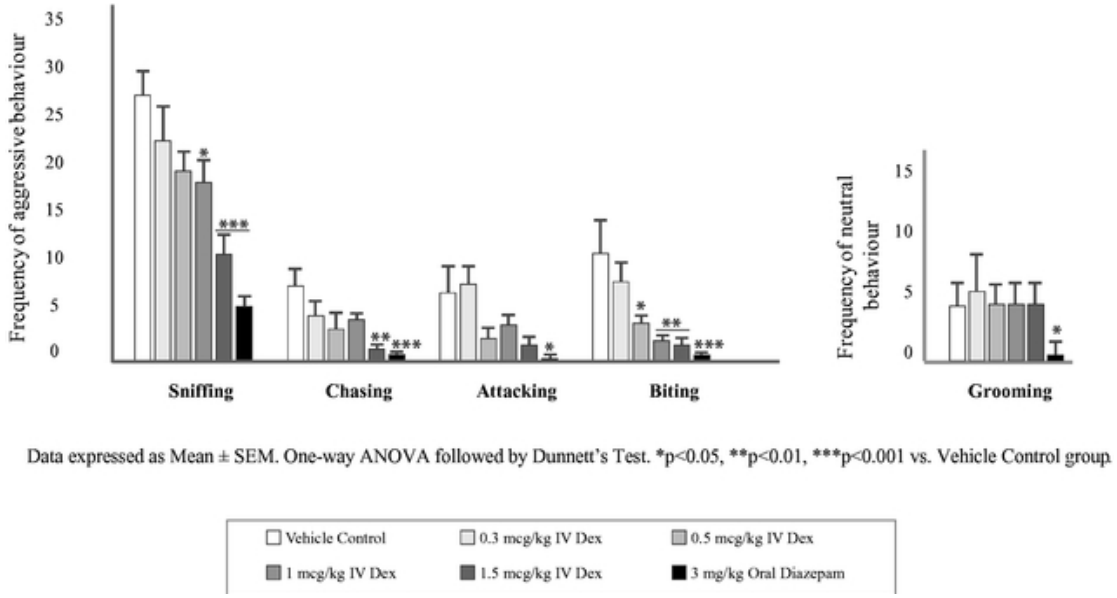

Figure 5. Evaluation of various doses of the IV formulation of Dex for treating aggression in a rat resident intruder model based on a study we sponsored in 2016 with a duration of 15 days. A dose dependent reduction in frequency and duration of aggressive behavior was observed as compared to controls. Dex also did not induce sedation, while oral diazepam did, as shown using the reduction in the normal grooming behavior as a surrogate of sedation.

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BXCL501 Clinical Program

Our fully integrated BXCL501 clinical program for treating agitation in AD and schizophrenia is outlined in the figure below. We plan to initially conduct ascending and descending dose studies of the IV formulation of Dex to evaluate PK/PD and safety in mild probable AD patients and schizophrenics on atypical antipsychotics. The planned studies using the IV formulation of Dex in mild probable AD patients and schizophrenics will determine the optimal exposure necessary to produce calm or an arousable sedation and control agitation in these patient groups. Following completion, we plan to initiate a PoC open label study, treating agitated AD and schizophrenia patients with the optimal dose of the IV formulation of Dex determined in the Phase 1b study. This will be followed by a bridging BA/BE study, potentially leading to registration trials with BXCL501, our sublingual thin film formulation of Dex.

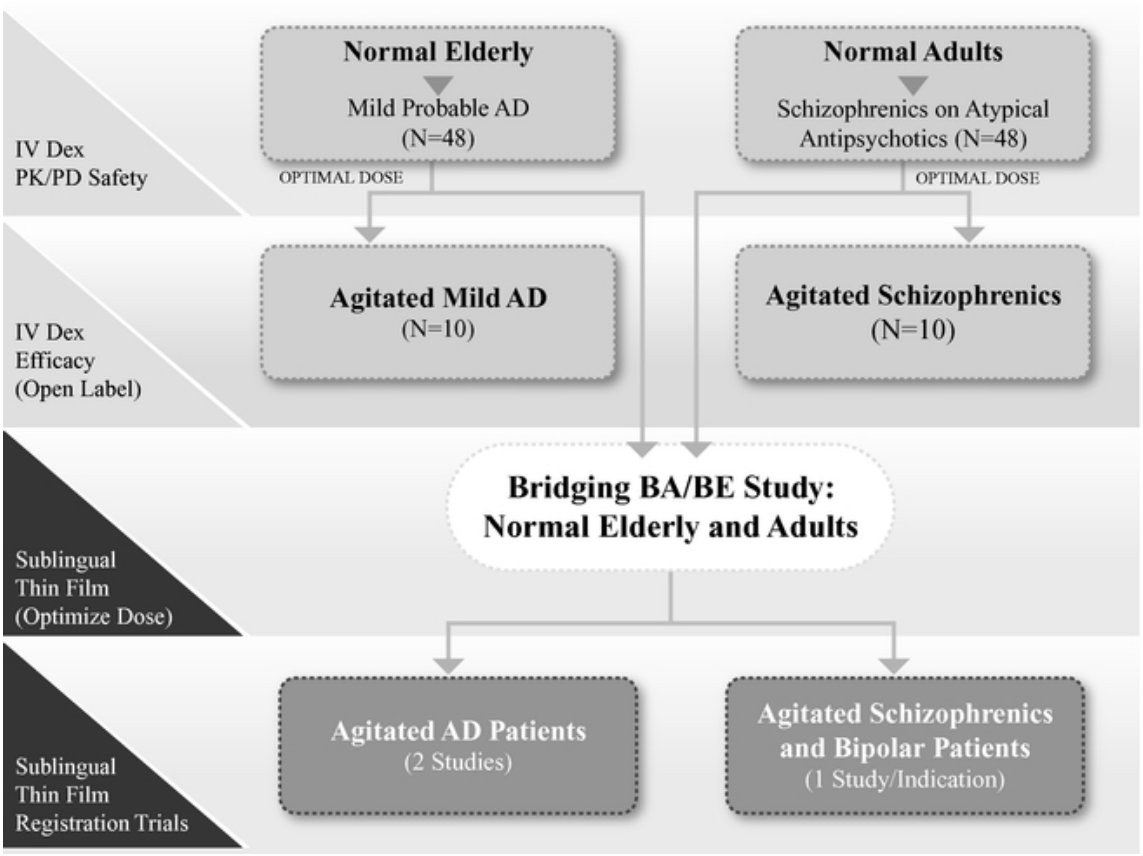

Figure 6. Integrated clinical development plan (subject to FDA approval) for BXCL501 for acute treatment of agitation in AD, schizophrenia and bipolar disease.

Agitation in Dementia

In December 2017 we commenced a Phase 1b single ascending or descending dose PK/PD study of an IV formulation of Dex in healthy volunteers followed by patients with mild probable AD, for a total of up to 48 individuals that we expect to be complete in the second half of 2018. The study design entails determining the optimal dose of an IV formulation of Dex and rate of delivery to provide an anti-agitation dose without patients experiencing adverse effects on respiratory drive, blood pressure and cognitive functioning. The primary endpoint will be to determine the optimal dose of an IV

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formulation of Dex in the target population to achieve the required anti-agitation, arousable sedation effect, as defined using RASS, a widely used method to determine a patient's level of sedation. The study will be double-blind, placebo-controlled adaptive trial and will include up to six cohorts. Each cohort will consist of eight individuals, six treated with IV formulation of Dex and two individuals treated with placebo. The first four cohorts will be healthy elderly volunteers and the last two cohorts will be in patients with mild probable AD. The initial dose of the IV formulation of Dex will be 0.1 mcg/kg/hr with no loading dose. The infusion can be continued for up to three hours with dose increases until arousable sedation is achieved. The subsequent cohorts (healthy volunteers) will enable dose and rate optimization using an adaptive approach. The optimized dose and rate will then be tested in patients with mild probable AD to understand whether the underlying pathology affects the activity or safety parameters. These data will be used to optimize our sublingual thin film.

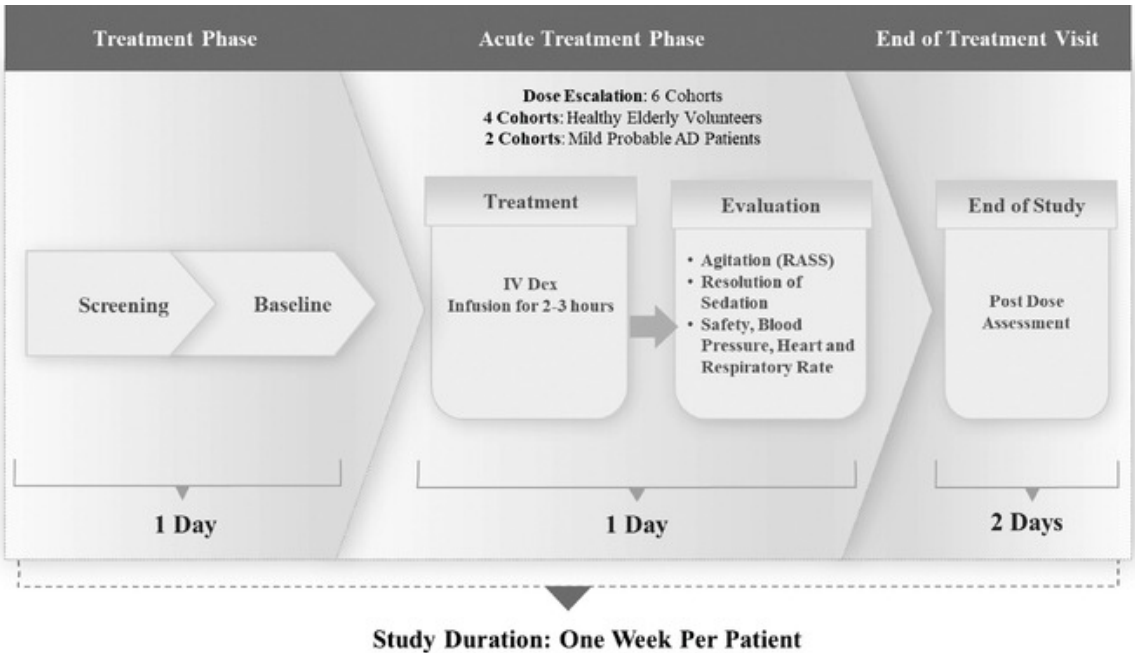

Figure 7. Initial ascending and descending dose study of the IV formulation of Dex for evaluating PK/PD safety in healthy elderly volunteers and mild probable AD patients. Each patient evaluation is expected to be completed in one week.

The optimal dose and rate from this initial study will subsequently be tested in an open label clinical trial to determine efficacy in AD patients with ongoing agitation. Upon completion of the sublingual thin film formulation, a bridging BA/BE study will be performed to determine the sublingual thin film dose necessary to achieve exposure levels that were found to be optimal for efficacy in these initial studies of the IV formulation of Dex. Following the bridging BA/BE study, the equivalent effective dose in the BXCL501 sublingual thin film formulation will be tested in a potential registration trial for the acute treatment of agitation in dementia, which we expect to commence in the first half of 2019.

Agitation in Schizophrenia

We plan to conduct a Phase 1b ascending and descending dose PK/PD and safety study with the IV formulation of Dex in schizophrenics currently being treated with an atypical antipsychotic. Following completion, we intend to conduct a PoC open label clinical trial that will be performed in agitated schizophrenics. Following the planned bridging BA/BE study, the equivalent effective dose in

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the BXCL501 sublingual thin film formulation will be tested in a registration trial for treating agitation in schizophrenia and bipolar disease, which we intend to commence in the first half of 2019.

Bridging Bioavailability and Bioequivalence Study: BXCL501 Sublingual Thin Film PK Study

The planned studies using the IV formulation of Dex in mild probable AD patients and schizophrenics are expected to determine the optimal exposure necessary to produce calm or an arousable sedation and control agitation in these patient groups. Through a bridging BA/BE study, we will determine the optimal dose of the BXCL501 sublingual thin film that will yield the same blood exposure that achieved efficacy in the IV formulation of Dex study. To achieve this, we plan to perform a randomized, double-blind, placebo controlled dose escalation study to determine the PK, safety and tolerability of a single sublingual thin film formulation of Dex in healthy adult and elderly volunteers. In this dose-escalation study, participants will be randomly assigned to receive four doses of BXCL501 or placebo. We currently expect to have four cohorts and within each group, participants will receive BXCL501 or a sublingual thin film placebo. The safety and tolerability of each dose level will be carefully reviewed before administration of the next higher dose.

Planned Phase 3 Registration Trials

We intend to conduct Phase 3 registration trials using BXCL501 for acute treatment of agitation in AD, schizophrenia and bipolar disease using the Section 505(b)(2) regulatory pathway. We anticipate that these studies will consist of multicenter, randomized, double-blind, placebo controlled parallel-group studies with a few hundred patients for each of the indications. In the dementia Phase 3 trial, we plan to test two doses of BXCL501 alongside placebo. We believe that RASS is well suited for in-patient settings and can be used to measure the planned primary endpoint, which will be the level of agitation or sedation in a patient. For the schizophrenia and bipolar registration trials, we expect the Brief Psychiatric Ratings Scale, or BPRS, will be used to assess efficacy. The BPRS can capture the change in levels of agitation as the primary endpoint as well measure other psychiatric secondary endpoints. All studies will be designed to be conducted in either a hospital or psychiatric in-patient setting. Depending on the outcome of the pre-IND meeting with the FDA expected in 2018, the planned trial design may need to be adjusted to fit the regulatory path agreed to with the FDA.

Other Neuropsychiatric/Neurodegenerative Indications

Given the differentiated properties of BXCL501 and its selective mechanism of action, we believe that BXCL501 has the potential for broad applicability across several indications where agitation is a symptom of a condition or underlying disease. Dementia and schizophrenia were chosen as our lead indications. Dementia was chosen based on high unmet medical need and lack of a standard of care for acute treatment of agitation in elderly patients suffering from AD. Schizophrenia was also chosen because of the high incidence of agitation in the emergency room and psychiatric outpatient setting resulting from agitation due to residual psychosis and the need for a non-invasive rapidly acting agent in this setting. There are additional neurological and psychiatric disorders as well as medical conditions where agitation is a symptom that needs treating. If we observe positive efficacy results in dementia and schizophrenia patients, we believe this will provide further proof of concept that BXCL501 has therapeutic potential in other neurodegenerative and psychiatric disorders where agitation is a disruptive symptom for patients and caregivers.

A brief description of potential indications that we could pursue in the future with BXCL501 is summarized below. We will determine the timing and prioritization of additional indications as warranted by emerging data.

- **Delirium.** There are a number of studies which suggest that Dex can either prevent or mitigate agitation resulting from delirium based on information in an article published in the

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International Journal of Scientific Reports in 2017 by Zhang et al. We believe BXCL501 could be used in non-surgical medical situations where hyperactive delirium is an outcome. We also believe BXCL501 would potentially be of high value in elderly patients in many medical situations outside of the ICU, such as the hospital floor and nursing homes. As a result of the delirium studies mentioned in the clinical section above, there is a defined therapeutic index in elderly patients which we believe may allow us to directly initiate a PoC clinical trial, without conducting the IV formulation of Dex study, potentially followed by a registration trial with BXCL501.

- **Alcohol Withdrawal Syndrome.** Acute alcohol withdrawal remains a widespread problem in hospitalized patients. Benzodiazepines remain the primary treatment for alcohol therapy to help control hyperadrenergic output in patients resulting in withdrawal. These patients are at increased risk of experiencing respiratory depression from benzodiazepine therapy. Based on information in an article published in The American Journal of Drug and Alcohol Abuse in 2015 by Wong et al., in clinical trials, IV administration of Dex has shown potential for treating alcohol withdrawal syndrome. We believe that performing a controlled clinical trial with BXCL501 in this population would be a logical next step to develop this product candidate.

- **Hyperarousal in PTSD.** Hyperarousal is a primary symptom of post-traumatic stress disorder, or PTSD. It occurs when a patient becomes hyperaroused as a result of thinking about their trauma. Even though real danger may not be present, their body acts as if it is, causing lasting stress after a traumatic event. The symptoms of hyperarousal include irritability, anger and angry outbursts, constant anxiety and sleeping problems. We believe that BXCL501 has the potential to reduce symptoms which lead to agitation as well to produce a more natural sleep if taken before bedtime.

- **Pretreatment for MRI.** Anxiety, due to feelings of claustrophobia or noise associated with an MRI, is common among patients who will undergo the procedure, which requires the patient to remain still. Currently, short acting oral benzodiazepines are used but must be taken well in advance of the MRI and could be followed by sluggishness and fatigue. We believe that BXCL501 has the potential to calm patients so that they remain still during the procedure.

BXCL701, Potential First-in-Class DPP 8/9 and FAP Inhibitor for the Treatment of tNEPC and Pancreatic Cancer

Neuroendocrine Prostate Cancer Overview and Market Opportunity

Prostate cancer is the most common malignancy and is the second leading cause of cancer death in men in the United States. In 2014, there were an estimated 3 million men with prostate cancer in the United States. According to estimates from Surveillance, Epidemiology and End Results Program, SEER, more than 161,000 men are expected to be diagnosed with and more than 27,000 men are expected to die from prostate cancer in 2017. While the five-year survival rate of local and regional prostate cancer is almost 100%, more aggressive forms of the disease such as metastatic prostate cancer have a five-year survival rate of approximately 30%. These aggressive forms of prostate cancer can initially be treated with androgen deprivation therapy, or ADT, however, almost all patients experience a recurrence in tumor growth which results in the patient having castrate resistant prostate cancer, or CRPC. An estimated 180,000 men in the United States are eligible for treatment with the second-generation anti-androgen drugs Zytiga and Xtandi. These drugs have widely become the standard of care and generated combined worldwide sales of over $4.5 billion in 2016.

Unfortunately, virtually all the patients who respond to Zytiga and Xtandi are expected to progress to even more aggressive forms of prostate cancer requiring further treatment. About one-third of the progressing patients will develop very aggressive, androgen receptor, or AR-independent tumors, or treatment-emergent neuroendocrine prostate cancer, or tNEPC, for which there is no effective

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treatment based on information in an article published in the Journal of the National Comprehensive Cancer Network in 2014 by Agarwal et. al. and an article published by Journal of Clinical Oncology in 2014 by Wang et. al. tNEPC specifically displays neuroendocrine differentiation, either pathologically with the presence of the typical neuroendocrine small cells, or molecularly by expressing neuroendocrine markers. As shown in the figure below, BXCL701 is designed to target this tumor segment because tNEPC has specific biology that is addressable by the mechanism of action of BXCL701. We believe that approximately 30,000 to 40,000 patients in the United States will develop tNEPC who can potentially be treated with BXCL701.

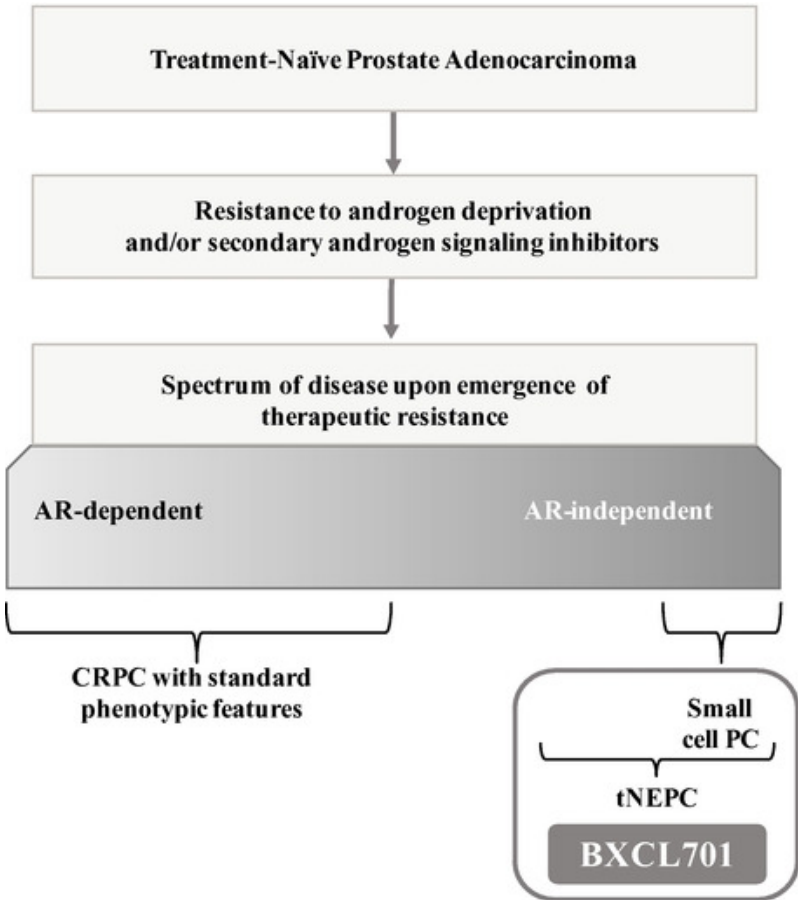

Figure 8. Schematic illustrates tNEPC arising post-ADT therapy treatment. BXCL701 targets this AR-independent subtype where there are no approved therapies and existing/emerging therapies have limited or no efficacy.

Limitations of Current Treatments for tNEPC

There is no approved therapy for tNEPC and therefore we intend to pursue breakthrough therapy designation for BXCL701. tNEPC patients are treated off-label with cytotoxic chemotherapies, such as platinum-based regimens. These treatments have poor efficacy due to their short duration of response and substantial toxicity. As discussed in more detail below, the immuno-oncology field has made several advances in the treatment of solid tumors. However, several trials of immuno-oncology agents in patients with prostate cancer, and specifically tNEPC, have shown limited or no anti-tumor activity. We

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believe BXCL701 is a potential first-in-class therapy in tNEPC given its ability to convert immuno-resistant tumors to immuno-sensitive tumors ("cold" to "hot" tumors).

Immuno-oncology Overview

Immuno-oncology is an emerging approach to treating cancer that is based on stimulating or enhancing an immune response to the tumor. This approach is based on the findings that the mutations occurring in cancer cells may be immunogenic and capable of eliciting an immune response against the tumor. Immuno-oncology therapies offer several potential advantages over existing cancer therapies. First, the immune system exhibits immunologic diversity and selectivity, which enables it to respond to a large number of potential cancer targets. Second, the immune response can be amplified, offering the potential to enhance the efficacy of treatment. Furthermore, once activated, the immune system possesses immunologic memory, potentially providing for a durable response. Finally, immunotherapies may be widely applicable to many types of cancer as immunotherapy mechanisms are generally broadly applicable across tissues. This enables these agents to be potentially active in a multitude of cancers. Checkpoint inhibitors remove the "breaks" on the immune system and unleash the immune system's broad cancer-destroying properties. Antibodies against CTLA-4, PD-1 receptor (or its ligand), and PD-L1 (collectively checkpoint inhibitors) have shown positive clinical results in many tumor types, leading to multiple FDA approvals, including Yervoy (ipilimumab; anti-CTLA-4) Opdivo (nivolumab; anti-PD-1), Keytruda (pembrolizumab; anti-PD-1), Tecentriq (atezolizumab; anti-PD-L1) and Bavencio (avelumab; anti-PD-L1). These checkpoint inhibitors are now the standard of care in several oncology settings and represent a substantial commercial opportunity for developing new treatments. It is estimated that the market for immuno-oncology therapies could exceed $27 billion by 2025.

Although checkpoint inhibitors provide benefits to some patients, they also have several limitations. Only a subset of treated patients, typically less than a third, exhibits robust anti-tumor responses (primary resistance). While anti-tumor responses from checkpoint inhibitors are more durable than with traditional therapies, many patients still relapse (secondary resistance). Checkpoint inhibitors have not shown activity as a single agent in patients with prostate cancer due to these resistance mechanisms. The scientific community believes that these resistance mechanisms are related

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to the immunity cycle. This cycle is a multistep process involving numerous stimulatory and inhibitory factors that amplify and broaden immuno-cell responses as seen in the figure below.

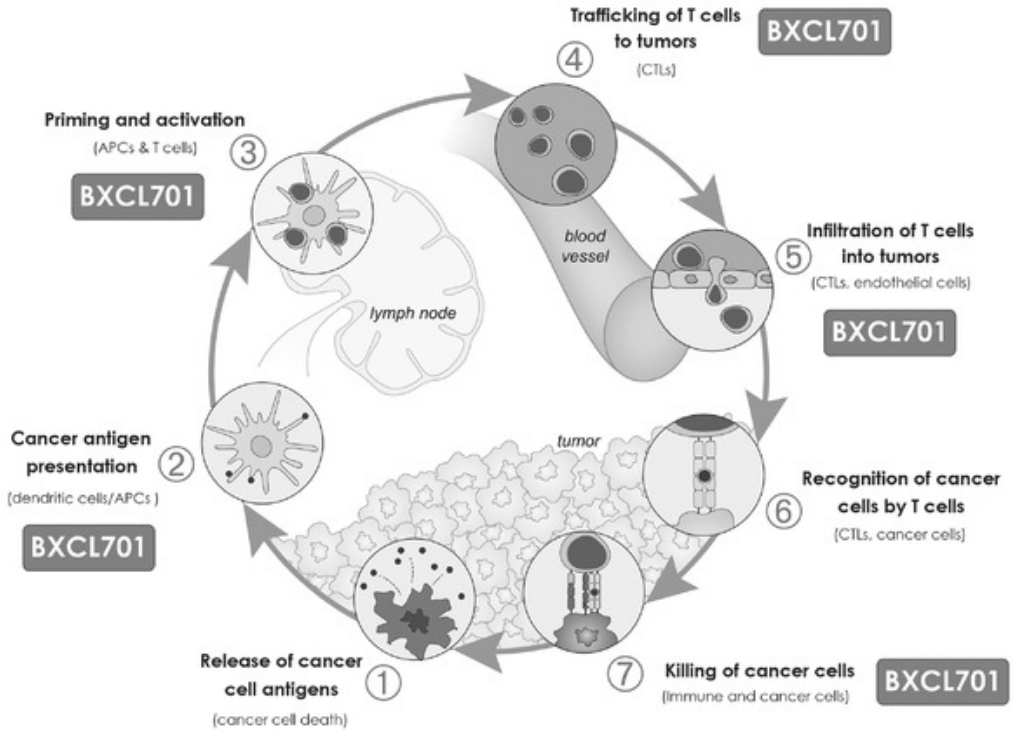

Figure 9a. The cancer immunity cycle as described by Chen and Mellman, Immunity 2013, and those stages where we believe BXCL701 may be active.

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Step	Immunity Cycle Step	Potential Role of BXCL701
2	Cancer antigen presentation by dendritic cells	BXCL701 stimulates the trafficking of dendritic cells to tumor draining lymph nodes.
3	Priming and activation of T-cell	BXCL701 accelerates tumor-induced priming of T-cells and the formation of potent cytotoxic T-lymphocytes (CTLs), which can be transferred to secondary hosts.
4	Trafficking of T-cell (and other immune cells) to the tumor	BXCL701 releases the FAP-mediated block of T-cell migration into the tumor.
5	Infiltration of T-cell (and other immune cells) into the tumor	BXCL701 induces the release of chemokines that attract T-effector cells but block T-regulatory cells, and also induce NK cell and neutrophil migration. In addition, the antiangiogenic activity also increases tumor infiltration.
7	Killing of tumor cells	BXCL701 induces the formation of potent CTL and NK cell expressing tumor killing perforin and granzyme and induces the formation of memory T-cells that can reject and kill tumor cells when they return.

Figure 9b. BXCL701's potential role in the cancer immunity cycle.

Importantly, checkpoint inhibitors only impact the final step of the immunity cycle (step 7 in the figure above), allowing other targets and pathways to be exploited by the tumor to create a non-responsive tumor micro-environment. Therefore, the scientific community believes that identifying combinations of immuno-oncology agents that target more than one of these steps along the immunity cycle will result in improved efficacy and reduced resistance. For example, the combination therapy of Opdivo (anti-PD-1) and Yervoy (anti-CTLA-4), which targets multiple steps in the immunity cycle, was recently approved for the treatment of melanoma. There are several additional targets that are currently in development in the clinic as combination agents, including targets like indoleamine 2,3-dioxygenase, or IDO, which mediates immuno-suppression in step 7, or in preclinical development, such as the novel target stimulator of interferon genes, or STING, which induces production of interferon gamma resulting in T-cell priming via dendritic cell stimulation.

Whereas most of these targets and their related compounds will only affect one step in the immunity cycle, we believe BXCL701 has the ability to affect multiple steps of the immunity cycle. We believe that this differentiating ability should give it an advantage over other agents when used in conjunction with checkpoint inhibitors in converting immuno-resistant tumors to immuno-sensitive tumors ("cold" to "hot" tumors). This activity should optimize the anti-tumor activity of the approved immune checkpoint inhibitors in a higher percentage of patients, including patients whose tumors show primary and secondary resistance to immune checkpoint treatment.

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DPP 8/9 & FAP Role in tNEPC and Immuno-oncology

DPP 8/9 and FAP are overexpressed in tNEPC and play a significant role in tumor growth. DPP 8/9 regulate the activity of neuropeptide Y, or NPY, a neuroendocrine peptide hormone upregulated in tNEPC. We selected tNEPC and pancreatic cancer as our lead indications after evaluating more than 100 different tumor types because they are represent of the top three cancers that overexpressed or amplified DPP 8/9 and FAP.

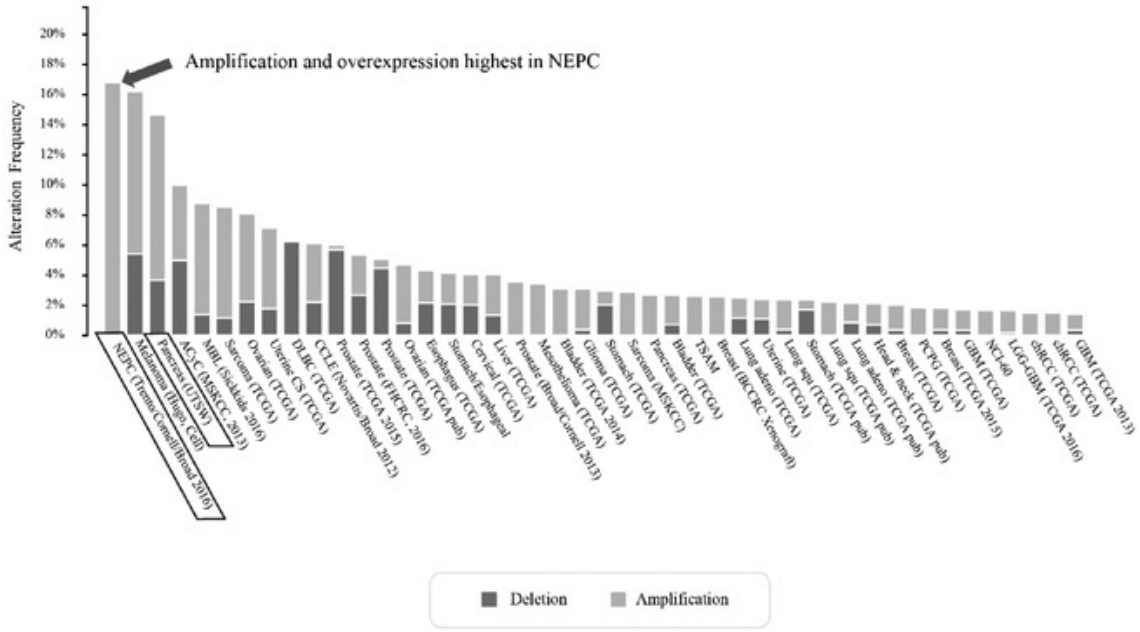

Figure 10. Genetic alteration analysis of DPP 8/9 and FAP (BXCL701 targets) demonstrates that tNEPC and pancreatic cancer have among the highest levels of overexpression and amplification (from The Cancer Genome Atlas).

In addition to this genomic signature, DPP 8/9 and FAP are critical regulators of the immune system and their inhibition causes pro-inflammatory cell death. DPP 8/9 have been shown to limit the activity of macrophages and inhibit the stimulation of the pro-inflammatory anti-tumor response. FAP+ cancer-associated stromal fibroblasts, or FAP+ CAFs, are the main immuno-suppressive cells in tNEPC tumors and blocking their signals leads to improved anti-tumor response. Depleting FAP+ CAFs can delay or prevent tNEPC development. Similarly, myeloid-derived suppressor cells, or MDSC, plays an immuno-suppressive role in the biology of tNEPC. Therefore, we believe inhibition of DPP 8/9 and FAP by BXCL701 can directly lead to tNEPC tumor cell death through the action of the immune system by blocking the activity of the immuno-suppressive cells present in tNEPC.

Our Solution: BXCL701, Potential First-in-Class, Oral, Small Molecule Inhibitor of DPP 8/9 and FAP

BXCL701 is a potential first-in-class, highly potent, oral small molecule immuno-modulator targeting tNEPC that stimulates both the innate and acquired immune system by inhibiting DPP 8/9 identified as novel immuno-checkpoints in based on information in an article published in the Nature Chemical Biology in 2016 by Okondo et al. and FAP, a major immuno-suppressive factor. BXCL701 is differentiated among DPP inhibitors because it is designed to inhibit DPP 8/9, whereas most other DPP inhibitors, including those that have been developed to treat diabetes, are selective for DPP 4. We are not aware of any clinical stage competitors of BXCL701 in the DPP inhibitor class. The product candidate is designed to address the various ways by which DPP 8/9 and FAP play a role in the biology

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of tNEPC. Specifically, it is able to directly affect tNEPC tumor cell survival and metastases and modulate immune system activity against tNEPC, as described below.

- **Inhibiting tNEPC Growth Factor NPY.** tNEPC is believed to be caused by neuroendocrine cells in the prostate that overexpress NPY. NPY activates the specific G protein-coupled receptor Y1-R, which then selectively stimulates growth of AR-independent, tNEPC-like cancer cells, while reducing growth in AR-dependent cells. NPY is a substrate of DPP 8/9, which cleaves it into biologically active forms. DPP 8/9 inhibition in tumor cells decreases the number of viable tumor cells by reducing NPY cleavage.

- **Inhibiting the Formation of tNEPC-type (Osteoclastic) Bone Metastasis.** Prostate cancer is characterized by the presence of bone-forming (osteoblastic) metastasis. In contrast, tNEPC is associated with bone-lysing (osteoclastic) metastasis. BXCL701 is designed to block the bone destruction by osteoclasts through the inhibition of osteoclast differentiation. In an animal model that recapitulated the formation of osteolytic metastasis of tNEPC, BXCL701 was observed to reduce osteoclast activity, bone resorption and tumor burden based on information in an article published in the British Journal of Haematology in 2009 by Pennisi et al.

- **Exhibiting Immuno-mediated Activity Against tNEPC.** BXCL701 may potentially have the ability to modulate the immune system in multiple ways based on information in an article published in the Journal of Cancer Research in 2004 by Adams et al. and an article published in PLOS One in 2013 by Walsh et al., several of which are relevant to its ability to treat tNEPC, including:

    - stimulating the activation of multiple immune cell types;
    - stimulating tumor cell killing by inducing the priming, migration and cytotoxicity of T-cells and the formation of memory T-cells;
    - stimulating tumor cell killing by inducing the proliferation and activation of neutrophils;
    - inhibiting the immune suppressive FAP+ CAF and MDSC and delaying or preventing tNEPC development; and
    - synergistically increasing checkpoint inhibitor anti-tumor activity.

The figure below summarizes the complex, multifaceted immuno-mediated mechanism of BXCL701. Through this mechanism, BXCL701 induces an immuno-permissive tumor microenvironment as it stimulates the priming, migration and cytotoxicity of pro-inflammatory cells while dampening the immuno-suppressive phenotype of negative regulatory cells through a unique cytokine and chemokine cascade.

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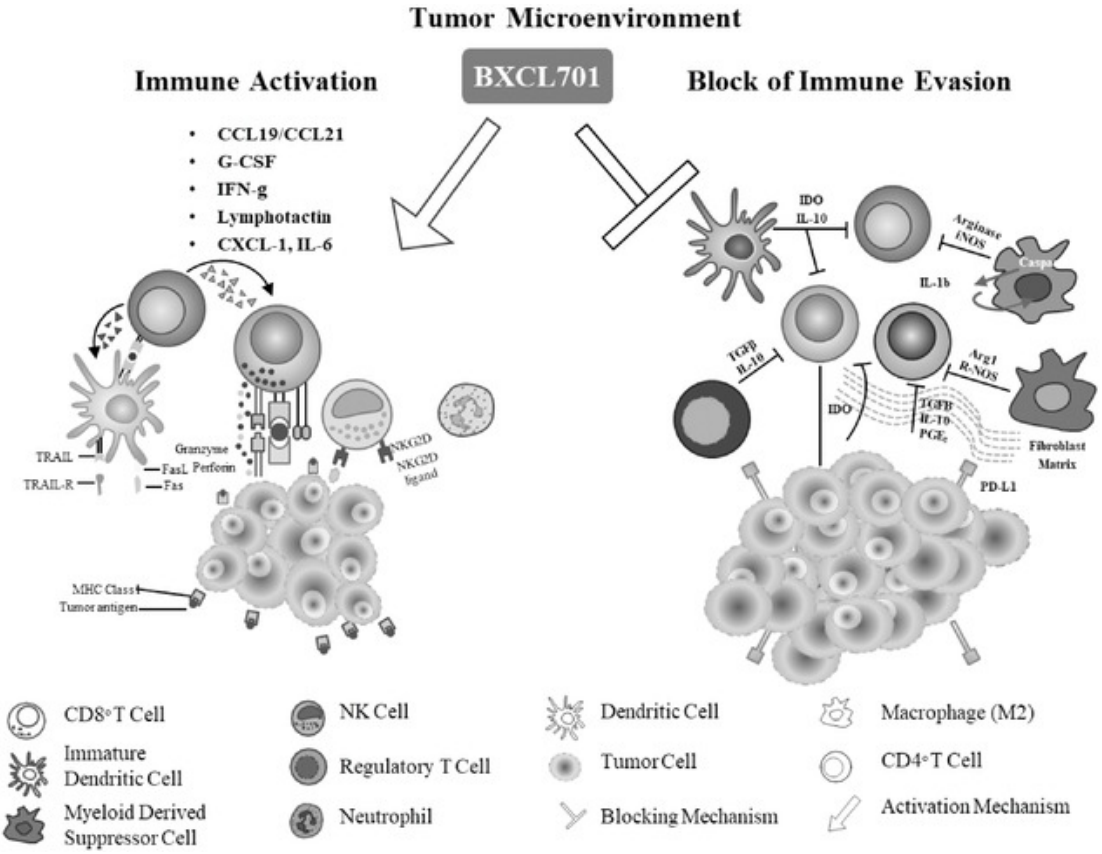

Figure 11. BXCL701 mechanism of action induces activation of the immune system via its stimulation of T-cells, NK cells and neutrophils which are then able to kill tumor cells. At the same time, BXCL701 blocks the immuno-evasion function of certain suppressor cells (FAP+ CAF and MDSC).

There are numerous aspects of BXCL701's mechanism of action that potentially make it a strong and novel combination agent for checkpoint inhibitors. Several aspects have been clinically observed in cancer patients in addition to healthy volunteers in the trials discussed in the clinical section below and reported in Figures 13a and 13b. BXCL701 has been shown to:

- induce wide spectrum cytokines and chemokines in humans, which was observed in healthy volunteer trials (CA168-001, CA168-002), a neutropenia trial (PTH-101), and in a single agent trial in melanoma (PTH-301);
- induce neutrophil/granulocyte proliferation and infiltration into tumors in humans, which was observed in healthy volunteer trials (CA168-001, CA168-002) and in a neutropenia trial (PTH-101);
- stimulate cytotoxic T-cells in humans, which was observed in healthy volunteer trials (CA168-001, CA168-002), and observed in a cancer trial combination of Rituxan and BXCL701 (PTH-203); and
- display direct, single agent anti-tumor effect in humans, which was observed in a melanoma trial (PTH-301).

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We sponsored and conducted a preclinical study of BXCL701 in 2016 as a single agent and in combination with Keytruda to test our hypothesis that combining BXCL701 with checkpoint inhibitors will result in synergistic anti-tumor activity. As shown in the figure below, the combination of Keytruda with BXCL701 produced better tumor control than either agent as a single agent.

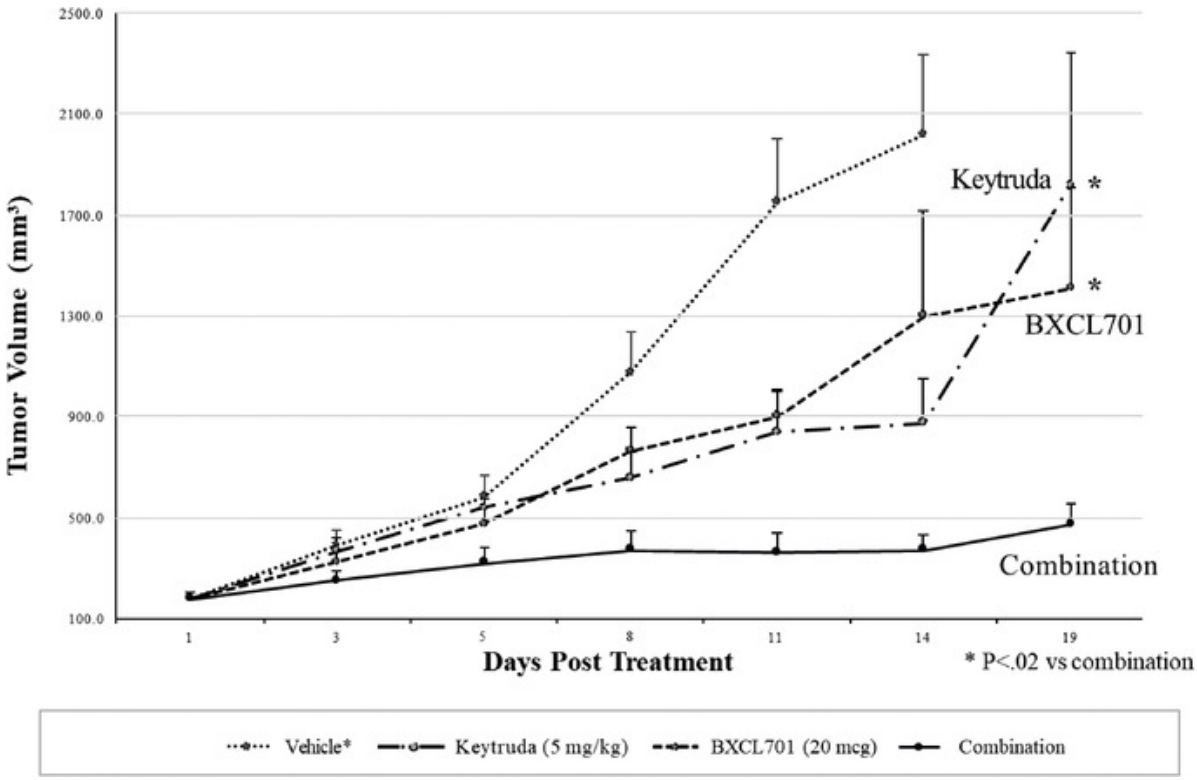

Figure 12. The combination of BXCL701 and a mouse surrogate of Keytruda was tested in MC38 mouse cancer model. Groups of animals were dosed with either BXCL701, Keytruda or the combination of both. The combination achieved a greater reduction in tumor value than either a single agent treatment with significant P-value (<0.02) for both.

The potential of this combination to enhance anti-tumor activity was also observed in a second, more aggressive mouse cancer model, where single agent treatment with Keytruda and BXCL701 alone had no effect while the combination inhibited tumor growth. The molecular and cellular mechanism by which the combination is synergistic has revealed how this synergy could be achieved. At the molecular level, several cytokines known to have strong anti-tumor activity such as Il-2 (an approved immunotherapy), IL-12 and granulocyte-macrophage colony-stimulating factor, appear to be synergistically up-regulated. Also, the combination synergistically increased CXCL9/MIG, which attracts T-effector cells into the tumor. At the cellular level, the combination mobilized activated tumor killing NK cells (expressing perforin and granzyme), from the blood to the tumor. At the same time, treatment with BXCL701 blocked relocation of immuno-suppressive T-regulatory cells to the tumor, an effect that is normally induced by treatment with immune checkpoint inhibitors.

The data and rationale presented above support the use of BXCL701 in combination with Keytruda in tNEPC. This combination could potentially offer tNEPC patients the deep and durable responses and increased survival that has been observed in other tumors upon treatment with immuno-oncology agents. In clinical data to date, BXCL701 has provided evidence of being well tolerated with no overlapping adverse events with checkpoint inhibitors, limiting potential toxicity of the combination.

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Summary of Existing BXCL701 Clinical Data (Previously Studied as Talabostat)

BXCL701 has been tested in multiple clinical trials, providing evidence of being well tolerated, proof of mechanism, and single agent anti-tumor activity in patients with melanoma, an immuno-sensitive tumor. BXCL701 was originally developed by Point Therapeutics, Inc. as Talabostat (PT-100).

The details of each of the trials conducted with Talabostat to date, including the date(s) and any results for efficacy endpoints, are disclosed in the figures below: Figure 13a, for dose finding and human pharmacology studies, and Figure 13b for Phase 2 and Phase 3 clinical trials.

Study # / Title dates/duration	Daily Doses (µg)	Number of patients who received Placebo	Number of patients who received Talabostat	Key observations and/or endpoints
CA168-001: SDT 10/1999 to 12/1999	10-2400	18	54	Target inhibition at doses ≤ 150 µg, Effects on PD seen ≥ 1200 µg.
CA168-002: MDT 2/2000 to /2000	25-1800	12	36	Target inhibition at doses ≥ 100 µg. Effects on PD seen ≥ 600 µg
Children with relapsed/refractory solid tumors 12/2005 to 4/2007	100, 200, 350, 600 (in µg/m2/day)	N/A	6	Target inhibition at doses ≥ 100 µg
PTH-101: Solid tumors receiving myelosuppressive therapy 8/2001 to 8/2003	100, 200, 400, 800, 1200	N/A	34	Observed reductions in grade 4 neutropenia at doses from 200 to 800 µg. Slight food effect on rate of absorption
PTH-103: Food Effect 8/2004 to 11/2004	200, 300, 400	6	18	No food effect on pharmacokinetics.
PTH-104: Antacid Effect 9/2004 to 11/2004	200, 300, 400	6	18	The co-administration of antacid did not impact pharmacokinetics
PTH-105: Relative BA of Talabostat Oral tablet & Oral solution 4/2006 to /2006	300	N/A	12	The oral formulation did not substantially impact overall exposure
PTH-106: Impaired Renal function 1/2007 to 4/2007	600	N/A	7	Study terminated early due to closure of project
PTH-201: B-Cell Malignancies (w/rituximab) 6/2003 to 12/2004	400, 600, 800	N/A	20	2 Patients (2/20; 10%) Partial response. 600 µg determined to be the Maximum Tolerated Dose (MTD).
	Total Patients (Phase I)	42	205	

Figure 13a. Summary of Talabostat Phase 1 and Human Pharmacology Studies.

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Study # / Title dates/duration	Daily Doses (µg)	Number of patients who received Placebo	Number of patients who received Talabostat	Key observations and/or endpoints
PTH-203: CLL (w/rituximab) 7/2004 to 12/2006	600	N/A	53	PR in 7 patients (ORR- 7/53= 13.2%)
PTH-301: Melanoma single-agent 7/2004 to 2/2006	600, 800	N/A	42	CR in 1 patient, PR in 1 patient
PTH-302: NSCLC (w/docetaxel) 3/2004 to 3/2006	400, 600	N/A	55	2CR, 3 PR (response rate = 9.1% (5/55)).
PTH-303: Melanoma (w/cisplatin) 5/2004 to 12/2006	600, 800	N/A	74	PR in 6 patients (response rate= 8.1%)
PTH-304: NSCLC (w/docetaxel) 2/2006 to 6/2007	400, 600	60	65	PFS and OS were significantly reduced following administration of Talabostat versus placebo. Trial was halted and the whole clinical program placed on hold.
PTH-305: NSCLC (w/pemetrexed) 2/2006 to 6/2007	400,600	136	138	OS was lower in Talabostat vs placebo. Trial was halted and the whole clinical program placed on hold.
PTH-320: Pancreatic (w/gemcitabine) 6/2005 to 6/2007	400, 600	N/A	68	3 PR, 1 CR (ORR of 5.88%).
Metastatic Colorectal Cancer 3/2005 to 2/2006	400, 600, 800	N/A	28	Trial was halted and whole clinical program placed on hold No Objective response. Observed Target inhibition by more than 90% within 1 week of administration and was sustained throughout treatment
	Total (Phase 2 +3)	196	524	

Figure 13b. Summary of Talabostat Phase 2/3 Studies.

The most frequently observed adverse events of all grades across studies that we believe are possibly related to Talabostat were edema/peripheral swelling (228 patients or approximately 39.1% of patients), hypotension (55 patients or approximately 9.4% of patients), dizziness (93 patients or approximately 15.9% of patients), hypovolemia (52 patients or approximately 8.9% of patients), fatigue (215 patients or approximately 36.9% of patients), nausea (181 patients or approximately 31.1% of patients), vomiting (85 patients or approximately 14.6% of patients), pyrexia (92 patients or approximately 15.8% of patients), rigors (56 patients or approximately 9.6% of patients) and rash (59 patients or approximately 10.1% of patients) and they were generally manageable and reversible. The most frequently observed serious adverse events related to Talabostat administered as a single agent were edema (33% to 66% across all grades with three instances of grade 3) and fatigue (50% to 65% across all grades with a single instance of grade 4).

Of the 25 colorectal cancer patients who received Talabostat alone at 200µg twice daily, the most common side effects during treatment were fatigue (54%), edema (46%), nausea/vomiting (36%) and anorexia (39%). Six patients required dose reductions due to toxicity: three for grade 3 toxicities (one for headache/syncope, one for edema, and one for alkaline phosphatase/ transaminase abnormalities) and three for grade 2 toxicities (one for rash, one for fever, and one for fatigue). Anemia and thrombocytopenia were observed in nine (32%) and six (21%) patients respectively. The cause of many side effects was difficult to differentiate between Talabostat toxicities and the symptoms of progressive

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disease. Edema was most commonly seen on the extremities, and usually did not require intervention or dose reduction (eleven grade 1, one grade 2, one grade 3). Eight patients died either while receiving study drug or within 30 days of stopping usage: 6 from disease progression, one from Talabostat related toxicity (renal failure; patient received 400mcg twice daily), and one from unrelated causes.

Of the 42 patients with Stage IV metastatic melanoma given Talabostat alone at 300µg twice daily, the most frequently reported adverse events were fatigue (64.3%), edema (54.8%), nausea (26.2%), dizziness, and vomiting (both at 23.8%). In terms of grade 3 and 4 events, grade 3 peripheral edema, vomiting, respiratory distress and gastrointestinal hemorrhage were each reported in 2 (4.8%) patients and grade 3 dyspnea was reported in 3 (7.1%) patients. Grade 4 hypovolemic renal failure (likely secondary to edema and third-spacing of fluid) was reported in one patient.

Across all clinical studies of Talabostat, the serious adverse events which occurred in greater than 5% of patients and were classified as grade 3 or 4 were: PTH-101 study (Phase I): neutropenia and febrile neutropenia (4 patients or approximately 11.8% of patients, each), syncope and leukopenia (3 patients or approximately 8.8% of patients, each), weakness, anemia and hypotension (2 patients or approximately 5.9% of patients, each); PTH-201 study (Phase I), Grade 3: dizziness (3 patients or approximately 15% of patients), thrombocytopenia, blood CPK increased (2 patients or approximately 10% of patients, each), neutropenia, fatigue, edema, pyrexia, infection NOS, eosinophil count increased, electrolyte imbalance, myalgia, rhabdomyolysis, adenocarcinoma, tumor lysis syndrome, syncope, pollakiuria, renal failure NOS, face edema (1 patient or approximately 5% of patients, each); PTH-203 study (Phase II): febrile neutropenia and dyspnea (5 patients or approximately 9.3% of patients, each), neutropenia (4 patients or approximately 7.4% of patients); thrombocytopenia and fatigue (3 patients or approximately 5.6% of patients, each); PTH-301 study (Phase II) Grade 3: dyspnea (3 patients or approximately 7.1% of patients); PTH-302 study (Phase II): neutropenia (23 patients or approximately 41.8% of patients), leukopenia (6 patients or approximately 10.9% of patients), febrile neutropenia and dyspnea (5 patients or approximately 9.1% of patients, each), asthenia and fatigue (4 patients or approximately 7.3% of patients, each), thrombocytopenia, pneumonia NOS and hypovolemia (3 patients or approximately 5.5% of patients, each); PTH-303 study (Phase II): thrombocytopenia (7 patients or approximately 9.5% of patients), fatigue (6 patients or approximately 8.1% of patients), vomiting (5 patients or approximately 6.8% of patients) and hypotension, dehydration, neutropenia and nausea (4 patients or approximately 5.4% of patients, each); PTH-304 study (Phase III): dyspnea (7 patients or approximately 7.7% of patients), pleural effusion and neutropenia (4 patients or approximately 6.2% of patients, each); PTH-320 study (Phase II): deep vein thrombosis (4 patients or approximately 5.9% of patients); Study of single agent Talabostat in metastatic colorectal cancer (Phase II): transaminase elevation (2 patients or approximately 7.1% of patients); PTH-305 study (Phase III) did not report any grade 3 or 4 adverse events occurring in at least 5% of patients.

These studies refer to the standardized definitions published by The National Cancer Institute of the National Institutes of Health for adverse events to describe the severity of organ toxicity for patients receiving cancer therapy. Grade refers to the severity of the adverse event, with grade 1 adverse events generally including mild or asymptomatic conditions or clinical or diagnostic observations only, grade 2 adverse events generally including moderate events or minimal, local or noninvasive intervention events, grade 3 adverse events generally including severe or medically significant events that are not immediately life-threatening, events requiring hospitalization or prolongation of hospitalization or disabling events and grade 4 adverse events generally including life-threatening consequences or urgent intervention events. The Medical Dictionary for Regulatory Activities, or MedDRA, was used throughout the trials to code reported adverse event terms. In some cases, however, terms were more narrowly defined than others. For example, a standard MedDRA term would be "edema, peripheral," where the corresponding broader terms used in these studies would be simply "edema" or "edema, not otherwise specified."

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Nine of BXCL701's clinical trials were dose finding and human pharmacology studies, which we have leveraged to define the dosing regimen for our clinical trials. The data obtained in these trials provides a comprehensive overview of safety, PK and full target inhibition plus downstream PD effect on cytokine increase and neutrophil stimulation. In addition, several foundational human pharmacology studies were conducted, including relative bioavailability, food effect and anti-acid effect. The key findings from these trials that we believe are relevant to the further development of BXCL701 include:

- predictable and dose proportional PK;
- maximum tolerated dose, or MTD, of 300 mcg dosed twice a day or 600 mcg dosed once a day; and
- target inhibition observed in human subjects with doses above 100 mcg.

Given these data and the strong anti-tumor activity observed in the preclinical studies, the focus shifted to oncology where the agent was tested in six Phase 2 and two Phase 3 clinical trials involving more than 500 patients. These trials provided an important understanding of the behavior of BXCL701 in cancer patients and provided the following key conclusions enabling us to pursue further development:

- evidence that the drug has anti-tumor activity as a single agent in an immuno-sensitive tumor (melanoma);
- recommended safe and tolerable dose to use in our planned Phase 2 efficacy trial; and
- well-defined adverse event profile, that does not overlap with checkpoint inhibitors, which we believe thereby avoiding the need for lengthy dose escalation in the combination arm of our Phase 2 trial.

A wide range of doses between 100 and 600 mcg administered once or twice daily were studied in these trials and the MTD was determined to be 600 mcg administered once daily. Anti-tumor activity was observed both as single agent and in combination in refractory solid tumors. The most frequent adverse events attributable to BXCL701 were fatigue, edema, dizziness, nausea, vomiting and fever. Edema was dose-related and probably related to a mild capillary-leak syndrome secondary to cytokine up-regulation. The edema observed in clinical studies to date has generally resolved within four to five days of interruption of BXCL701 treatment; patients have resumed BXCL701 either without further occurrence of edema or to a lesser degree of recurrence. While objective clinical responses were seen as single agent and in combination in refractory patients, we believe BXCL701's immuno-modulatory activity was most likely limited by the effect of immune checkpoint expression in the tumor. In addition, most of the BXCL701 trials were conducted in combination with cytotoxic agents, which are generally immuno-suppressive. Therefore, we believe these combinations did not optimally leverage BXCL701's immuno-stimulatory prospects.

Point Therapeutics, Inc. (acquired by Midatech Pharma USA, Inc.) terminated the development of BXCL701 after an interim analysis of the two Phase 3 trials showed that the primary and secondary efficacy endpoints would not be met in non-small cell lung cancer, or NSCLC. In the BXCL701 combination trial with docetaxel (PTH-304), the BXCL701 arm of the study showed higher patient mortality than the placebo arm, which caused the FDA to place Point Therapeutics, Inc. IND for BXCL701 on clinical hold on May 21, 2007 (thereby putting on hold all ongoing clinical trials (PTH-304, PTH-305 and PTH-320)), which remained in place at the time Point Therapeutics ceased development of Talabostat and terminated all clinical trials. We undertook a complete analysis of the clinical data of both Phase 3 trials and concluded that BXCL701 did not contribute to the excess mortality results. Rather, we attributed the observed mortality to a statistical imbalance in the randomization of subjects with more advanced disease in the BXCL701 arm. The second NSCLC study, in combination with pemetrexed, conducted in a similar patient population and often in the same clinical site as the BXCL701 combination trial with docetaxel, did not show the same excess mortality. We shared our analysis with the FDA in a pre-IND meeting and in a follow-up type C meeting, who

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acknowledged our conclusion but indicated the data available could not rule out potential safety issues. However, the agency stated that our plan to initiate clinical trials with BXCL701 appeared reasonable and that it has no objection to our approach to combine BXCL701 with checkpoint inhibitors. As a result, we do not believe that the FDA's clinical hold on Point Therapeutics' IND will affect our proposed plans.

Taken together, we believe this extensive set of clinical data covering safety, PK parameters, target inhibition and downstream PD effect and anti-tumor activity, coupled with the genomic and mechanistic work gave us the confidence to build our BXCL701 clinical program.

BXCL701 Clinical Program in tNEPC

We anticipate initiating a Phase 2, two-arm, open label, clinical trial testing BXCL701, as both a single agent and in combination with Keytruda, in patients with tNEPC that have progressed on Zytiga or Xtandi and who had previously been treated with chemotherapy.

Based on preclinical and clinical data, we plan to use a dose of 600 mcg, administered once daily, in both arms of our Phase 2 trial. This dose was previously found to be well tolerated, to inhibit the DPP 8/9 and FAP targets and to stimulate the immune system. In addition, the gene expression for DPP 8 and DPP 9 will be analyzed retrospectively. tNEPC patients are characterized by the presence of soft tissue metastasis that is amenable to biopsy. We expect that patients with tNEPC will show high levels of expression of our biomarkers.

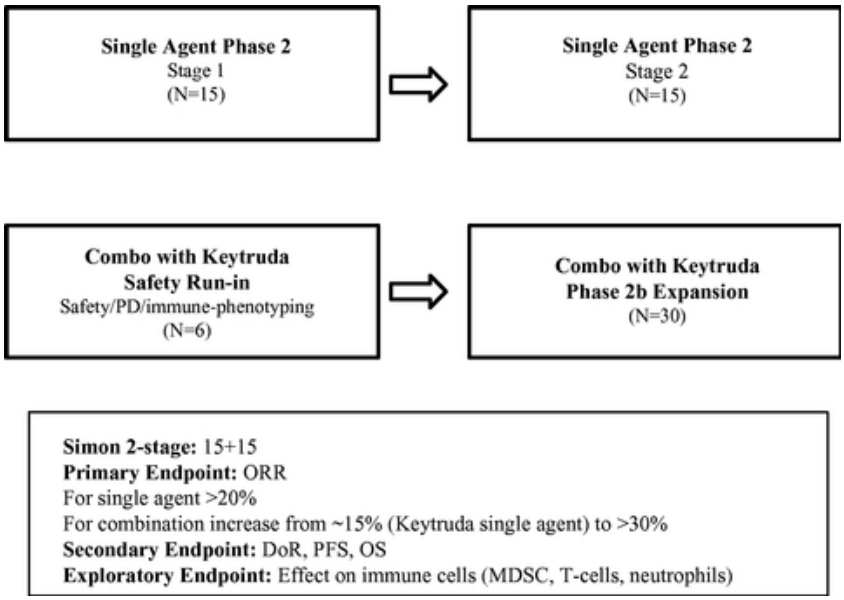

Figure 14. BXCL701 Phase 2 trial design.

As shown in the figure above, the study will consist of two arms:

- **Single agent arm.** Evaluate BXCL701 activity with a Simon 2 stage approach, 15+15 patients. The predictive power of DPP expression as a biomarker will be assessed during the first stage to decide whether it can be used to prospectively select patients for inclusion in the second stage of this trial.

- **Combination arm.** This will consist of a safety run-in that will examine the safety and tolerability of combining BXCL701 and Keytruda in six tNEPC patients. Patients will be dosed with BXCL701 once daily on Days 1-14 of a 21-day cycle plus IV administration of 200 mcg of Keytruda on Day 8 every 21 days. The 7-day BXCL701 rest period is to optimize immune system stimulation. If dose limiting toxicities, or DLTs, are observed during the safety run-in, 400 mcg once daily will be tested.

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The Phase 2 primary endpoint will be objective response rate, or ORR. The secondary endpoints will be duration of response, or DoR, progression-free survival, or PFS, and overall survival, or OS. We expect this trial to take approximately two years to complete and to have preliminary data in the first half of 2019 for the single agent arm. The Keytruda prostate cancer single agent trial (Keynote 199), which includes a subset of tNEPC patients, will represent the reference trial to determine the relative range of our primary endpoint.

We plan to request orphan drug designation and breakthrough therapy designation for neuroendocrine prostate cancer as soon as we obtain relevant preliminary efficacy data. We will plan our follow-on clinical strategy based on the results of the PoC trial and discussions with the FDA. The FDA has granted accelerated approval to drugs in tumors like tNEPC that have no available therapies and represent a high unmet medical need based on single arm, ORR-based large Phase 2 or even expanded Phase 1 trials. Therefore we believe there is potential for an accelerated path to approval for BXCL701 if this initial trial shows a relevant percentage of durable responses.

BXCL701 for the Treatment of Pancreatic Cancer

Pancreatic Cancer Overview and Market Opportunity

Pancreatic adenocarcinoma, more commonly referred as pancreatic cancer, represents one of the highest unmet needs in oncology. The American Cancer Society estimates that in 2017 there will be approximately 53,000 new diagnoses and 43,000 deaths. Pancreatic cancer has a median five-year survival rate of only about 8%. Recently, several new therapies have been developed consisting of new formulations of approved chemotherapies. However, these new therapies have limited efficacy with relatively short survival advantages, and well-known toxicities. It is well understood that the development of new efficacious drugs with manageable toxicity is required to achieve durable responses and increase survival in pancreatic cancer. Pancreatic cancer is thought to be a highly immuno-resistant tumor. Multiple attempts to show anti-tumor activity of immunotherapies including immune checkpoints have failed due to primary resistance mechanisms. We believe BXCL701 has the potential to eliminate the resistance to immune checkpoint inhibitors (to convert "cold" tumors "hot") and the combination with Keytruda could generate long and profound responses and the survival increase needed to make a true breakthrough in the treatment of pancreatic cancer.

Abraxane, a new formulation of the chemotherapy agent paclitaxel in combination with gemcitabine, is considered to be the standard of care for newly diagnosed pancreatic cancer in U.S. markets, with annual sales of almost $1 billion. Onivyde, a liposomal formulation of the chemotherapy agent irinotecan, was recently approved for use in second-line pancreatic cancer based on a two-month survival increase (six months vs. four months) and only 7.7% ORR, with annual sales of approximately $80 million. Our initial clinical development plan will target second-line or later pretreated patients, specifically the 50% that remain in good clinical condition after first-line treatment and thus may receive one or more subsequent lines of chemotherapy. Therefore, we believe that the potential number of patients treatable with the combination of BXCL701 and Keytruda, if successfully developed and approved, would be approximately 20,000.

Pancreatic cancer is a high unmet medical need, where approved therapies have limited activity and patients have short survival. In addition, as shown previously in Figure 11 summarizing the genetic data from the The Cancer Genome Atlas database, among all the tumor datasets available for analysis, a high level of overexpression and amplification of DPP 8/9 and FAP is present in pancreatic cancer. Pancreatic cancer is also characterized by the presence of the immuno-suppressive FAP+ CAF. As in tNEPC, single agent immune checkpoint inhibition has not shown single agent anti-tumor activity in pancreatic cancer patients, indicating the need for a molecule like BXCL701 to optimize their activity. Preclinical studies indicate that the combination of FAP and immune checkpoint inhibition is active.

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BXCL701 has been granted orphan drug designation from the FDA for the treatment of pancreatic cancer.

FAP Role in Pancreatic Cancer

Pancreatic cancer is characterized by dense fibrotic stroma called desmoplasia (consisting mostly of FAP+ CAFS), which can comprise as much as 90% of tumor mass. It is widely believed that drugs have not been effective in treating pancreatic cancer primarily due to the stroma impeding their ability to penetrate the tumor. As depicted in the figure below, FAP+ CAFs mediate immuno-suppression by producing the chemokine (C-X-C motif) ligand 12 (CXCL12) which binds to the CXCR4 receptor on T-cells. As a result, T-cells are excluded from the tumor and are prevented from killing the tumor cells. As a result of the immuno-suppressive microenvironment driven by FAP+ CAF and MDSC, pancreatic cancer is thought to be the prototypical "cold" tumor. This results in primary resistance to immune checkpoint single agent treatment and limited objective responses.

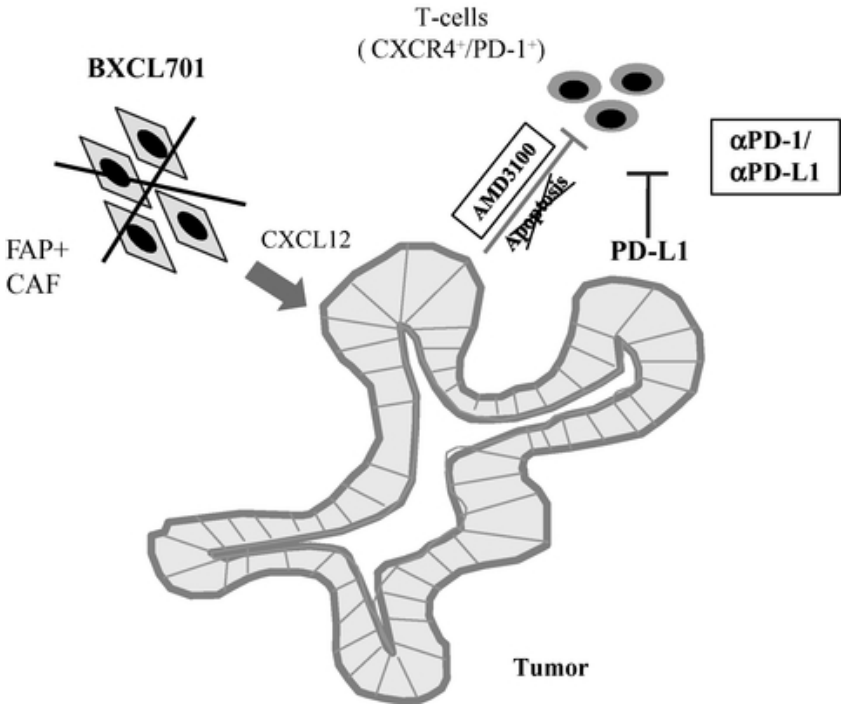

Figure 15. Model of the immuno-suppressive action of FAP+ CAF via secretion of CXCL12 blocking the entrance of T-cells in the tumor. By blocking FAP+ CAF activation, BXCL701 permits T-cells to penetrate the tumor and kill the tumor cells.

Several publications have shown that inhibiting or blocking the activity of these FAP+ CAFs results in decreased tumor growth, including: (i) an article published in Cancer Research in 2015 by Lo et al., (ii) an article published in BMC Gastroenterology in 2015 by Kawase et al. and (iii) an article published in JCI Insight in 2017 by Lo et al. Additionally, preclinical studies have demonstrated that eliminating FAP+ cells combined with the administration of CTLA-4 or PD-L1 acts synergistically to decrease pancreatic cancer growth in animal models as based on information in an article published in the Proceedings of the National Academy of Sciences of the United Stated of America in 2013 by Feig et al. As a result, these studies indicate that the FAP+ cells may contribute to the resistance to these checkpoint antagonists. BXCL701, which inhibits FAP+ CAF, has been shown to decrease tumor growth of human pancreatic tumors in animal models both as a single agent and in combination with Keytruda. In addition, under a collaboration with Nektar Therapeutics, Inc., or Nektar, a triple

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combination of BXCL701 with Keytruda and Nektar's NKTR-214 compound was tested in a Pan02 mouse model of pancreatic cancer. Nektar's NKTR-214 compound is a CD122-biased agonist designed to grow specific cancer-killing T-cells and natural killer cell populations in the body which fight cancer as a third non-immuno-checkpoint, immunotherapy agent. As shown in Figure 16a, this triple combination resulted in complete tumor regression in the mice treated. Most of these mice became resistant to re-challenge with new tumors injected more than two months after dosing was stopped, indicating that memory T-cells formed. Observations from this re-challenge experiment were consistent with previous data that BXCL701 has the potential to induce memory T-cells through the induction of IL-15 and IL-7, as published in the Journal of Cancer Research in 2004 by Adams et al. along with new data developed by us. The formation of memory T-cells in humans could translate into durable and profound anti-tumor responses. Immunohistochemistry, or IHC, of the tumors from satellite animals sacrificed on day 3 of the study revealed that BXCL701 significantly reduced FAP expression, and that the double or triple combination therapies containing BXCL701 and Nektar's NKTR-214 had a stronger FAP reduction as shown in Figure 16b. The triple combination therapy increased the number of immune cell infiltrates in the tumor, especially the number of neutrophils as expected based on the previously generated preclinical and clinical data of BXCL701. The triple combination therapy was well-tolerated by the animals. The results suggest that removal of fibrotic barriers to immune infiltration is an important mechanism for overcoming immune escape by tumors otherwise resistant to immune therapy. These results provide therapeutic rationale for treatment of pancreatic cancer patients with this triple combination therapy. Given that the combination of NKTR-214 with anti-PD1 has shown anti-tumor activity in human in PD-L1 negative patients, we believe that the triple combination has the opportunity to further expand the pool of cancer patients that might respond to immunotherapy. We believe the results of this experiment support our belief that BXCL701 is a combination agent that has the potential to improve the anti-tumor activity of immunotherapies beyond immuno-checkpoints. We are not aware of any clinical stage FAP inhibitor competitors of BXCL701.

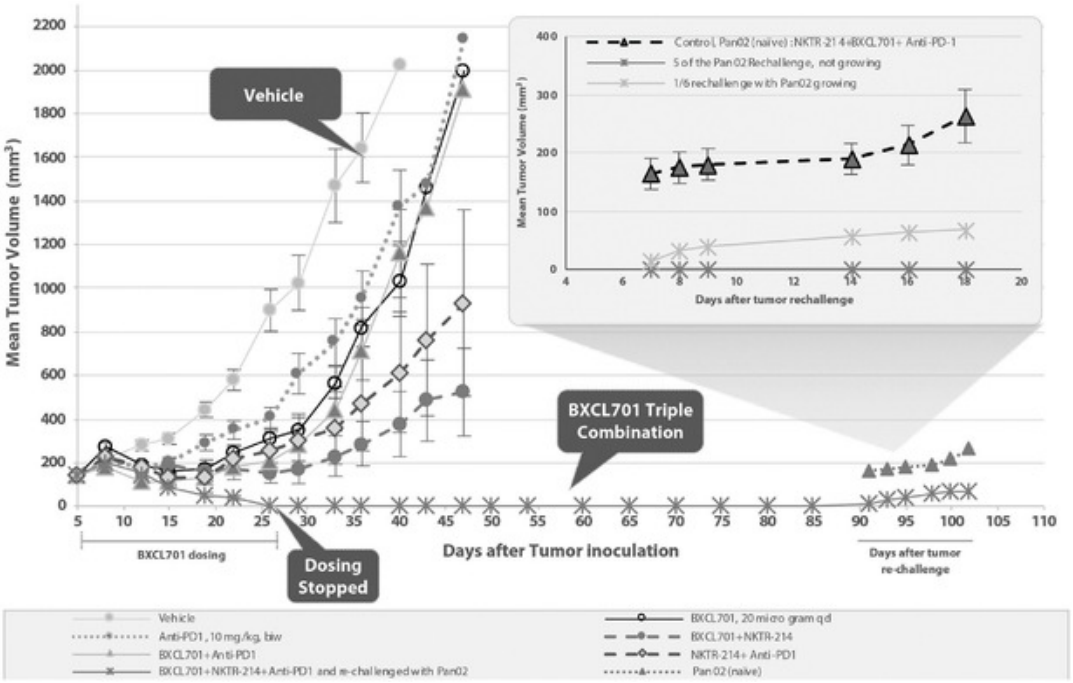

Figure 16a. The triple combination of BXCL701, a mouse surrogate of Keytruda and NKTR-214 was tested in Pan02 mouse model of pancreatic cancer. The triple combination achieved complete tumor regression that was maintained even after dosing stopped.

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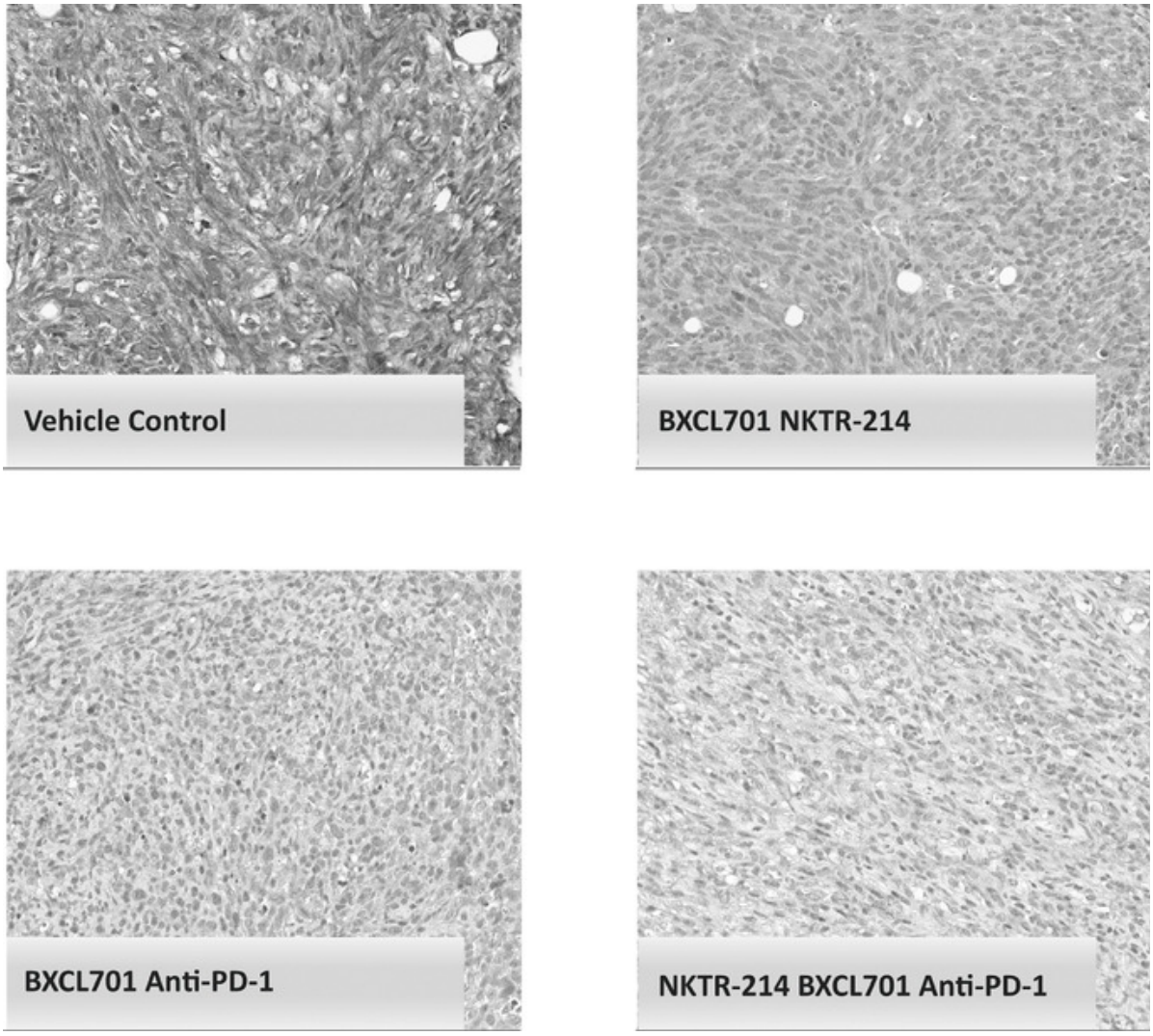

Figure 16b. IHC for BXCL701 target FAP revealed that BXCL701 significantly reduced FAP expression and that the double combination or triple combination containing BXCL701 and NKTR-214 had a stronger reduction.

BXCL701 Clinical Program in Pancreatic Cancer

The role of FAP+ CAF in mediating immuno-suppression has been well documented by leading investigators, including Dr. Louis Weiner, currently a director at the Lombardi Cancer Center at Georgetown University. We are collaborating with Dr. Weiner and his team to further characterize the activity of BXCL701 in the context of immune checkpoint resistance in combination with Keytruda.

As shown in the figure below, the clinical development plan for BXCL701 in pancreatic cancer, developed in collaboration with Dr. Weiner, will consist of two overlapping trials. We plan to initiate two Phase 2 open label trials with BXCL701 in patients with metastatic pancreatic cancer. The first trial will examine BXCL701 in the neoadjuvant setting (before surgery). We expect to enroll ten patients who will be treated for three weeks with BXCL701 before surgery. The trial will examine immune cell infiltration and activation and is expected to commence in the second half of 2018 with

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results available in the first half of 2019. The second trial will examine BXCL701 in combination with Keytruda in approximately 30 patients that have previously received gemcitabine. We expect the second trial to commence in the second half of 2018 with preliminary results available in the first half of 2019. We believe this trial, if successful, could lay the foundation for a potential follow up registration trial in pancreatic cancer.

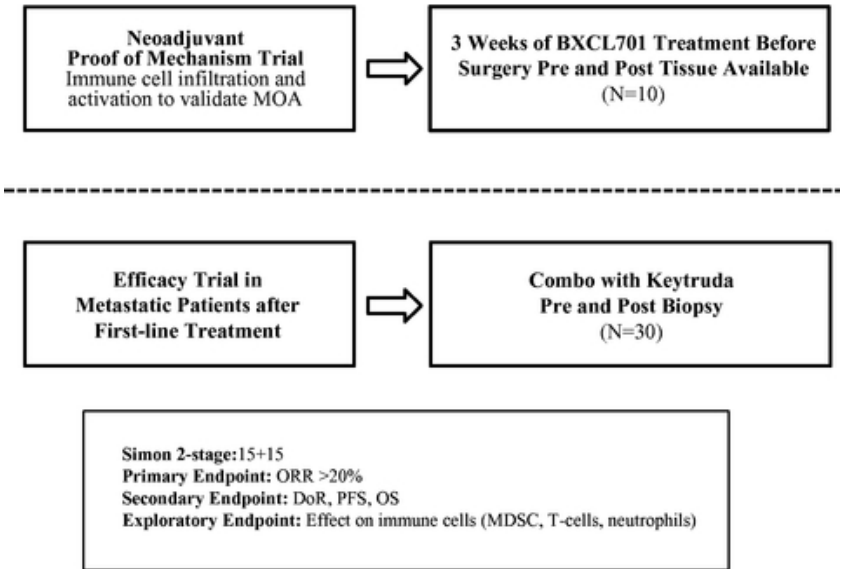

Figure 17. BXCL701 Phase 2 trial design in pancreatic cancer as a single agent (proof of mechanism of action, or MOA) and in combination with Keytruda (PoC).

Other Immuno–oncology Indications

In addition to tNEPC and pancreatic cancer, we plan to leverage our existing preclinical and clinical data to identify other cancer types with high unmet medical need that would benefit from BXCL701's novel mechanism of action. We are prioritizing those where the immuno-suppressive microenvironment is driven by the molecular and cellular targets of BXCL701 and where the single agent activity of approved immune checkpoint inhibitors is limited.

In addition, based on the mechanism of action described in the figure below, we believe BXCL701 provides a platform for combination with immunotherapy modalities that go beyond the currently approved immune checkpoint agents that target the PD-1/PD-L1 axis. Following our PoC trials, we plan to conduct clinical trials covering a broad range of additional combinations with other immunotherapy agents including:

- immune checkpoint inhibitors (other than PD-1/PD-L1);
- cellular therapies (CAR-T and chimeric antigen receptor natural killer cells);

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- therapeutic vaccines; and/or
- antibody-dependent cell-mediated cytotoxicity, or ADCC, driven monoclonal antibodies.

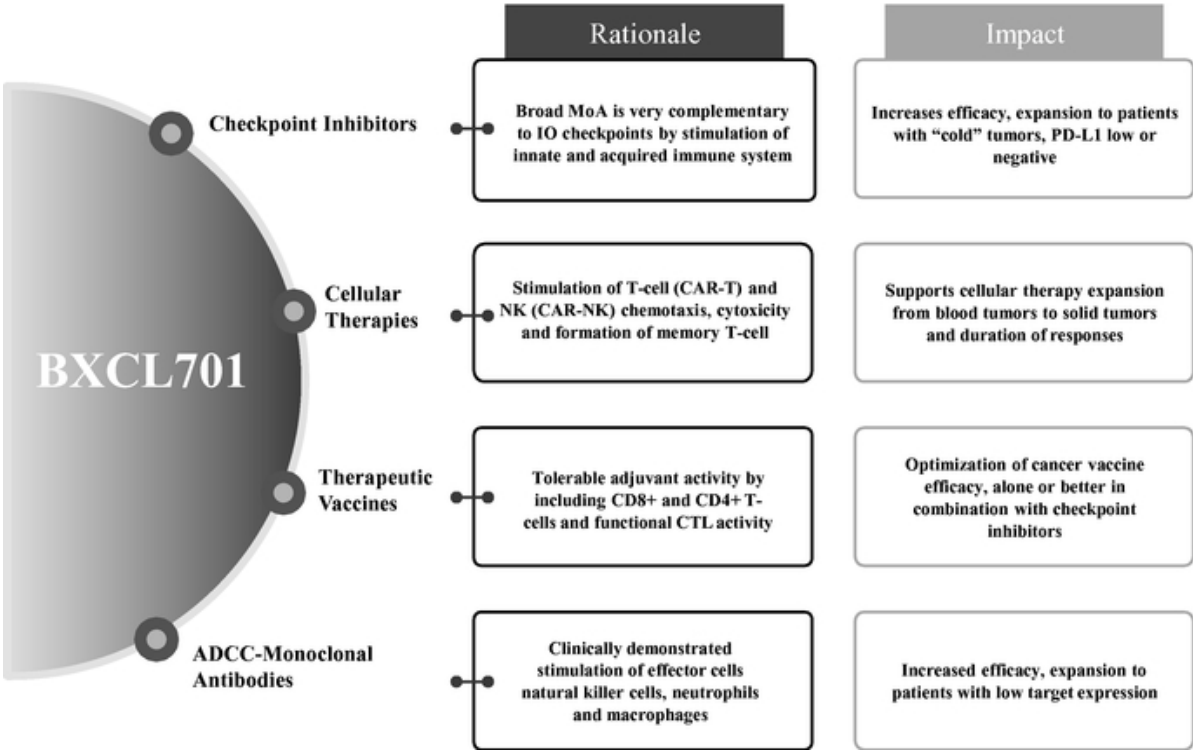

Figure 18. Mechanism of action-based rationale and impact for combination with BXCL701 and immunotherapy modalities beyond anti-PD-1/PD-L1.

Other Product Candidates

Neuroscience Program

We are targeting neuroscience disorders where there is high unmet medical need and therefore a requirement for symptom management is a priority (like agitation, seizures, dyskinesias) as well for transformative care for monogenic rare CNS disorders.

For symptomatic approaches, our neuroscience program is developing a FDA Section 505(b)(2) opportunities with a focus on treating symptoms for various neurological and psychiatric disorders. This entails re-innovating existing agents through formulation changes and deuteration. The utilization of EvolverAI has identified several monogenic diseases with available animal models across rare neuroscience diseases. We utilize proprietary algorithms to identify associated mechanisms with existing pharmacology to test whether these agents can improve the disease profile in the animal model either through disease modification or symptomatic manner. The agents identified must be those that we believe are Phase 2 ready with a potential for a short, cost-effective development plan (four to five years to NDA filing).

We have identified our next candidate as a FDA Section 505(b)(2) opportunity, BXCL502, for symptomatic improvement of a CNS disorder with a high unmet medical need. Additional product candidates are routinely screened, prioritized and selected using a combination of specific algorithms and relevant translation research, formulation and deuteration strategies.

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Immuno-oncology Program

Our immuno-oncology program is based on utilizing a comprehensive map of all known relationships that link immuno-evasion and immuno-activation pathways and targets with thousands of pharmacological agents and tumor indications. This comprehensive map has permitted us to select a potential pipeline of candidates based on our ability to alter the tumor micro-environment and the potential to address relevant unmet medical needs for various tumor types.

The lead candidates are clinically validated in oncology and therefore represent opportunities where we believe clinical development risk may be reduced.

BXCL702 is an example of the set of oncology candidates. BXCL702 is designed to have a dual anti-tumor mechanism of action: a direct mechanism to kill tumor cells and an indirect mechanism to stimulate the anti-tumor activity of immuno-therapy agents. We believe BXCL702 offers the opportunity to bring the benefit of immuno-oncology to hematological malignancies. Based on the preclinical and clinical supporting data, FDA granted BXCL702 orphan drug designation for the treatment of AML.

Competition

The pharmaceutical and biotechnology industries are characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products. The immuno-oncology, neuroscience and rare disease segments of the industry in particular are highly competitive. While we believe that our technology, development experience and scientific knowledge provide competitive advantages, we face potential competition from many different sources, including major pharmaceutical, specialty pharmaceutical and biotechnology companies, academic institutions and governmental agencies, and public and private research institutions.

Many of our competitors may have significantly greater financial resources and expertise in research and development, manufacturing, preclinical studies, conducting clinical trials, obtaining regulatory approvals and marketing approved medicines than we do. Mergers and acquisitions in the pharmaceutical, biotechnology and diagnostic industries may result in even more resources being concentrated among a smaller number of our competitors. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and in establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Smaller or early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

The key competitive factors affecting the success of all of our product candidates, if approved, are likely to be their efficacy, safety, convenience, price, the effectiveness of companion diagnostics in guiding the use of related therapeutics, if any, the level of generic competition and the availability of reimbursement from government and other third-party payors.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize medicines that are safer, more effective, have fewer or less severe side effects, are more convenient or less expensive than any medicines we may develop. Our competitors also may obtain FDA or other regulatory approval for their medicines more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. In addition, our ability to compete may be affected in many cases by insurers or other third-party payors seeking to encourage the use of generic medicines. There are many generic medicines currently on the market for certain of the indications that we are pursuing and additional generics are expected to become available over the coming years. If our therapeutic product candidates are approved, we expect that they will be priced at a significant premium over competitive generic medicines.

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Any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future. If the product candidates of our priority programs are approved for the indications for which we are currently planning clinical trials, they will compete with the drugs discussed below and will likely compete with other drugs currently in development.

Neurological and Psychiatric Disorders

Drugs used for the acute treatment of agitation resulting from psychosis in schizophrenia and mania in bipolar disease are atypical antipsychotics administered IM and require patient restraint. These include IM aripiprazole, olanzapine, ziprasidone and haloperidol. Oral products include the benzodiazepines, lorazepam and midazolam. Saphris (sublingual tablet asenapine) is an atypical antipsychotic that has been prescribed for use in children and teens for acute treatment of manic or mixed episodes associated with bipolar disease. Adasuve (inhaled loxapine) from Alexza is also a non-invasive treatment. Avanir is currently in Phase 3 with Nuedexta, a combination of dextromethorphan and quinidine for treating chronic agitation in dementia.

Immuno-oncology

The immuno-oncology field is characterized by the rapid evolution of technologies and products and by fierce competition based on the development of compounds, often with similar mechanisms of action. Clinical development plans are further compounded by the possibility of overlapping intellectual property. A wide variety of commercial players, large pharmaceutical companies, established and emerging biotechnology companies, and several not-for-profit entities are actively developing potentially competitive products in immuno-oncology and in our lead indications.

While we believe our product candidates, technology, knowledge, and experience provide us with competitive advantages, we face competition from established and emerging pharmaceutical and biotechnology companies. Such companies include:

- **Major pharmaceutical companies developing multiple immuno-oncology agents:** AstraZeneca PLC, Bristol-Myers Squibb Company, Celgene Corporation, Merck & Co., Inc., Novartis AG, Pfizer Inc., Roche Holding Ltd. and Sanofi SA.

- **Companies developing agents aimed at stimulating the immune response:** AdaptImmune LLC, Idera Pharmaceuticals, Inc., Immune Design Corp., NewLink Genetic Corporation, Advaxis, Inc., Argos Therapeutics, Inc., Biovest International, Inc., ImmunoCellular Therapeutics, Ltd., Immune Design, Inc., Inovio Pharmaceuticals, Inc., Intrexon Corporation and Northwest Biotherapeutics, Inc.

- **Companies developing cell-based immunotherapy approaches:** Intrexon Corporation, Juno Therapeutics, Inc., Kite Pharma, Inc. (acquired by Gilead Sciences, Inc.), Novartis AG and Pfizer Inc.

Nektar Therapeutics Collaborative Research Agreement

On August 27, 2017, we entered into a collaborative research agreement, or Collaboration Agreement, with Nektar, pursuant to which we and Nektar agreed to supply BXCL701 and NKTR-214, respectively, to a CRO, solely for the purpose of performing certain experiments and testing of such materials, or the Evaluation, in the interest of evaluating a potential business relationship between the parties. Pursuant to the terms of the Collaboration Agreement, any inventions or discoveries that are specific to the use of BXCL701 and NKTR-214 in combination or otherwise related to BXCL701 and NKTR-214 that are made after the date of the Collaboration Agreement will be jointly owned by both parties. In addition, we and Nektar have joint ownership of all evaluation data that relates to the

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combined use of the materials. As part of the Collaboration Agreement, the Company agreed to reimburse Nektar for 50% of the actual out-of-pocket expenses billed by the CRO, which total costs are anticipated to be up to $80,720. The term of the Collaboration Agreement is until the earlier of (i) completion of the Evaluation, (ii) the termination of the relationship with the CRO or (iii) the first anniversary of the date of the Collaboration Agreement. Either party may terminate the Collaboration Agreement at any time upon 30 days' prior written notice to the other party or upon 30 days' written notice for any breach by the other party of the provisions of the Collaboration Agreement, provided that the breaching party does not cure such breach in such 30 day period.

Manufacturing

We do not have any manufacturing facilities or personnel. We currently rely, and expect to continue to rely, on third parties for the manufacturing of our product candidates for preclinical as well as for commercial manufacturing if our product candidates receive marketing approval.

For the commercial supply of Dex for our BXCL501 clinical program, potential vendors have been identified, and GMP and United States Pharmacopeia, or USP, grade material is readily available. ARx LLC, USA is responsible for the development and manufacturing of sublingual thin films for BXCL501, which is currently in progress.

We have contracted to restart the production of a clinical batch of BXCL701 under exclusivity with the original manufacturers for API and tablets. We intend to contact other suppliers, including potential strategic partners for the commercial material.

Commercialization

We plan to retain our worldwide commercialization rights for some of our key product candidates while for other product candidates we might consider collaboration opportunities to maximize returns.

While we currently have no sales, marketing or commercial product distribution capabilities and have no experience as a company in commercializing products, we intend to build our own commercialization organization and capabilities over time. When appropriate, we will decide whether to build a specialty sales force to manage commercialization for these product candidates on our own or in combination with a larger pharmaceutical partner, to maximize patient coverage in the United States and to support global expansion especially as our programs have substantial opportunity for additional follow-up indications alone or in combinations.

As product candidates advance through our pipeline, our commercial plans may change. Clinical data, the size of the development programs, the size of the target market, the size of a commercial infrastructure and manufacturing needs may all influence our United States, European Union and rest-of-world strategies.

Our Relationship with BioXcel Corporation

We are currently a 93% owned subsidiary of BioXcel and our pipeline compounds have been identified by applying BioXcel's R&D engine, EvolverAI, for drug re-innovation.

We have entered into an asset contribution agreement, effective June 30, 2017, with BioXcel, as amended and restated on November 7, 2017, or the Contribution Agreement, pursuant to which BioXcel agreed to contribute to us, and we agree to acquire from BioXcel, all of BioXcel's rights, title and interest in and to BXCL501, BXCL701, BXCL502 and BXCL702, collectively, the Candidates, and all of the assets and liabilities associated with the Candidates. In addition, pursuant to the Contribution Agreement, upon completion of this offering, BioXcel will grant us a first right to negotiate exclusive rights to any additional product candidates in the fields of neuroscience and immuno-oncology, that BioXcel may identify on its own, excluding the Candidates, and not in connection with BioXcel's

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provision of services to us under the Services Agreement as defined and described below. This option for first negotiation shall be valid for a period of five years from the date of this offering. Prior to the fifth anniversary of our initial public offering, BioXcel has also agreed to not provide product identification collaborative services to third parties in the fields of neuroscience or immuno-oncology when such third parties utilize EvolverAI. See the section titled "Certain Relationships and Related Person Transactions—Amended and Restated Asset Contribution Agreement with BioXcel" for additional information.

We have entered into a separation and shared services agreement with BioXcel that took effect on June 30, 2017, as amended and restated on November 7, 2017, or the Services Agreement, pursuant to which BioXcel will allow us to continue to use the office space, equipment, services and leased employees based on the agreed upon terms and conditions for a payment of defined monthly and/or hourly fees. The parties have agreed that the services and office space provided under the Services Agreement shall decrease over time until the 12 month anniversary of the date of the Services Agreement, except for services to be provided by BioXcel through its subsidiary in India, which shall decrease until the 24 to 36 month anniversary of the date of the Services Agreement, provided such dates may be extended upon mutual agreement between the parties. On or before December 31, 2019, we shall have the option to enter into a collaborative services agreement with BioXcel pursuant to which BioXcel shall perform product identification and related services for us utilizing EvolverAI. BioXcel shall continue to make such product identification and related services available to us for at least 60 months from June 30, 2017. See the section titled "Certain Relationships and Related Person Transactions—Amended and Restated Separation and Shared Services Agreement with BioXcel" for additional information.

In connection with the Services Agreement, BioXcel agreed to provide us a line of credit, which shall be capped at $1 million, or the Total Funding Amount, pursuant to the terms of a grid note, or the Grid Note. The Grid Note shall be payable upon the earlier of (i) the completion of this offering and (ii) December 31, 2018. As of December 31, 2017, we have drawn an amount of $371,000 under the Grid Note. See the section titled "Certain Relationships and Related Person Transactions—Amended and Restated Separation and Shared Services Agreement with BioXcel" for additional information.

Intellectual Property

Our policy is to protect and enhance the proprietary technologies, inventions, and improvements that are commercially important to our business by filing patent applications in the United States and other jurisdictions related to our proprietary technology, inventions, improvements and product candidates. We also rely on trademarks, trade secrets, and know-how relating to our proprietary technologies and product candidates, continuing innovation and in-licensing technology and products. This reliance is expected to develop, strengthen, and maintain our proprietary position for novel therapeutics and novel formulations of existing therapeutics across multiple therapeutic areas. We also plan to rely on data exclusivity market exclusivity and patent term extensions when available.

Patent Portfolio

We have filed patent applications to protect our proprietary drug programs in immuno-oncology, CNS and agitation. This encompasses our proprietary drug programs in immuno-oncology, CNS and agitation. These proprietary products and methods of use are covered in three separate Patent Cooperation Treaty applications, four pending national phase applications and three pending United States provisional applications to date. However, we intend to file national phase patent applications in all other major counties (Europe, Canada, Japan, Australia and China) in the future.

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The term of individual patents depends upon the legal term for patents in the countries in which they are obtained. In most countries, including the United States, the patent term is 20 years from the earliest filing date of a non-provisional patent application. Depending upon the timing, duration and specifics of FDA approval of our product candidates, a United States patent we own or license may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Act. The Hatch-Waxman Act permits a patent restoration term of up to five years as compensation for patent term lost during product development and the drug approval regulatory review process. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date. The patent term restoration period is generally one-half the time between the effective date of an IND, and the submission date of a NDA, plus the time between the submission date of an NDA and the approval of that application. Only one patent applicable to an approved drug is eligible for the extension and the application for extension must be made prior to expiration of the patent. The United States Patent and Trademark Office, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we intend to apply for restorations of patent term for some of our currently owned or licensed patents to add patent life beyond their current expiration date, depending on the expected length of clinical trials and other factors involved in the submission of the relevant NDA.

The patent positions of companies such as ours are generally uncertain and involve complex legal and factual questions. No consistent policy regarding the scope of claims allowable in patents in the field of method of use patents or reformulation patents has emerged in the United States. The relevant patent laws and their interpretation outside of the United States are also uncertain. Changes in either the patent laws or their interpretation in the United States and other countries may diminish our ability to protect our technology or product candidates and enforce the patent rights that we license, and also could affect the value of such intellectual property. In particular, our ability to stop third parties from making, using, selling, offering to sell, or importing products that infringe our intellectual property will depend in part on our success in obtaining and enforcing patent claims that cover our technology, inventions, and improvements. With respect to both licensed and company-owned intellectual property, we cannot guarantee that patents will be granted with respect to any of our pending patent applications or with respect to any patent applications we may file in the future, nor can we be sure that any patents that may be granted to us in the future will be commercially useful in protecting our products, the methods of use, or the manufacture of those products. Patent and other intellectual property rights in the pharmaceutical and biotechnology space are evolving and involve many risks and uncertainties. For example, third parties may have blocking patents that could be used to prevent us from commercializing our product candidates and practicing our proprietary technology, and the issued patents that we in-license and those that may issue in the future may be challenged, invalidated, or circumvented, which could limit our ability to stop competitors from marketing related products or could limit the term of patent protection that otherwise may exist for our product candidates. In addition, the scope of the rights granted under any issued patents may not provide us with protection or competitive advantages against competitors with similar technology. Furthermore, our competitors may independently develop similar technologies that are outside the scope of the rights granted under any issued patents that we own or exclusively in-license. For these reasons, we may face competition with respect to our product candidates. Moreover, because of the extensive time required for development, testing, and regulatory review of a potential product, it is possible that, before any particular product candidate can be commercialized, any patent protection for such product may expire or remain in force for only a short period following commercialization, thereby reducing the commercial advantage the patent provides.

Midatech Data Purchase Agreement Related to BXCL701

On January 4, 2016, BioXcel executed a Data Purchase Agreement with Midatech Pharma US Inc., the successor of Dara Biosciences, itself successor of the original developer of Talabostat mesylate, or Talabostat, pursuant to which Midatech transferred to BioXcel all rights, title, and interests

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to all preclinical, clinicial, Chemistry, Manufacturing and Controls and any other relevant data related to Talabostat. Subsequently, Midatech also transferred the ownership of Talabostat IND 62379 to BioXcel and communicated such transfer to the FDA. This agreement was assigned to us pursuant to the Contribution Agreement.

Government Regulation

The FDA and comparable regulatory authorities in state and local jurisdictions and in other countries impose substantial and burdensome requirements upon companies involved in the clinical development, manufacture, marketing and distribution of drugs and medical devices, such as those we are developing. These agencies and other federal, state and local entities regulate, among other things, the research and development, testing, manufacture, quality control, safety, effectiveness, labeling, storage, record keeping, approval, advertising and promotion, distribution, post-approval monitoring and reporting, sampling and export and import of our product candidates.

U.S. Government Regulation of Drug Products

In the United States, the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act, or FDCA, and its implementing regulations. The process of obtaining regulatory approvals and the subsequent compliance with applicable federal, state, local and foreign statutes and regulations requires the expenditure of substantial time and financial resources. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approval, may subject an applicant to a variety of administrative or judicial sanctions, such as the FDA's refusal to approve pending NDAs, withdrawal of an approval, imposition of a clinical hold, issuance of warning letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, restitution, disgorgement or civil or criminal penalties.

The process required by the FDA before product candidates may be marketed in the United States generally involves the following:

- submission to the FDA of an IND application which must become effective before human clinical trials may begin and must be updated annually;
- completion of extensive preclinical laboratory tests and preclinical animal studies, all performed in accordance with the FDA's GLP regulations. Preclinical testing generally includes evaluation of our products in the laboratory or in animals to characterize the product and determine safety and efficacy;
- approval by an independent institutional review board, or IRB, at each clinical site before each trial may be initiated;
- performance of adequate and well-controlled human clinical trials in accordance with good clinical practice, or GCP, requirements to establish the safety and efficacy of the product candidate for each proposed indication;
- submission to the FDA of an NDA after completion of all pivotal clinical trials;
- a determination by the FDA within 60 days of its receipt of an NDA to accept the filing for review;
- satisfactory completion of an FDA advisory committee review, if applicable;

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- satisfactory completion of an FDA pre-approval inspection of the manufacturing facilities at which the active pharmaceutical ingredient, or API, and finished drug product are produced and tested to assess compliance with cGMP regulations and to assure that the facilities, methods and controls are adequate to preserve the drug's identity, strength, quality and purity;

- satisfactory completion of FDA audits of clinical trial sites to assure compliance with GCPs and the integrity of the clinical data;

- payment of user fees and securing FDA approval of the NDA; and

- compliance with any post-approval requirements, including the potential requirement to implement a REMS and the potential requirement to conduct post-approval studies.

Preclinical studies include laboratory evaluation of product chemistry, toxicity and formulation, as well as animal studies to assess potential safety and efficacy. An IND is a request for authorization from the FDA to administer an investigational drug product to humans. The central focus of an IND submission is on the general investigational plan and the protocol(s) for human studies. The IND also includes results of preclinical studies or other human studies, as appropriate, as well as manufacturing information, analytical data and any available clinical data or literature to support the use of the investigational new drug. An IND must become effective before human clinical trials may begin. An IND will automatically become effective 30 days after receipt by the FDA, unless before that time the FDA raises concerns or questions related to the proposed clinical trials. In such a case, the IND may be placed on clinical hold and the IND sponsor and the FDA must resolve any outstanding concerns or questions before clinical trials can begin. Accordingly, submission of an IND may or may not result in the FDA allowing clinical trials to commence.

Clinical trials involve the administration of the new investigational drug to human subjects under the supervision of qualified investigators in accordance with GCPs, which include the requirement that all research subjects provide their informed consent for their participation in any clinical trial. Clinical trials are conducted under protocols detailing, among other things, the objectives of the study, the parameters to be used in monitoring safety, and the efficacy criteria to be evaluated. A protocol for each clinical trial and any subsequent protocol amendments must be submitted to the FDA as part of the IND.

Additionally, approval must also be obtained from each clinical trial site's IRB before the trials may be initiated, and the IRB must monitor the study until completed. There are also requirements governing the reporting of ongoing clinical trials and clinical trial results to public registries.

The clinical investigation of a drug is generally divided into three phases. Although the phases are usually conducted sequentially, they may overlap or be combined. The three phases of an investigation are as follows:

- **Phase 1.** Phase 1 includes the initial introduction of an investigational new drug into humans. Phase 1 clinical trials are typically closely monitored and may be conducted in patients with the target disease or condition or in healthy volunteers. These studies are designed to evaluate the safety, dosage tolerance, metabolism and pharmacologic actions of the investigational drug in humans, the side effects associated with increasing doses, and if possible, to gain early evidence on effectiveness. During Phase 1 clinical trials, sufficient information about the investigational drug's pharmacokinetics and pharmacological effects may be obtained to permit the design of well-controlled and scientifically valid Phase 2 clinical trials.

- **Phase 2.** Phase 2 includes controlled clinical trials conducted to preliminarily or further evaluate the effectiveness of the investigational drug for a particular indication(s) in patients with the disease or condition under study, to determine dosage tolerance and optimal dosage, and to identify possible adverse side effects and safety risks associated with the drug. Phase 2 clinical

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trials are typically well-controlled, closely monitored, and conducted in a limited patient population.

- **Phase 3.** Phase 3 clinical trials are generally controlled clinical trials conducted in an expanded patient population generally at geographically dispersed clinical trial sites. They are performed after preliminary evidence suggesting effectiveness of the drug has been obtained, and are intended to further evaluate dosage, clinical effectiveness and safety, to establish the overall benefit-risk relationship of the investigational drug product, and to provide an adequate basis for product approval.

A registration study is any clinical study, which adequately meets regulatory agency requirements for the evaluation of a product candidate's efficacy and safety such that it can be used to justify the approval of the product. Generally, pivotal studies are Phase 3 studies but may also be Phase 2 studies if the trial design provides a well-controlled and reliable assessment of clinical benefit, particularly in situations where there is an unmet medical need.

Progress reports detailing the results of the clinical trials must be submitted at least annually to the FDA and more frequently if serious adverse events occur. Phase 1, Phase 2 and Phase 3 clinical trials may not be completed successfully within any specified period, or at all. The FDA, the IRB, or the clinical trial sponsor may suspend or terminate a clinical trial at any time on various grounds, including a finding that the research subjects are being exposed to an unacceptable health risk. Additionally, some clinical trials are overseen by an independent group of qualified experts organized by the clinical trial sponsor, known as a data safety monitoring board or committee. This group provides authorization for whether or not a trial may move forward at designated check points based on access to certain data from the study. We may also suspend or terminate a clinical trial based on evolving business objectives and/or competitive climate.

Assuming successful completion of all required testing in accordance with all applicable regulatory requirements, detailed investigational drug product information is submitted to the FDA in the form of an NDA requesting approval to market the product for one or more indications.

The application includes all relevant data available from pertinent preclinical and clinical trials, including negative or ambiguous results as well as positive findings, together with detailed information relating to the product's chemistry, manufacturing, controls and proposed labeling, among other things. Data can come from company-sponsored clinical trials intended to test the safety and effectiveness of a use of a product, or from a number of alternative sources, including studies initiated by investigators. To support marketing approval, the data submitted must be sufficient in quality and quantity to establish the safety and effectiveness of the investigational drug product to the satisfaction of the FDA.

In most cases, the submission of an NDA is subject to a substantial application user fee. Under the Prescription Drug User Fee Act, or PDUFA, guidelines that are currently in effect, the FDA has a goal of ten months from the date of "filing" of an NDA to review and act on the submission. This review typically takes twelve months from the date the NDA is submitted to FDA because the FDA has approximately two months to make a "filing" decision.

In addition, under the Pediatric Research Equity Act of 2003, or PREA, as amended and reauthorized, certain NDAs or supplements to an NDA must contain data that are adequate to assess the safety and effectiveness of the drug for the claimed indications in all relevant pediatric subpopulations, and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults, or full or partial waivers from the pediatric data requirements.

The FDA also may require submission of a REMS plan to ensure that the benefits of the drug outweigh its risks. The REMS plan could include medication guides, physician communication plans,

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assessment plans, and/or elements to assure safe use, such as restricted distribution methods, patient registries, or other risk minimization tools.

The FDA conducts a preliminary review of all NDAs within the first 60 days after submission, before accepting them for filing, to determine whether they are sufficiently complete to permit substantive review. The FDA may request additional information rather than accept an NDA for filing. In this event, the application must be resubmitted with the additional information. The resubmitted application is also subject to review before the FDA accepts it for filing. Once the NDA submission has been accepted for filing, the FDA's goal is to review applications within ten months of submission or, if the application relates to an unmet medical need in a serious or life-threatening indication, six months from submission. The review process is often significantly extended by FDA requests for additional information or clarification. The FDA may refer the application to an advisory committee for review, evaluation and recommendation as to whether the application should be approved. The FDA is not bound by the recommendation of an advisory committee but it typically follows such recommendations.

The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA, the FDA may inspect one or more clinical trial sites to assure compliance with GCP requirements. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA, the FDA may inspect one or more clinical trial sites to assure compliance with GCP requirements.

After the FDA evaluates the NDA and conducts inspections of manufacturing facilities where the drug product and/or its active pharmaceutical ingredient, or API, will be produced, it may issue an approval letter or a Complete Response Letter. An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications. A Complete Response Letter indicates that the review cycle of the application is complete and the application is not ready for approval. A Complete Response Letter may require additional clinical data and/or an additional pivotal Phase 3 clinical trial(s), and/or other significant, expensive and time-consuming requirements related to clinical trials, preclinical studies or manufacturing. Even if such additional information is submitted, the FDA may ultimately decide that the NDA does not satisfy the criteria for approval. The FDA could also approve the NDA with a REMS plan to mitigate risks, which could include medication guides, physician communication plans, or elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. The FDA also may condition approval on, among other things, changes to proposed labeling, development of adequate controls and specifications, or a commitment to conduct one or more post-market studies or clinical trials. Such post-market testing may include Phase 4 clinical trials and surveillance to further assess and monitor the product's safety and effectiveness after commercialization. Regulatory approval of oncology products often requires that patients in clinical trials be followed for long periods to determine the overall survival benefit of the drug. The FDA may prevent or limit further marketing of a product based on the results of post-marketing studies or surveillance programs. After approval, some types of changes to the approved product, such as adding new indications, manufacturing changes, and additional labeling claims, are subject to further testing requirements and FDA review and approval.

After regulatory approval of a drug product is obtained, we are required to comply with a number of post-approval requirements. As a holder of an approved NDA, we would be required to report, among other things, certain adverse reactions and production problems to the FDA, to provide updated safety and efficacy information, and to comply with requirements concerning advertising and promotional labeling for any of our products. Also, quality control and manufacturing procedures must continue to conform to cGMP after approval to ensure and preserve the long-term stability of the drug product. In addition, drug manufacturers and other entities involved in the manufacture and

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distribution of approved drugs are required to register their establishments with the FDA and state agencies, and are subject to periodic unannounced inspections by the FDA and these state agencies for compliance with cGMP requirements. In addition, changes to the manufacturing process are strictly regulated, and, depending on the significance of the change, may require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP and impose reporting and documentation requirements upon us and any third-party manufacturers that we may decide to use. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain compliance with cGMP and other aspects of regulatory compliance.

We rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of our product candidates. Future FDA and state inspections may identify compliance issues at our facilities or at the facilities of our contract manufacturers that may disrupt production or distribution, or require substantial resources to correct. In addition, discovery of previously unknown problems with a product or the failure to comply with applicable requirements may result in, among other things,

- restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;
- fines, warning letters or holds on post-approval clinical trials;
- refusal of the FDA to approve pending NDAs or supplements to approved NDAs, or suspension or revocation of product approvals;
- product seizure or detention, or refusal to permit the import or export of products; or
- injunctions or the imposition of civil or criminal penalties.

Newly discovered or developed safety or effectiveness data may require changes to a product's approved labeling, including the addition of new warnings and contraindications, and also may require the implementation of other risk management measures. The FDA strictly regulates marketing, labeling, advertising and promotion of products that are placed on the market. Drugs or devices may be promoted only for the approved indications and in accordance with the provisions of the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability. Also, from time to time, legislation is drafted, introduced and passed in Congress that could significantly change the statutory provisions governing the approval, manufacturing and marketing of products regulated by the FDA. In addition, FDA regulations and guidance are often revised or reinterpreted by the agency in ways that may significantly affect our business and our products. It is impossible to predict whether legislative changes will be enacted, or FDA regulations, guidance or interpretations changed or what the impact of such changes, if any, may be.

Marketing Exclusivity

Market exclusivity provisions under the FDCA also can delay the submission or the approval of certain applications. The FDCA provides a five-year period of non-patent marketing exclusivity within the United States to the first applicant to obtain approval of an NDA for a new chemical entity. A drug is a new chemical entity if the FDA has not previously approved any other new drug containing the same active moiety, which is the molecule or ion responsible for the action of the drug substance. During the exclusivity period, the FDA may not accept for review an abbreviated new drug application, or ANDA, or a 505(b)(2) NDA submitted by another company for another version of such drug where the applicant does not own or have a legal right of reference to all the data required for approval. However, an application may be submitted after four years if it contains a certification of patent invalidity or non-infringement. The FDCA also provides three years of marketing exclusivity for an

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NDA, 505(b)(2) NDA or supplement to an approved NDA if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant are deemed by the FDA to be essential to the approval of the application, for example, for new indications, dosages or strengths of an existing drug. This three-year exclusivity covers only the conditions associated with the new clinical investigations and does not prohibit the FDA from approving ANDAs for drugs containing the original active pharmaceutical ingredient. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA; however, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to all of the preclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness.

Available Special Regulatory Procedures

The FDA has various programs, including fast track designation, accelerated approval, priority review, and breakthrough therapy designation, which are intended to expedite or simplify the process for the development and FDA review of drugs that are intended for the treatment of serious or life threatening diseases or conditions and demonstrate the potential to address unmet medical needs. The purpose of these programs is to provide important new drugs to patients earlier than under standard FDA review procedures.

Fast Track Designation

To be eligible for a fast track designation, the FDA must determine, based on the request of a sponsor, that a product is intended to treat a serious or life-threatening disease or condition and demonstrates the potential to address an unmet medical need. The FDA will determine that a product will fill an unmet medical need if it will provide a therapy where none exists or provide a therapy that may be potentially superior to existing therapy based on efficacy or safety factors. The FDA may review sections of the NDA for a fast track product on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the NDA, the FDA agrees to accept sections of the NDA and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the NDA.

Priority Review

The FDA may give a priority review designation to drugs that offer major advances in treatment, or provide a treatment where no adequate therapy exists. A priority review means that the goal for the FDA to review an application is six months, rather than the standard review of ten months under current PDUFA guidelines. Under the new PDUFA agreement, these six and ten month review periods are measured from the "filing" date rather than the receipt date for NDAs for new molecular entities, which typically adds approximately two months to the timeline for review and decision from the date of submission. Most products that are eligible for fast track designation are also likely to be considered appropriate to receive a priority review.

Breakthrough Therapy Designation

Under the provisions of the Food and Drug Administration Safety and Innovation Act, or FDASIA, passed in July 2012, a sponsor can request designation of a product candidate as a "breakthrough therapy." A breakthrough therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. Drugs designated as breakthrough therapies are also eligible for accelerated approval. The FDA must take certain actions, such as holding timely meetings and

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providing advice, intended to expedite the development and review of an application for approval of a breakthrough therapy.

Accelerated Approval Pathway

The FDA may grant accelerated approval to a drug for a serious or life-threatening condition that provides meaningful therapeutic advantage to patients over existing treatments based upon a determination that the drug has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit. The FDA may also grant accelerated approval for such a condition when the product has an effect on an intermediate clinical endpoint that can be measured earlier than an effect on irreversible morbidity or mortality, or IMM, and that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity or prevalence of the condition and the availability or lack of alternative treatments. Drugs granted accelerated approval must meet the same statutory standards for safety and effectiveness as those granted traditional approval.

For the purposes of accelerated approval, a surrogate endpoint is a marker, such as a laboratory measurement, radiographic image, physical sign or other measure that is thought to predict clinical benefit, but is not itself a measure of clinical benefit. Surrogate endpoints can often be measured more easily or more rapidly than clinical endpoints. An intermediate clinical endpoint is a measurement of a therapeutic effect that is considered reasonably likely to predict the clinical benefit of a drug, such as an effect on IMM. The FDA has limited experience with accelerated approvals based on intermediate clinical endpoints, but has indicated that such endpoints generally may support accelerated approval where the therapeutic effect measured by the endpoint is not itself a clinical benefit and basis for traditional approval, if there is a basis for concluding that the therapeutic effect is reasonably likely to predict the ultimate clinical benefit of a drug.

The accelerated approval pathway is most often used in settings in which the course of a disease is long and an extended period of time is required to measure the intended clinical benefit of a drug, even if the effect on the surrogate or intermediate clinical endpoint occurs rapidly. Thus, accelerated approval has been used extensively in the development and approval of drugs for treatment of a variety of cancers in which the goal of therapy is generally to improve survival or decrease morbidity and the duration of the typical disease course requires lengthy and sometimes large trials to demonstrate a clinical or survival benefit.

The accelerated approval pathway is usually contingent on a sponsor's agreement to conduct, in a diligent manner, additional post-approval confirmatory studies to verify and describe the drug's clinical benefit. As a result, a product candidate approved on this basis is subject to rigorous post-marketing compliance requirements, including the completion of Phase 4 or post-approval clinical trials to confirm the effect on the clinical endpoint. Failure to conduct required post-approval studies, or confirm a clinical benefit during post-marketing studies, would allow the FDA to withdraw the drug from the market on an expedited basis. All promotional materials for product candidates approved under accelerated regulations are subject to prior review by the FDA.

Even if a product qualifies for one or more of these programs, the FDA may later decide that the product no longer meets the conditions for qualification or decide that the time period for FDA review or approval will not be shortened. We may explore some of these opportunities for our product candidates as appropriate.

The Hatch-Waxman Amendments: 505(b)(2) Approval Process

Section 505(b)(2) of the FDCA provides an alternate regulatory pathway to FDA approval for new or improved formulations or new uses of previously approved drug products. Specifically, Section 505(b)(2) permits the filing of an NDA where at least some of the information required for

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approval comes from studies not conducted by or for the applicant and for which the applicant has not obtained a right of reference. The applicant may rely upon the FDA's findings of safety and effectiveness for an approved product that acts as the Reference Listed Drug, or RLD. If the 505(b)(2) applicant can establish that reliance on FDA's previous findings of safety and effectiveness is scientifically appropriate, it may eliminate the need to conduct certain preclinical or clinical studies of the new product. The FDA may also require 505(b)(2) applicants to perform additional studies or measurements to support the change from the RLD. The FDA may then approve the new product candidate for all or some of the labeled indications for which the referenced product has been approved, as well as for any new indication sought by the Section 505(b)(2) applicant.

In seeking approval for a drug through an NDA, including a 505(b)(2) NDA, applicants are required to list with the FDA certain patents whose claims cover the applicant's product. Upon approval of an NDA, each of the patents listed in the application for the drug is then published in the Orange Book. Any applicant who files an ANDA seeking approval of a generic equivalent version of a drug listed in the Orange Book or a 505(b)(2) NDA referencing a drug listed in the Orange Book must certify to the FDA that (i) no patent information on the drug product that is the subject of the application has been submitted to the FDA; (ii) such patent has expired; (iii) the date on which such patent expires; or (iv) such patent is invalid or will not be infringed upon by the manufacture, use or sale of the drug product for which the application is submitted. This last certification is known as a paragraph IV certification. A notice of the paragraph IV certification must be provided to each owner of the patent that is the subject of the certification and to the holder of the approved NDA to which the ANDA or 505(b)(2) application refers. The applicant may also elect to submit a "section viii" statement certifying that its proposed label does not contain (or carves out) any language regarding the patented method-of-use rather than certify to a listed method-of-use patent. If the reference NDA holder and patent owners assert a patent challenge directed to one of the Orange Book listed patents within 45 days of the receipt of the paragraph IV certification notice, the FDA is prohibited from approving the application until the earlier of 30 months from the receipt of the paragraph IV certification expiration of the patent, settlement of the lawsuit or a decision in the infringement case that is favorable to the applicant. The ANDA or 505(b)(2) application also will not be approved until any applicable non-patent exclusivity listed in the Orange Book for the branded reference drug has expired.

Our current and anticipated product candidates will be based on already approved active pharmaceutical ingredients, or APIs, rather than new chemical entities, and a formulation that has been through Phase 1 studies. Accordingly, we expect to be able to rely on information from previously conducted formulation studies involving our clinical development plans and our NDA submissions. For product candidates that involve novel fixed-dose combinations of existing drugs or for studies of an existing product or product candidate in a new indication, we believe we generally will be able to initiate Phase 2/3 studies without conducting any new non-clinical or Phase 1 studies, though the FDA may not agree with our conclusions and may require us to conduct additional clinical or preclinical studies prior to initiating Phase 3 or other pivotal clinical trials. In those instances where our product candidate is a pharmacokinetically enhanced version of an approved API, we will need to conduct certain non-clinical and Phase 1 studies to confirm the pharmacokinetic profile of the product candidate prior to conducting Phase 2/3 studies.

Orphan Drug Designation and Exclusivity

The Orphan Drug Act provides incentives for the development of products intended to treat rare diseases or conditions. Under the Orphan Drug Act, the FDA may grant orphan drug designation to a drug or biological product intended to treat a rare disease or condition, which is generally a disease or condition that affects fewer than 200,000 individuals in the United States, or more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of

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developing and making a drug or biological product available in the United States for this type of disease or condition will be recovered from sales of the product. If a sponsor demonstrates that a drug is intended to treat rare diseases or conditions, the FDA will grant orphan drug designation for that product for the orphan disease indication. Orphan drug designation must be requested before submitting an NDA. After the FDA grants orphan drug designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. Orphan drug designation, however, does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

Orphan drug designation provides manufacturers with research grants, tax credits and eligibility for orphan drug exclusivity. If a product that has orphan drug designation subsequently receives the first FDA approval of the active moiety for that disease or condition for which it has such designation, the product is entitled to orphan drug exclusivity, which for seven years prohibits the FDA from approving another product with the same active ingredient for the same indication, except in limited circumstances. If a drug designated as an orphan product receives marketing approval for an indication broader than the orphan drug indication for which it received the designation, it will not be entitled to orphan drug exclusivity. Orphan drug exclusivity will not bar approval of another product under certain circumstances, including if a subsequent product with the same active ingredient for the same indication is shown to be clinically superior to the approved product on the basis of greater efficacy or safety, or providing a major contribution to patient care, or if the company with orphan drug exclusivity is not able to meet market demand. Further, the FDA may approve more than one product for the same orphan drug indication or disease as long as the products contain different active ingredients. Moreover, competitors may receive approval of different products for the indication for which the orphan drug product has exclusivity or obtain approval for the same product but for a different indication for which the orphan drug product has exclusivity. As a result, even if one of our product candidates receives orphan exclusivity, we may still be subject to competition. Orphan drug exclusivity also could block the approval of one of our products for seven years if a competitor obtains approval of the same drug or if our product candidate is determined to be contained within the competitor's product for the same indication or disease.

International Regulations

In addition to regulations in the United States, we are and will be subject to a variety of foreign regulations regarding development, approval, commercial sales and distribution of our products. Whether or not we obtain FDA approval for a product, we must obtain the necessary approvals by the comparable regulatory authorities of foreign countries before we can commence clinical trials or marketing of the product in those countries. The approval process varies from country to country and can involve additional product testing and additional review periods, and the time may be longer or shorter than that required to obtain FDA approval. The requirements governing, among other things, the conduct of clinical trials, product licensing, pricing and reimbursement vary greatly from country to country. Regulatory approval in one country does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country may negatively impact the regulatory process in others. If we fail to comply with applicable foreign regulatory requirements, we may be subject to fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

To obtain regulatory approval of an investigational drug under European Union regulatory systems, we must submit a marketing authorization application. The application used to file the NDA in the United States is similar to that required in Europe, with the exception of, among other things, country-specific document requirements. For other countries outside of the European Union, such as countries in Eastern Europe, Latin America or Asia, the requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. In all cases, again, the

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clinical trials are conducted in accordance with GCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

If we fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

Centralized Procedure

The European Medicines Agency, or EMA, implemented the centralized procedure for the approval of human medicines to facilitate marketing authorizations that are valid throughout the EU. This procedure results in a single marketing authorization issued by the European Commission following a favorable opinion by the EMA that is valid across the European Union, as well as Iceland, Liechtenstein, and Norway. The centralized procedure is compulsory for human medicines that are: derived from biotechnology processes, such as genetic engineering, contain a new active substance indicated for the treatment of certain diseases, such as HIV/AIDS, cancer, diabetes, neurodegenerative disorders or autoimmune diseases and other immune dysfunctions, and officially designated orphan medicines. For medicines that do not fall within these categories, an applicant has the option of submitting an application for a centralized marketing authorization to the EMA, as long as the medicine concerned is a significant therapeutic, scientific or technical innovation, or if its authorization would be in the interest of public health.

There are also two other possible routes to authorize medicinal products in several European Union countries, which are available for investigational medicinal products that fall outside the scope of the centralized procedure: the decentralized procedure and the mutual recognition procedure. Under the decentralized procedure, an applicant may apply for simultaneous authorization in more than one EU country for medicinal products that have not yet been authorized in any EU country and that do not fall within the mandatory scope of the centralized procedure. Under the mutual recognition procedure, a medicine is first authorized in one EU Member State, in accordance with the national procedures of that country. Following a national authorization, the applicant may seek further marketing authorizations from other EU countries under a procedure whereby the countries concerned agree to recognize the validity of the original, national marketing authorization.

In the EU, medicinal products designated as orphan drug products benefit from financial incentives such as reductions in marketing authorization application fees or fee waivers and 10 years of marketing exclusivity following medicinal product approval. For a medicinal product to qualify as orphan drugs: (i) it must be intended for the treatment, prevention or diagnosis of a disease that is life-threatening or chronically debilitating; (ii) the prevalence of the condition in the EU must not be more than five in 10,000 or it must be unlikely that marketing of the medicine would generate sufficient returns to justify the investment needed for its development; and (iii) no satisfactory method of diagnosis, prevention or treatment of the condition concerned can be authorized, or, if such a method exists, the medicine must be of significant benefit to those affected by the condition.

Accelerated Review (European Union)

Based on results of the Phase 3 clinical trial(s) submitted in an NDA, upon the request of an applicant, the FDA may grant the NDA a priority review designation, which sets the target date for FDA action on the application at six months. Priority review is granted where preliminary estimates indicate that a product, if approved, has the potential to provide a safe and effective therapy where no satisfactory alternative therapy exists, or a significant improvement compared to marketed products is possible. If criteria are not met for priority review, the NDA is subject to the standard FDA review period of 10 months. Priority review designation does not change the scientific/medical standard for approval or the quality of evidence necessary to support approval.

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Under the Centralized Procedure in the European Union, the maximum timeframe for the evaluation of a marketing authorization application is 210 days (excluding clock stops, when additional written or oral information is to be provided by the applicant in response to questions asked by the CHMP, accelerated evaluation might be granted by the CHMP in exceptional cases, when a medicinal product is expected to be of a major public health interest, defined by three cumulative criteria: the seriousness of the disease (e.g. heavy disabling or life-threatening diseases) to be treated; the absence or insufficiency of an appropriate alternative therapeutic approach; and anticipation of high therapeutic benefit. In this circumstance, EMA ensures that the opinion of the CHMP is given within 150 days, excluding clock stops.

There can be no assurance that we or any of our partners would be able to satisfy one or more of these requirements to conduct preclinical or clinical trials or receive any regulatory approvals.

Pharmaceutical Coverage, Pricing and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any drug products for which we obtain regulatory approval. In the United States and markets in other countries, sales of any products for which we receive regulatory approval for commercial sale will depend in part on the availability of reimbursement from third-party payors. Third-party payors include government health administrative authorities, managed care providers, private health insurers and other organizations. The process for determining whether a payor will provide coverage for a drug product may be separate from the process for setting the price or reimbursement rate that the payor will pay for the drug product. Third-party payors may limit coverage to specific drug products on an approved list, or formulary, which might not include all of the FDA-approved drugs for a particular indication. Third-party payors are increasingly challenging the price and examining the medical necessity and cost-effectiveness of medical products and services, in addition to their safety and efficacy. We may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of our products, in addition to the costs required to obtain FDA approvals. Our product candidates may not be considered medically necessary or cost-effective. A payor's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Adequate third-party reimbursement may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in product development.

In 2003, the U.S. government enacted legislation providing a partial prescription drug benefit for Medicare beneficiaries, which became effective at the beginning of 2006. Government payment for some of the costs of prescription drugs may increase demand for any products for which we receive marketing approval. However, to obtain payments under this program, we would be required to sell products to Medicare recipients through prescription drug plans operating pursuant to this legislation. These plans will likely negotiate discounted prices for our products. Further, the Healthcare Reform Law substantially changes the way healthcare is financed in the United States by both government and private insurers. Among other cost containment measures, the Healthcare Reform Law establishes:

- an annual, nondeductible fee on any entity that manufactures or imports certain branded prescription drugs and biologic agents;
- a new Medicare Part D coverage gap discount program, in which pharmaceutical manufacturers who wish to have their drugs covered under Part D must offer discounts to eligible beneficiaries during their coverage gap period, or the "donut hole"; and
- a new formula that increases the rebates a manufacturer must pay under the Medicaid Drug Rebate Program.

We expect that federal, state and local governments in the United States will continue to consider legislation to limit the growth of healthcare costs, including the cost of prescription drugs. Future

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legislation could limit payments for pharmaceuticals such as the product candidates that we are developing.

Different pricing and reimbursement schemes exist in other countries. In the European Community, governments influence the price of pharmaceutical products through their pricing and reimbursement rules and control of national health care systems that fund a large part of the cost of those products to consumers. Some jurisdictions operate positive and negative list systems under which products may only be marketed once a reimbursement price has been agreed. To obtain reimbursement or pricing approval, some of these countries may require the completion of clinical trials that compare the cost-effectiveness of a particular product candidate to currently available therapies. Other member states allow companies to fix their own prices for medicines, but monitor and control company profits. The downward pressure on health care costs in general, particularly prescription drugs, has become very intense. As a result, new products are facing increasingly high barriers to entry. In addition, in some countries, cross-border imports from low-priced markets exert a commercial pressure on pricing within a country.

The marketability of any products for which we receive regulatory approval for commercial sale may suffer if the government and third-party payors fail to provide adequate coverage and reimbursement. In addition, an increasing emphasis on managed care in the United States has increased and we expect will continue to increase the pressure on pharmaceutical pricing. Coverage policies and third-party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is secured for one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

Other Healthcare Laws and Compliance Requirements

If we obtain regulatory approval for any of our product candidates, we may be subject to various federal and state laws targeting fraud and abuse in the healthcare industry. For example, in the United States, there are federal and state anti-kickback laws that prohibit the payment or receipt of kickbacks, bribes or other remuneration intended to induce the purchase or recommendation of healthcare products and services or reward past purchases or recommendations. Violations of these laws can lead to civil and criminal penalties, including fines, imprisonment and exclusion from participation in federal healthcare programs.

The federal Anti-Kickback Statute prohibits persons from knowingly and willfully soliciting, receiving, offering or paying remuneration, directly or indirectly, to induce either the referral of an individual, or the furnishing, recommending, or arranging for a good or service, for which payment may be made under a federal healthcare program, such as the Medicare and Medicaid programs. The reach of the Anti-Kickback Statute was broadened by the Healthcare Reform Law, which, among other things, amends the intent requirement of the federal Anti-Kickback Statute and the applicable criminal healthcare fraud statutes contained within 42 USC. §1320a-7b, effective March 23, 2010. Pursuant to the statutory amendment, a person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation. In addition, the Healthcare Reform Law provides that the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the civil False Claims Act (discussed below) or the civil monetary penalties statute. Many states have adopted laws similar to the federal Anti-Kickback Statute, some of which apply to the referral of patients for healthcare items or services reimbursed by any source, not only the Medicare and Medicaid programs.

The federal False Claims Act imposes liability on any person who, among other things, knowingly presents, or causes to be presented, a false or fraudulent claim for payment by a federal healthcare program. The "qui tam" provisions of the False Claims Act allow a private individual to bring civil

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actions on behalf of the federal government alleging that the defendant has submitted a false claim to the federal government, and to share in any monetary recovery. In addition, various states have enacted false claims laws analogous to the False Claims Act. Many of these state laws apply where a claim is submitted to any third-party payer and not merely a federal healthcare program. When an entity is determined to have violated the False Claims Act, it may be required to pay up to three times the actual damages sustained by the government, plus civil penalties of $5,500 to $11,000 for each separate false claim.

Also, the Health Insurance Portability and Accountability Act of 1996, or HIPAA, created several new federal crimes, including healthcare fraud, and false statements relating to healthcare matters. The health care fraud statute prohibits knowingly and willfully executing a scheme to defraud any health care benefit program, including private third-party payers. The false statements statute prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for health care benefits, items or services.

In addition, we may be subject to, or our marketing activities may be limited by, HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, and its implementing regulations, which established uniform standards for certain "covered entities" (healthcare providers, health plans and healthcare clearinghouses) and their business associates governing the conduct of certain electronic healthcare transactions and protecting the security and privacy of protected health information.

In order to raise sufficient financial resources to continue to advance our product candidates, we will need to address pricing pressures and potential third-party reimbursement coverage for our product candidates. In the United States and elsewhere, sales of pharmaceutical products depend in significant part on the availability of reimbursement to the consumer from third-party payors, such as government and private insurance plans. Third-party payors are increasingly challenging the prices charged for medical products and services. It is and will continue to be time-consuming and expensive for us or our strategic collaborators to go through the process of seeking reimbursement from Medicare and private payors. Our products may not be considered cost effective, and coverage and reimbursement may not be available or sufficient to allow us to sell our products on a competitive and profitable basis.

The Physician Payment Sunshine Act

The Physician Payment Sunshine Act, or the Sunshine Act, which was enacted as part of the Patient Protection and Affordable Care Act, or the ACA, requires applicable manufacturers of drugs, devices, biologicals, or medical supplies covered under Medicare, Medicaid or the Children's Health Insurance Program, to report annually to the Secretary of the Department of Health and Human Services payments or other transfers of value made by that entity, or by a third party as directed by that entity, to physicians and teaching hospitals, or to third parties on behalf of physicians or teaching hospitals, during the course of the preceding calendar year. The Final Rule implementing the Sunshine Act, published on February 8, 2013, requires data collection on payments to begin on August 1, 2013. The first annual report, comprised of data collected from August 1, 2013 to December 31, 2013, is due March 31, 2014. Failure to comply with the reporting requirements can result in significant civil monetary penalties ranging from $1,000 to $10,000 for each payment or other transfer of value that is not reported (up to a maximum per annual report of $150,000) and from $10,000 to $100,000 for each knowing failure to report (up to a maximum per annual report of $1 million). We will be required to collect data on and report these payments.

In many foreign markets, including the countries in the European Union, pricing of pharmaceutical products is subject to governmental control. In the United States, there have been, and we expect that

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there will continue to be, a number of federal and state proposals to implement similar governmental pricing control.

Employees

As of December 31, 2017, we employed a total of four full-time employees and our parent, BioXcel, has two employees who provide services to us pursuant to our separation and shared services agreement between us and BioXcel. In addition, we will have access to certain of BioXcel employees and resources through the various agreements we have entered into with BioXcel. We are not a party to any collective bargaining agreements. We believe that we maintain good relations with our employees.

Facilities

Our corporate headquarters and executive offices are provided to us by BioXcel under the shared services agreement discussed above and are located in Branford, Connecticut. We believe that our existing facilities are suitable and adequate to meet our current needs. We intend to add new facilities or expand existing facilities as we add employees, and we believe that suitable additional or substitute space will be available as needed to accommodate any such expansion of our operations.

Legal Proceedings

We may be involved from time to time in ordinary litigation, negotiation, and settlement matters that will not have a material effect on our operations or finances. We are not currently party to any material legal proceedings, and we are not aware of any pending or threatened litigation against us that we believe could have a material adverse effect on our business, operating results or financial condition.

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MANAGEMENT

Executive Officers and Directors

The following table sets forth the name, age and position of each of our executive officers, key employees and directors as of December 31, 2017.

Name	Age	Position
Executive Officers:		
Vimal Mehta, Ph.D.	57	Chief Executive Officer, President, Secretary and Director
Vincent J. O'Neill, M.D.	48	Chief Medical Officer
Richard Steinhart	60	Chief Financial Officer
Frank Yocca, Ph.D.	62	Chief Scientific Officer
Key Employees:		
Luca Rastelli, Ph.D.	50	Vice President—Oncology R&D
Chids Mahadevan	46	Vice President—Finance
Non-Employee Directors:		
Peter Mueller, Ph.D.	61	Chairman of the Board of Directors
Sandeep Laumas, M.D.	49	Director
Krishnan Nandabalan, Ph.D.	55	Director

Executive Officers

Vimal Mehta, Ph.D. has served as a director since April 2017 and as our Chief Executive Officer, President and Secretary since May 2017. He is a co-founder of BioXcel Corporation and has served as its Chairman of the Board since 2005 and its Chief Executive Officer since September 2014. Dr. Mehta has held various senior scientific and business development positions, including Senior Vice President of Business Development at London-based Inpharmatica Ltd, a global predictive informatics company, from 2002 to 2006 and Senior Vice President, Business Development for Jubilant Life Sciences, an integrated global pharmaceutical and life sciences company, from 2006 to 2007. Previously, Dr. Mehta served as Business Development Manager at CuraGen Corporation, a biotechnology company, from 1996 to 2002. He held multiple positions in the Department of Radiology at the University of Texas, Southwestern Medical Center from 1989 to 1996, including Postdoctoral Fellow, Instructor and Assistant Professor. Dr. Mehta holds a Ph.D. in Chemistry from the University of Delhi, India and completed a Post-Doctoral Fellowship in Chemistry at the University of Montpellier, France. During the length of his career, Dr. Mehta has garnered a deep understanding of the biopharma and healthcare ecosystem and has been actively involved in diverse global value generating initiatives encompassing corporate strategy and planning, global business development, and corporate fundraising. He has helped to shape the company's strategic and business trajectory and which the Board believes qualifies him to serve as a director of our company.

Vincent J. O'Neill, M.D. has served as our Chief Medical Officer since July 2017. He served as the Chief Medical Officer of Mirna Therapeutics, Inc. from April 2016 to May 2017. From June 2014 to May 2016, he served as the Chief Medical Officer of Exosome Diagnostics, Inc., a diagnostics company. From 2012 to 2014, Dr. O'Neill was global head Personalized of Medicine and Companion Diagnostics at Sanofi S.A., a pharmaceutical company. From 2009 to 2012, Dr. O'Neill served as Group Director at Genentech, Inc. where he was involved in the expanded approval of products such as Avastin and Tarceva. From 2006 to 2009, Dr. O'Neill served as Director, Discovery Medicine at GlaxoSmithkline plc. Dr. O'Neill holds an M.D., MBChd and M.Sc. in Pathology from the University of Glasgow, UK.

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Richard I. Steinhart has served as our Chief Financial Officer since October 2017. From October 2015 to June 2017 he was Vice President and CFO at Remedy Pharmaceuticals, Inc. From January 2014 to September 2015 Mr. Steinhart worked as a financial and strategic consultant to the biotechnology and medical device industries. From April 2006 through December 2013, Mr. Steinhart was employed by MELA Sciences, Inc., as their Vice President, Finance and Chief Financial Officer, Treasurer and Secretary from April 2006 to April 2012 and as Sr. Vice President, Finance and Chief Financial Officer from April 2012 to December 2013. From May 1992 until joining MELA Sciences, Mr. Steinhart was a Managing Director of Forest Street Capital/SAE Ventures, a boutique investment banking, venture capital, and management consulting firm focused on healthcare and technology companies. Prior to Forest Street Capital/SAE Ventures, he was Vice President and Chief Financial Officer of Emisphere Technologies, Inc. Mr. Steinhart's other experience includes seven years at CW Group, Inc., a venture capital firm focused on medical technology and biopharmaceutical companies, where he was a General Partner and Chief Financial Officer. Mr. Steinhart is a member of the Board of Directors of Actinium Pharmaceuticals, Inc., a position he assumed in November 2013, and Atossa Genetics, Inc., where he began his service in March 2014. Mr. Steinhart serves as the Chairman of the Audit Committee at Actinium Pharmaceuticals, where he also sits on the Compensation and Corporate Governance Committees. Mr. Steinhart serves as the Chairman of Atossa Genetics Audit Committee and is a member of its Compensation Committee. He holds B.B.A. and M.B.A. degrees from Pace University and is a Certified Public Accountant (inactive).

Frank D. Yocca, Ph.D. has served as our Chief Scientific Officer since June 2017. From April 2015 to April 2017, he was Senior Vice President, CNS R&D of BioXcel. From 2005 to 2015, Dr. Yocca held multiple leadership roles at AstraZeneca plc, including Vice President, Strategy and Externalization, Neuroscience Virtual Innovative Medicine Unit (iMed) (2011-2015), Vice President and Head, Strategy Unit, CNS and Pain Innovative Medicine Unit (iMed) (2010 to 2011) and Vice President and Head, CNS Pain Discovery (2005 to 2010). Prior to this he was Executive Director at the Bristol Myers Squibb Pharmaceutical Research Institute from 1984 to 2004 where he served concurrent leadership responsibilities within the Neuroscience Clinical Group for Early and Late Clinical Development Studies. Prior to this Dr. Yocca served as Executive Director, Neuroscience Discovery from 1997 to 2003, where he was a collaborator in the development and implementation of corporate strategic plans and leader for the Neuroscience Biology Department in the discovery of psychiatry and Alzheimer's clinical candidates. He was a core member of the Abilify Product Development and Commercialization Team from 1999 to 2002 and a core member of the Early and Late Discovery and Development Teams from 1984 to 2001. Dr. Yocca holds a B.S. in biochemistry from Manhattan College and an M.S. in pharmacology and a Ph.D. in neuropharmacology for St. John's University.

Key Employees

Chids Mahadevan has served as our Vice President—Finance since June 2017. Since April 2015 he has served as Vice President—Finance and Chief Accounting Officer of BioXcel. Prior to joining BioXcel, From 2010 to 2015, Mr. Mahadevan was the Senior Vice President, Finance at GoldenSource Corp, an enterprise data management software company where he led the global finance and accounting team. From 2007 to 2010, he was the Director of Finance at inVentiv Health Inc., a professional services organization that accelerates the clinical and commercial success of biopharmaceutical companies worldwide. Mr. Mahadevan started his career at Ramco Systems, a provider of adaptive enterprise solutions in a global market in 1996 where he progressed to become Head of Finance for the United States operations and the Finance Lead for the global aviation software segment and remained until 2007. Mr. Mahadevan holds a Bachelors in Commerce from Madras University. Mr. Mahadevan is a Certified Public Accountant in the United States and also a Chartered Accountant from India.

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Luca Rastelli, Ph.D. has served as our Vice President—Oncology R&D since June 2017. Previously, he was the Vice President of Oncology R&D of BioXcel from May 2015 to June 2017. Dr. Rastelli has more than 20 years of drug discovery and development experience in pharmaceutical, biotech and start-up companies. Dr. Rastelli has held multiple preclinical and clinical project leadership positions. He served as Head of Translational Oncology at Boston Strategics Corporation, a pharmaceutical research and development company, from 2013 to 2014, and as Global Project Leader at EMD Serono Inc., a subsidiary of Merck KGaA, Darmstadt, Germany from 2006 to 2013. Dr. Rastelli served as Senior Director Biology from 2003 to 2006 at Sopherion Therapeutics, Inc., a company that designed and developed, and commercialized novel anti-cancer drugs and molecules. Dr. Rastelli holds a Ph.D. in Molecular Biology of Development from the University of Geneva, Switzerland.

Non-Employee Directors

Peter Mueller, Ph.D. has served as a director of our company since April 2017 and Chairman of the Board since August 2017. With over 30 years of global pharma and biotech experience, Dr. Mueller is currently the President of the Mueller Health Foundation, a private foundation tackling globally lethal infectious diseases such as tuberculosis by addressing latency and the ever growing challenges of antimicrobial resistance. From 2014 to 2016, he was President of R&D and Chief Scientific Officer of Axcella Health, a biotechnology company. From 2003 to 2014, Dr. Mueller served as Executive Vice President Global Research and Development & Chief Scientific Officer for Vertex Pharmaceuticals, Incorporated, a biotechnology company. He was involved in the development of Incivek (2011), Kalydeco (2012), and Orkambi (2014). Prior to his tenure at Vertex, he served as Senior Vice President, Research and Development, for Boehringer Ingelheim Pharmaceuticals, Inc. overseeing global research programs (immunology, inflammation, cardiovascular diseases and gene therapy) and the development of all drug candidates of the company's worldwide portfolio in North and South America, Canada and Japan, beginning in 1997. He was involved in the development of Spiriva, Combivent, Atrovent and Viramune. Dr. Mueller received both an undergraduate degree and a Ph.D. in Chemistry at the Albert Einstein University of Ulm, Germany, where he also holds a Professorship in Theoretical Organic Chemistry. He completed fellowships in Quantum Pharmacology at Oxford University and in Biophysics at Rochester University. He is a member of various scientific and political societies and currently serves on the Board of Inhibikase Therapeutics and the US-India Chamber of Commerce Biotech. He also services as chairman of the Scientific Advisory Board of BioXcel and is an advisor to the University Iowa (CBB). We believe that Dr. Mueller's extensive experience in the life sciences industry as a scientist and executive qualifies him to serve as a director of our company.

Sandeep Laumas, M.D. has served as a director of our company since September 2017. He has served as a Director of BioXcel since May 2013. In August 2007, Dr. Laumas founded Bearing Circle Capital, an investment firm, and has served as its Managing Director since such time. Dr. Laumas was a Managing Director of North Sound Capital from 2003 to 2007, where he was responsible for the global healthcare investment portfolio. Dr. Laumas was an analyst at Balyasny Asset Management from 2001 to 2003. He began his career at Goldman Sachs & Co. in 1996 as an equity analyst in the healthcare investment banking division before transitioning to the healthcare equity research division. From February 2011 to February 2012 he was a member of the board of directors of Super Religare Laboratories Limited, Southeast Asia's largest clinical laboratory service company. Dr. Laumas also served as a Director of Parkway Holdings Ltd. from May to August 2010 and currently has served as the executive chairman of Innovate Biopharmaceuticals, Inc. (NASDAQ: INNT) since 2014. Dr. Laumas received his A.B. (Chemistry) from Cornell University in 1990, his M.D. from Albany Medical College, with a research year at the Dana-Farber Cancer Institute and completed his medical internship at the Yale University School of Medicine. Dr. Laumas has a novel industry perspective, particularly in both public and private investments and financial transactions in the healthcare arena, which we believe qualifies him to serve as a director of our company.

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Krishnan Nandabalan, Ph.D. has served as a director of our company since May 2017. He is a co-founder of BioXcel and has served as its President and Secretary since 2005 and Chief Scientific Officer since September 2014. He has served as a director of BioXcel since March 2005. From August 2004 to September 2005, Dr. Nandabalan served as the Vice President of Corporate Development at Genaissance Pharmaceuticals, a population genomics company, from October 2000 to August 2004, he was Vice President of Product Development, Alliances and Business Development, and from October 1998 to October 2000, he was Executive Director of Technology Systems. Prior to this, he served as Group Leader of the Functional Genomics Group at CuraGen Corporation from January 1995 to September 1998. Dr. Nandabalan was also a Founding Director of Ayugen BioSciences, a privately held company that specializes in genomic tests and services, from March 2006 to October 2015. Dr. Nandabalan holds a B.Sc. and M.Sc. in agricultural science from Tamil Nadu Agricultural University and a Ph.D. in biochemistry and molecular biology from Indian Institute of Science. During his career, Dr. Nandabalan has acquired a thorough understanding of market trends impacting the global healthcare environment, the pharma value chain, the current unmet medical needs, and in applying novel technologies to solve these needs, which we believe qualifies him to serve as a director of our company.

Family Relationships

There are no family relationships among any of our executive officers or directors.

Composition of our Board of Directors

Our board of directors currently consists of four directors. Our amended and restated certificate of incorporation will provide that the number of directors on our board of directors shall be fixed exclusively by resolution adopted by our board of directors. Our amended and restated certificate of incorporation and amended and restated bylaws will provide that our board of directors will be divided into three classes, as nearly equal in number as possible, with the directors in each class serving for a three-year term, and one class being elected each year by our stockholders.

When considering whether directors have the experience, qualifications, attributes or skills, taken as a whole, to enable our board of directors to satisfy its oversight responsibilities effectively in light of our business and structure, the board of directors focuses primarily on each person's background and experience as reflected in the information discussed in each of the directors' individual biographies set forth above. We believe that our directors provide an appropriate mix of experience and skills relevant to the size and nature of our business.

In accordance with our amended and restated certificate of incorporation and amended and restated bylaws, each of which will be in effect immediately prior to the consummation of this offering, our board of directors will be divided into three classes with staggered three year terms. At each annual meeting of stockholders after the initial classification, the successors to the directors whose terms will then expire will be elected to serve from the time of election and qualification until the third annual meeting following their election. Our directors will be divided among the three classes as follows:

- the Class I director will be Krishnan Nandabalan and his term will expire at the annual meeting of stockholders to be held in 2018;
- the Class II director will be Sandeep Laumas and his term will expire at the annual meeting of stockholders to be held in 2019; and
- the Class III directors will be Vimal Mehta and Peter Mueller and their terms will expire at the annual meeting of stockholders to be held in 2020.

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Any increase or decrease in the number of directors will be distributed among the three classes so that, as nearly as possible, each class will consist of one-third of the directors. This classification of our board of directors may have the effect of delaying or preventing changes in control of our Company.

Pursuant to the terms of our amended and restated certificate of incorporation, directors may only be removed for cause by the affirmative vote of the holders of at least a majority of our outstanding shares of common stock which are present in person or by proxy and entitled to vote.

Director Independence

Prior to the consummation of this offering, our board of directors undertook a review of the independence of our directors and considered whether any director has a relationship with us that could compromise that director's ability to exercise independent judgment in carrying out that director's responsibilities. Our board of directors has affirmatively determined that Peter Mueller and Sandeep Laumas are each an "independent director," as defined under the Nasdaq rules.

Controlled Company Exception

After the consummation of this offering, BioXcel, will, in the aggregate, have more than 50% of the combined voting power for the election of directors. As a result, we will be a "controlled company" within the meaning of the Nasdaq rules and may elect not to comply with certain corporate governance standards, including that: (i) a majority of our board of directors consists of "independent directors," as defined under the Nasdaq rules; (ii) we have a nominating and corporate governance committee that is composed entirely of independent directors with a written charter addressing the committee's purpose and responsibilities; (iii) we have a compensation committee that is composed entirely of independent directors with a written charter addressing the committee's purpose and responsibilities; and (iv) we perform annual performance evaluations of the nominating and corporate governance and compensation committees. We intend to rely on the foregoing exemptions provided to controlled companies under the Nasdaq rules. Therefore, immediately following the consummation of this offering, we may not have a majority of independent directors on our board of directors, an entirely independent nominating and corporate governance committee, an entirely independent compensation committee or perform annual performance evaluations of the nominating and corporate governance and compensation committees unless and until such time as we are required to do so. Accordingly, you may not have the same protections afforded to stockholders of companies that are subject to all of these corporate governance requirements. In the event that we cease to be a "controlled company" and our shares continue to be listed on The Nasdaq Stock Market, we will be required to comply with these provisions within the applicable transition periods. See "Risk Factors—Risks Related to Our Relationship with BioXcel" for additional information.

Committees of Our Board of Directors

Our board of directors directs the management of our business and affairs, as provided by Delaware law, and conducts its business through meetings of the board of directors and its standing committees. We will have a standing audit committee, nominating and corporate governance committee and compensation committee. In addition, from time to time, special committees may be established under the direction of the board of directors when necessary to address specific issues.

Audit Committee

Our audit committee will be responsible for, among other things:

- approve and retain the independent auditors to conduct the annual audit of our financial statements;

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- review the proposed scope and results of the audit;
- review and pre-approve audit and non-audit fees and services;
- review accounting and financial controls with the independent auditors and our financial and accounting staff;
- review and approve transactions between us and our directors, officers and affiliates;
- establish procedures for complaints received by us regarding accounting matters;
- oversee internal audit functions, if any; and
- prepare the report of the audit committee that the rules of the Securities and Exchange Commission require to be included in our annual meeting proxy statement.

Upon the consummation of this offering, our audit committee will consist of Peter Mueller and Sandeep Laumas, with Sandeep Laumas serving as chair. Rule 10A-3 of the Exchange Act and the Nasdaq rules require that our audit committee have at least one independent member upon the listing of our common stock, have a majority of independent members within 90 days of the date of this prospectus and be composed entirely of independent members within one year of the date of this prospectus. Our board of directors has affirmatively determined that Peter Mueller and Sandeep Laumas each meet the definition of "independent director" under the Nasdaq rules, and that Peter Mueller and Sandeep Laumas meets the independence standards under Rule 10A-3. Each member of our audit committee meets the financial literacy requirements of the Nasdaq rules. In addition, our board of directors has determined that Sandeep Laumas will qualify as an "audit committee financial expert," as such term is defined in Item 407(d)(5) of Regulation S-K. Our board of directors will adopt a written charter for the audit committee, which will be available on our principal corporate website at *www.bioxceltherapeutics.com* substantially concurrently with the consummation of this offering. The information on any of our websites is deemed not to be incorporated in this prospectus or to be part of this prospectus.

Compensation Committee

Our compensation committee is responsible for, among other things:

- review and recommend the compensation arrangements for management, including the compensation for our president and chief executive officer;
- establish and review general compensation policies with the objective to attract and retain superior talent, to reward individual performance and to achieve our financial goals;
- administer our stock incentive plans; and
- prepare the report of the compensation committee that the rules of the Securities and Exchange Commission require to be included in our annual meeting proxy statement.

Upon the consummation of this offering, our compensation committee will consist of Peter Mueller and Sandeep Laumas, with Peter Mueller serving as chair. Our board has determined that Sandeep Laumas and Peter Mueller are "non-employee directors" as defined in Section 16b-3 of the Exchange Act. We intend to avail ourselves of the "controlled company" exception under the Nasdaq rules, which exempts us from the requirement that we have a compensation committee composed entirely of independent directors. Our board of directors will adopt a written charter for the compensation committee, which will be available on our principal corporate website at *www.bioxceltherapeutics.com* substantially concurrently with the consummation of this offering. The information on any of our websites is deemed not to be incorporated in this prospectus or to be part of this prospectus.

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Nominating and Governance Committee

Our nominating and governance committee is responsible for, among other things:

- identify and nominate members of the board of directors;
- develop and recommend to the board of directors a set of corporate governance principles applicable to our company; and
- oversee the evaluation of our board of directors.

Upon the consummation of this offering, our nominating and corporate governance committee will consist of Sandeep Laumas, Peter Mueller and Vimal Mehta, with Peter Mueller serving as chair. We intend to avail ourselves of the "controlled company" exception under the Nasdaq rules, which exempts us from the requirement that we have a nominating and corporate governance composed entirely of independent directors. Our board of directors will adopt a written charter for the nominating and corporate governance committee, which will be available on our principal corporate website at *www.bioxceltherapeutics.com* substantially concurrently with the consummation of this offering. The information on any of our websites is deemed not to be incorporated in this prospectus or to be part of this prospectus.

Compensation Committee Interlocks and Insider Participation

None of our executive officers serves as a member of the board of directors or compensation committee (or other committee performing equivalent functions) of any entity that has one or more executive officers serving on our board of directors or compensation committee.

Code of Ethics and Code of Conduct

Prior to the completion of this offering, we will adopt a written code of business conduct and ethics that applies to our directors, officers and employees, including our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions. A copy of the code will be posted on our website, *www.bioxceltherapeutics.com*. In addition, we intend to post on our website all disclosures that are required by law or the Nasdaq rules concerning any amendments to, or waivers from, any provision of the code. The information on any of our websites is deemed not to be incorporated in this prospectus or to be part of this prospectus.

Limitations on Liability and Indemnification Matters

Upon the closing of this offering, our amended and restated certificate of incorporation will contain provisions that limit the liability of our current and former directors for monetary damages to the fullest extent permitted by Delaware law. Delaware law provides that directors of a corporation will not be personally liable for monetary damages for any breach of fiduciary duties as directors, except liability for:

- any breach of the director's duty of loyalty to the corporation or its stockholders;
- any act or omission not in good faith or that involves intentional misconduct or a knowing violation of law;
- unlawful payments of dividends or unlawful stock repurchases or redemptions as provided in Section 174 of the Delaware General Corporation Law; or
- any transaction from which the director derived an improper personal benefit.

This limitation of liability does not apply to liabilities arising under federal securities laws and does not affect the availability of equitable remedies such as injunctive relief or rescission.

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Our amended and restated certificate of incorporation to be in effect upon the closing of this offering will provide that we are authorized to indemnify our directors and officers to the fullest extent permitted by Delaware law. Our amended and restated bylaws to be in effect upon the closing of this offering will provide that we are required to indemnify our directors and executive officers to the fullest extent permitted by Delaware law. Our amended and restated bylaws will also provide that, upon satisfaction of certain conditions, we are required to advance expenses incurred by a director or executive officer in advance of the final disposition of any action or proceeding, and permit us to secure insurance on behalf of any officer, director, employee or other agent for any liability arising out of his or her actions in that capacity regardless of whether we would otherwise be permitted to indemnify him or her under the provisions of Delaware law. Our amended and restated bylaws will also provide our board of directors with discretion to indemnify our other officers and employees when determined appropriate by our board of directors. We have entered and expect to continue to enter into agreements to indemnify our directors, executive officers and other employees as determined by the board of directors. With certain exceptions, these agreements provide for indemnification for related expenses, including, among other things, attorneys' fees, judgments, fines and settlement amounts incurred by any of these individuals in any action or proceeding. We believe that these provisions and agreements are necessary to attract and retain qualified persons as directors and officers. We also maintain customary directors' and officers' liability insurance.

The limitation of liability and indemnification provisions in our amended and restated certificate of incorporation and amended and restated bylaws to be in effect upon the closing of this offering may discourage stockholders from bringing a lawsuit against our directors for breach of their fiduciary duty. They may also reduce the likelihood of derivative litigation against our directors and officers, even though an action, if successful, might benefit us and other stockholders. Further, a stockholder's investment may be adversely affected to the extent that we pay the costs of settlement and damage awards against directors and officers as required by these indemnification provisions. At present, there is no pending litigation or proceeding involving any of our directors, officers or employees for which indemnification is sought, and we are not aware of any threatened litigation that may result in claims for indemnification.

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EXECUTIVE AND DIRECTOR COMPENSATION

Our named executive officers for the years ended December 31, 2017 and 2016 include our principal executive officer and the next most highly compensated executive officers during the years ended December 31, 2017 and 2016:

- Vimal Mehta, Ph.D., our Chief Executive Officer;
- Frank Yocca, Ph.D., our Chief Scientific Officer; and
- Richard Steinhart, our Chief Financial Officer.

Summary Compensation Table

The following table presents the compensation awarded to, earned by or paid to each of our named executive officers for the years ended December 31, 2017 and 2016.

Name and Principal Position	Year	Salary ($)	Bonus ($)	Option Awards ($)[2]	All Other Compensation ($)	Total ($)
Vimal Mehta, Ph.D.[1]	2017	147,000[3]	—	125,932	10,599[4]	283,531
Chief Executive Officer, President, Secretary and Director	2016	62,250	—	—	5,098	67,348
Frank Yocca, Ph.D.	2017	168,000[5]	—	41,999	—	209,999
Chief Scientific Officer	2016	108,000	—	—	—	108,000
Richard Steinhart[6]	2017	30,000	—	318,211	6,443[7]	354,654
Chief Financial Officer						

1 Dr. Mehta is an employee of our parent, BioXcel. He provides services to us pursuant to a services agreement between us and BioXcel.

2 These amounts represent the aggregate grant date fair value for option awards for the fiscal year ended December 31, 2017, computed in accordance with Financial Accounting Standards Board Accounting Standards Codification Topic 718, or ASC 718. A discussion of the assumptions used in determining grant date fair value may be found in Note 9 to our consolidated financial statements appearing elsewhere in this prospectus.

3 Includes $10,500 of salary that was accrued but unpaid during the fiscal year ended December 31, 2017.

4 Includes the dollar value of life insurance premiums and car allowance we paid for the benefit of Dr. Mehta.

5 Includes $34,500 of salary that was accrued by unpaid during the fiscal year ended December 31, 2017.

6 Mr. Steinhart was appointed Chief Financial Officer of the Company on October 2, 2017.

7 Includes the dollar value of COBRA payments we intend to reimburse to Mr. Steinhart.

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Outstanding Equity Awards at December 31, 2017

The following table sets forth information concerning outstanding equity awards held by our 2017 named executive officers as of December 31, 2017. All equity awards set forth in the table below were granted under our Plan.

	Option Awards			
NAME	Number Of Securities Underlying Unexercised Options (#) Exercisable[1]	Number Of Securities Underlying Unexercised Options (#) Unexercisable	Option Exercise Price ($)	Option Expiration Date
Vimal Mehta, Ph.D.[1]	—	474,000	0.41	8/23/2027
Richard Steinhart[2]	—	83,898	5.55	10/2/2027
Frank Yocca, Ph.D.[3]	—	149,310	0.41	8/23/2027

1 On August 23, 2017, Dr. Mehta was awarded an option to purchase 474,000 shares of our common stock under our Plan. The shares underlying this option vest on March 31, 2018.

2 On October 2, 2017, Mr. Steinhart was awarded an option to purchase 83,898 shares of common stock under our Plan. The shares underlying this option vest as follows: 21,093 shares shall vest on October 1, 2018 and the remaining 62,805 options shall vest monthly over 36 months from October 2, 2018 through October 1, 2021.

3 On August 23, 2017, Dr. Yocca was awarded an option to purchase 149,310 shares of our common stock under our Plan. The shares underlying this option vest as follows: 37,209 shares shall vest on March 31, 2018 and the remaining 112,101 shares shall vest monthly over 36 months from August 23, 2018 through August 22, 2021.

Non-Employee Director Compensation

The following table presents the total compensation for each person who served as a non-employee member of our board of directors and received compensation for such service during the fiscal year ended December 31, 2017. Other than as set forth in the table and described more fully below, we did not pay any compensation, make any equity awards or non-equity awards to, or pay any other compensation to any of the non-employee members of our board of directors in 2017.

NAME	Fees Earned or Paid in Cash ($)	Option Awards ($)[1]	Total ($)
Peter Mueller, Ph.D.[2]	—	32,725	32,725
Sandeep Laumas, M.D.[3]	—	24,208	24,208
Krishnan Nandabalan, Ph.D.[4]	—	125,932	125,932

1 These amounts represent the grant date fair value of option awards granted to each director in the fiscal year ended December 31, 2017, computed in accordance with ASC 718. A discussion of the assumptions used in determining grant date fair value may be found in Note 9 to our consolidated financial statements appearing elsewhere in this prospectus.

2 As of December 31, 2017, Dr. Mueller held an option to purchase 37,209 shares of our common stock and an option to purchase 86,979 shares of our common stock. On December 28, 2017, the board of directors accelerated the vesting of options to purchase 124,188 shares of common stock previously granted to Dr. Mueller because of the unique scientific and business skills and guidance he has provided to us, which has resulted in an IND Exemption for BXCL501 and a clinical development plan for BXCL701. As a result,

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under ASC 718 this is considered a type 1 probable to probable modification of a vesting condition and was accounted for under ASC 718-by expensing the balance of the award during the period ending December 31, 2017. The board has no plans to accelerate any other stock options granted by the Company.

3 As of December 31, 2017, Dr. Laumas held an option to purchase 86,979 shares of our common stock, none of which has vested as of such date.

4 As of December 31, 2017, Dr. Nandabalan held an option to purchase 474,000 shares of our common stock, none of which has vested as of such date.

Non-Employee Director Compensation Policy

We plan to adopt a non-employee director compensation policy, effective upon effectiveness of the registration statement of which this prospectus forms a part, that is designed to enable us to attract and retain, on a long-term basis, highly qualified non-employee directors. Under the policy, each director who is not an employee will be paid cash compensation from and after the completion of this offering, as set forth below:

	Member Annual Fee ($)	Chairman Additional Annual Fee ($)
Board of Directors	35,000	30,000
Audit Committee	7,500	20,000
Compensation Committee	5,000	10,000
Nominating and Corporate Governance Committee	3,500	7,000

We have also agreed to issue each non-employee director options to purchase 37,209 shares of our common stock upon completion of this offering with an exercise price equal to the initial public offering price of this offering and which shall vest in three equal installments beginning on the first anniversary of the closing date of this offering. In addition, on the date of each annual meeting of stockholders of our company beginning after the completion of this offering, each non-employee director will be granted an annual equity-based award granted under our 2017 Equity Incentive Plan, equal to 10,000 shares of common stock, which shall vest in three equal installments beginning on the first anniversary of such meeting.

On August 23, 2017, in connection with the appointment of each non-employee director, we granted each of them options to purchase 86,979 shares of our common stock with an exercise price of $0.41, which vest as follows: options to purchase 29,151 shares shall vest of August 22, 2018 and options to purchase 28,914 shares shall vest on each of August 22, 2019 and August 22, 2020. In addition, on August 23, 2017, in connection with his appointment as chairman of the board of directors, we granted Dr. Mueller options to purchase 37,209 shares of our common stock with an exercise price of $0.41, which vest as follows: options to purchase 12,561 shares shall vest of August 22, 2018 and options to purchase 12,324 shares shall vest on each of August 22, 2019 and August 22, 2020. On December 28, 2017, the board of directors agreed to fully vest all of Dr. Mueller's options.

Employment Arrangements

Each of our executive officers, other than Frank Yocca, Vincent O'Neill and Richard Steinhart, are employed by our parent, BioXcel, and provide services to us pursuant to the Services Agreement between us and BioXcel. Dr. Yocca and Mr. Steinhart are each employed directly by us. Dr. O'Neill currently has a consulting agreement with us. On or prior to the date of this offering, BioXcel will have an employment agreement with each of our executive officers that sets forth the initial terms and conditions of employment. These agreements will provide for at-will employment and set forth the

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executive officer's annual base salary, performance bonus target opportunity, initial equity incentive grant, terms of severance and eligibility for employee benefits. The annual target bonus that each executive officer will be eligible to receive will be payable based on our board of director's assessment of each executive officer's individual performance and overall company performance.

Prior to this offering, our business was owned by BioXcel. Therefore, BioXcel's historical compensation strategy has been determined primarily by BioXcel's Board of Directors. The discussion below of our employment arrangements may serve as a template for our anticipated compensation structure for our named executive officers on after completion of this offering. BioXcel's compensation philosophy may be relevant to us because it is anticipated that the elements of our compensation will be similar to the elements of BioXcel's compensation. However, our compensation committee will review the impact of our separation from BioXcel and will review all aspects of compensation and make appropriate adjustments in structuring our executive compensation arrangements. As of the date hereof, our board of directors has reviewed our executive compensation arrangements however the specifics of our compensation programs and policies have not yet been determined.

Employment Agreements with BioXcel

On September 14, 2014, Vimal Mehta entered into an executive employment agreement with BioXcel in which he agreed to serve as Chief Executive Officer. The term of the agreement was effective as of September 1, 2014, continues until September 1, 2017 and automatically renews for successive one year periods at the end of each term until either party delivers written notice of their intent not to review at least 60 days prior to the expiration of the then effective term. Dr. Mehta's base salary was $125,000 per year. He is eligible to receive a bonus of up to 50% of his base salary per year at the discretion of the BioXcel Compensation Committee or as agreed to by Dr. Mehta and the Board of Directors. Dr. Mehta was also entitled to a car lease allowance of up to $750 per month. Dr. Mehta is entitled to participate in any and all benefit plans, from time to time, in effect for senior management, along with vacation, sick and holiday pay in accordance with our policies established and in effect from time to time. Life insurance premium for Dr. Mehta amounting to $5,673.20 per quarter is paid by us as an additional benefit. The agreement may be terminated by us at any time and for any reason (or no reason), and with or without cause, provided if the agreement is terminated without cause, we are required to provide him at least 60 days prior written notice. Dr. Mehta may terminate the agreement for any reasons (or no reason) upon 60 days prior written notice. If the employment agreement is terminated by us other than for cause or if Dr. Mehta terminates his employment for good reason, which includes a change of control, Dr. Mehta shall receive (i) a severance payment equal to his base compensation for the year; (ii) immediate vesting of all unvested stock options and the extension of the exercise period of such options to the later of the longest period permitted by our stock option plans or ten years following the termination date; (iii) payment in respect of any bonus earned but not yet paid; and (iv) payment of the cost of medical insurance for a period of 12 months following termination.

The employment agreement also contains covenants: (i) restricting the executive from engaging in any activity competitive with our business during the term of the employment agreement and in the event of termination for cause or without good reason, for a period of one year thereafter; (ii) prohibiting the executive from disclosing confidential information regarding us; and (iii) soliciting our suppliers, employees, customers and prospective customers during the term of the employment agreement and for a period of one year thereafter. On December 21, 2017, BioXcel entered into an amendment to Dr. Mehta's employment agreement pursuant to which his base salary was increased to $240,000 per year and his monthly car allowance was increased to $1,250, effective September 1, 2017.

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Employment Agreements with BTI

Dr. Mehta Employment Agreement

On the effective date of the registration statement of which this prospectus forms a part, Vimal Mehta will enter into an executive employment agreement with us in which he will agree to serve as Chief Executive Officer. The term of the agreement will continue for a period of 2 years from the date of execution and automatically renews for successive one year periods at the end of each term until either party delivers written notice of their intent not to review at least 90 days prior to the expiration of the then effective term. Dr. Mehta's base salary will $240,000 per year and will increased to $450,000 per year upon completion of the Company's initial public offering, or the IPO. Upon completion of the IPO, he will eligible to receive an annual bonus of up to 50% of his base salary per year at the discretion of the compensation committee as well as a special bonus of $90,000 payable upon completion of the IPO. Dr. Mehta is entitled to participate in any and all benefit plans, from time to time, in effect for senior management, along with vacation, sick and holiday pay in accordance with the Company's policies established and in effect from time to time. The agreement may be terminated by us at any time and for any reason (or no reason), and with or without cause, provided if the agreement is terminated without cause, we are required to provide him at least 90 days prior written notice. Dr. Mehta may terminate the agreement for any reasons (or no reason) upon 90 days prior written notice. If the employment agreement is terminated by us other than for cause or if Dr. Mehta terminates his employment for good reason, which includes a change of control, Dr. Mehta shall receive (i) a pro-rated bonus for the year in which such termination became effective, (ii) continued payment of his base compensation during the 24 month period following termination; (iii) immediate vesting of 50% all unvested equity awards held immediately prior to his termination date and (iv) payment of the cost of medical insurance for a period of 18 months following termination. If the Company terminates Dr. Mehta's employment and a change of control is either consummated (i) within 6 months of the effective date of such termination or (ii) no more than 12 months prior to the effective date of such termination, Dr. Mehta shall be entitled to receive a lump sum payment equal to 24 months of his base compensation. The employment agreement also contains covenants: (i) restricting the executive from engaging in any activity competitive with our business during the term of the employment agreement and for a period of one year thereafter; (ii) prohibiting the executive from disclosing confidential information regarding us; and (iii) soliciting our suppliers, employees, customers and prospective customers during the term of the employment agreement and for a period of one year thereafter.

Dr. Yocca Employment Agreement

On February 12, 2018, Frank Yocca entered into an executive employment agreement with us in which he has agreed to serve as Chief Scientific Officer. The term of the agreement will continue for a period of 2 years from the date of execution and automatically renews for successive one year periods at the end of each term until either party delivers written notice of their intent not to review at least 90 days prior to the expiration of the then effective term. Dr. Yocca's base salary will be $180,000 per year and will increased to $280,000 per year upon completion of the Company's IPO. Upon completion of the IPO, he will eligible to receive an annual bonus of up to 35% of his base salary per year at the discretion of the compensation committee as well as a special bonus of $15,000 payable upon completion of the IPO. In addition, upon completion of the IPO, Dr. Yocca will receive an option to purchase 36,498 shares of the Company's common stock at the fair market value on the date of grant. The options vest as follows: 25% on the first anniversary of the date of grant and the remaining 75% in equal monthly installments over the next 36 months following the first anniversary of the date of grant. These options will be issued under the Company's 2017 Equity Incentive Plan. Dr. Yocca is entitled to participate in any and all benefit plans, from time to time, in effect for senior management, along with vacation, sick and holiday pay in accordance with the Company's policies established and in effect from

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time to time. The agreement may be terminated by us at any time and for any reason (or no reason), and with or without cause, provided if the agreement is terminated without cause, we are required to provide him at least 90 days prior written notice. Dr. Yocca may terminate the agreement for any reasons (or no reason) upon 90 days prior written notice. If the employment agreement is terminated by us other than for cause or if Dr. Yocca terminates his employment for good reason, which includes a change of control, Dr. Yocca shall receive (i) a pro-rated bonus for the year in which such termination became effective, and (ii) continued payment of his base compensation during the 3 month period following termination, or after the IPO, continued payment of his base compensation during the 6 month period following termination. After the IPO, if the Company terminates Dr. Yocca's employment and a change of control is either consummated (i) within 6 months of the effective date of such termination or (ii) no more than 12 months prior to the effective date of such termination, Dr. Yocca shall be entitled to receive a lump sum payment equal to 6 months of his base compensation. The employment agreement also contains covenants: (i) restricting the executive from engaging in any activity competitive with our business during the term of the employment agreement and for a period of one year thereafter; (ii) prohibiting the executive from disclosing confidential information regarding us; and (iii) soliciting our suppliers, employees, customers and prospective customers during the term of the employment agreement and for a period of one year thereafter.

Mr. Steinhart Employment Agreement

Richard Steinhart entered into an executive employment agreement with us, effective October 2, 2017, in which he has agreed to serve as Chief Financial Officer. The term of the agreement will continue for a period of 2 years from the effective date and automatically renews for successive one year periods at the end of each term until either party delivers written notice of their intent not to review at least 90 days prior to the expiration of the then effective term. Mr. Steinhart's base salary will be $10,000 per month and will increased to $280,000 per year upon completion of the Company's IPO. Upon completion of the IPO, he will eligible to receive an annual bonus of up to 40% of his base salary per year at the discretion of the compensation committee as well as a special bonus of $60,000 payable upon completion of the IPO. In addition, upon completion of the IPO, Mr. Steinhart will receive an option to purchase 32,232 shares of the Company's common stock at the fair market value on the date of grant. The options vest as follows: 25% on the first anniversary of the date of grant and the remaining 75% in equal monthly installments over the next 36 months following the first anniversary of the date of grant. These options will be issued under the Company's 2017 Equity Incentive Plan. Mr. Steinhart is entitled to participate in any and all benefit plans, from time to time, in effect for senior management, along with vacation, sick and holiday pay in accordance with the Company's policies established and in effect from time to time. The agreement may be terminated by us at any time and for any reason (or no reason), and with or without cause, provided if the agreement is terminated without cause, we are required to provide him at least 90 days prior written notice. Mr. Steinhart may terminate the agreement for any reasons (or no reason) upon 90 days prior written notice. If the employment agreement is terminated by us other than for cause or if Mr. Steinhart terminates his employment for good reason, which includes a change of control, Mr. Steinhart shall receive (i) a pro-rated bonus for the year in which such termination became effective, and (ii) continued payment of his base compensation during the 3 month period following termination, or after the IPO, continued payment of his base compensation during the 6 month period following termination. After the IPO, if the Company terminates Mr. Steinhart's employment and a change of control is either consummated (i) within 6 months of the effective date of such termination or (ii) no more than 12 months prior to the effective date of such termination, Mr. Steinhart shall be entitled to receive a lump sum payment equal to 6 months of his base compensation. The employment agreement also contains covenants: (i) restricting the executive from engaging in any activity competitive with our business during the term of the employment agreement and for a period of one year thereafter; (ii) prohibiting the executive from disclosing confidential information regarding us; and (iii) soliciting

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our suppliers, employees, customers and prospective customers during the term of the employment agreement and for a period of one year thereafter.

O'Neill Strategic Advisor Agreement

Effective July 10, 2017, Vince O'Neill entered into an strategic advisor agreement with us in which he agreed to serve as our Chief Medical Officer. The term of the agreement was effective as of July 10, 2017, continues until July 10, 2018 and automatically renews for successive one year periods until terminated. We agreed to pay Dr. O'Neill $90,000 per year, provided he works for us for at least 25% of the total business hours in a month, including attending meetings, consultations, clinical strategy/plans and presentations to investors at our request and grant him non-qualified options under our 2017 Equity Incentive Plan to purchase 124,425 shares of our common stock at the fair market value on the date of grant. We also shall promptly reimburse the Dr. O'Neill for pre-approved out-of-pocket expenses, including, without limitation, reasonable travel expenses incurred in the performance of services under the Agreement. In addition, upon completion of the IPO, Dr. O'Neill will receive an option to purchase 31,047 shares of our common stock at the fair market value on the date of grant. The options vest in equal monthly installments over the 48 months following the date of grant. These options will be issued under the Company's 2017 Equity Incentive Plan. The agreement may be terminated by us or Dr. O'Neill upon 60 day written notice and for any reason (or no reason), and with or without cause, provided if the agreement is terminated by us for cause, we are not required to provide notice of termination. The agreement also contains covenants: (i) restricting the executive from engaging in any activity competitive with our business during the term of the employment agreement and for a period of six months thereafter; and (ii) prohibiting the executive from disclosing confidential information regarding us. On January 22, 2018, we entered into an amendment to Dr. O'Neill's strategic advisor agreement pursuant to which his compensation was amended to the following: (a) $90,000 annually up to and including October 9, 2017; (b) $114,000 annually from October 10, 2017 up to and including November 9, 2017; and (c) $180,000 annually from November 10, 2017 thereafter, for each full year (up to 50% of the total business hours in a month) that the Dr. O'Neill devotes to the performance of the services under the Agreement.

2017 Equity Incentive Plan

Our board of directors adopted the 2017 Equity Incentive Plan, or the Plan, on August 22, 2017. The Plan will expire on August 21, 2027. On March 4, 2018, our board and shareholders agreed to amend the Plan to add an additional 500,070 shares of common stock that may be issued pursuant to awards granted under the Plan. The purpose of the Plan is to attract and retain key personnel and to provide a means for directors, officers, managers, employees, consultants and advisors to acquire and maintain an interest in the Company, which interest may be measured by reference to the value of its common stock. The material terms of the 2017 Plan are summarized below.

Administration

The Company's board of directors or a committee appointed by the board of directors (the "Committee") will administer the Plan. The Committee will have the authority, without limitation (i) to designate Participants (defined below) to receive awards under the Plan ("Awards"), (ii) determine the types of Awards to be granted to Participants, (iii) determine the number of shares of common stock to be covered by Awards, (iv) determine the terms and conditions of any Awards granted under the Plan, (v) determine to what extent and under what circumstances Awards may be settled in cash, shares of common stock, other securities, other Awards or other property, or canceled, forfeited or suspended, (vi) determine whether, to what extent, and under what circumstances the delivery of cash, common stock, other securities, other Awards or other property and other amounts payable with respect to an Award shall be made; (vii) interpret, administer, reconcile any inconsistency in, settle any controversy

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regarding, correct any defect in and/or complete any omission in the Plan and any instrument or agreement relating to, or Award granted under, the Plan; (viii) establish, amend, suspend, or waive any rules and regulations and appoint such agents as the Committee shall deem appropriate for the proper administration of the Plan; (ix) accelerate the vesting or exercisability of, payment for or lapse of restrictions on, Awards; (x) reprice existing Awards with shareholder approval or to grant Awards in connection with or in consideration of the cancellation of an outstanding Award with a higher price; and (xi) make any other determination and take any other action that the Committee deems necessary or desirable for the administration of the Plan. The Committee will have full discretion to administer and interpret the Plan and to adopt such rules, regulations and procedures as it deems necessary or advisable and to determine, among other things, the time or times at which the awards may be exercised and whether and under what circumstances an award may be exercised.

Eligibility

Employees, directors, officers, advisors and consultants of the Company or its affiliates are eligible to participate in the Plan and are referred to as "Participants". The Committee has the sole and complete authority to determine who will be granted an Award under the Plan, however, it may delegate such authority to one or more officers of the Company under the circumstances set forth in the Plan.

Number of Shares Authorized

Up to 3,462,570 shares of common stock may be issued pursuant to awards granted under the Plan.

If an Award is forfeited, canceled, or if any Option terminates, expires or lapses without being exercised, the common stock subject to such Award will again be made available for future grant. However, shares that are used to pay the exercise price of an Option or that are withheld to satisfy the Participant's tax withholding obligation will not be available for re-grant under the Plan.

If there is any change in the Company's corporate capitalization or structure, the Committee in its sole discretion may make substitutions or adjustments to the number of shares of common stock reserved for issuance under the Plan, the number of shares covered by Awards then outstanding under the Plan, the limitations on Awards under the Plan, the exercise price of outstanding Options and such other equitable substitution or adjustments as it may determine appropriate.

The Plan will have a term of ten years and no further Awards may be granted under the Plan after that date.

Awards Available for Grant

The Committee may grant Awards of Non-Qualified Stock Options, Incentive Stock Options, Stock Appreciation Rights, Restricted Stock Awards, Restricted Stock Units, Stock Bonus Awards, Performance Compensation Awards (including cash bonus awards) or any combination of the foregoing. Notwithstanding, the Committee may not grant to any one person in any one calendar year Awards (i) for more than 50% of the available shares under the Plan in the aggregate or (ii) payable in cash in an amount exceeding $10,000,000 in the aggregate.

Options

The Committee will be authorized to grant Options to purchase common stock that are either "qualified," meaning they are intended to satisfy the requirements of Section 422 of the Internal Revenue Code of 1986, as amended (the "Code") for Incentive Stock Options, or "non-qualified," meaning they are not intended to satisfy the requirements of Section 422 of the Code. Options granted

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under the Plan will be subject to the terms and conditions established by the Committee. Under the terms of the Plan, unless the Committee determines otherwise in the case of an Option substituted for another Option in connection with a corporate transaction, the exercise price of the Options will not be less than the fair market value (as determined under the Plan) of the shares of common stock on the date of grant. Options granted under the Plan will be subject to such terms, including the exercise price and the conditions and timing of exercise, as may be determined by the Committee and specified in the applicable award agreement. The maximum term of an Option granted under the Plan will be ten years from the date of grant (or five years in the case of an Incentive Stock Option granted to a 10% stockholder). Payment in respect of the exercise of an Option may be made in cash or by check, by surrender of unrestricted shares of common stock (at their fair market value on the date of exercise) that have been held by the participant for any period deemed necessary by the Company's accountants to avoid an additional compensation charge or have been purchased on the open market, or the Committee may, in its discretion and to the extent permitted by law, allow such payment to be made through a broker-assisted cashless exercise mechanism, a net exercise method, or by such other method as the Committee may determine to be appropriate.

Stock Appreciation Rights

The Committee will be authorized to award Stock Appreciation Rights (or SARs) under the Plan. SARs will be subject to such terms and conditions as established by the Committee. A SAR is a contractual right that allows a participant to receive, either in the form of cash, shares or any combination of cash and shares, the appreciation, if any, in the value of a share over a certain period of time. A SAR granted under the Plan may be granted in tandem with an option and SARs may also be awarded to a participant independent of the grant of an Option. SARs granted in connection with an Option shall be subject to terms similar to the Option which corresponds to such SARs. SARs shall be subject to terms established by the Committee and reflected in the award agreement.

Restricted Stock

The Committee will be authorized to award Restricted Stock under the Plan. The Committee will determine the terms of such Restricted Stock awards. Restricted Stock are shares of common stock that generally are non-transferable and subject to other restrictions determined by the Committee for a specified period. Unless the Committee determines otherwise or specifies otherwise in an award agreement, if the Participant terminates employment or services during the restricted period, then any unvested restricted stock will be forfeited.

Restricted Stock Unit Awards

The Committee will be authorized to award Restricted Stock Unit awards. The Committee will determine the terms of such Restricted Stock Units. Unless the Committee determines otherwise or specifies otherwise in an award agreement, if the Participant terminates employment or services during the period of time over which all or a portion of the units are to be earned, then any unvested units will be forfeited. At the election of the Committee, the Participant will receive a number of shares of common stock equal to the number of units earned or an amount in cash equal to the fair market value of that number of shares at the expiration of the period over which the units are to be earned or at a later date selected by the Committee.

Stock Bonus Awards

The Committee will be authorized to grant Awards of unrestricted shares of common stock or other Awards denominated in shares of common stock, either alone or in tandem with other Awards, under such terms and conditions as the Committee may determine.

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Performance Compensation Awards

The Committee will be authorized to grant any Award under the Plan in the form of a Performance Compensation Award by conditioning the vesting of the Award on the attainment of specific performance criteria of the Company and/or one or more affiliates, divisions or operational units, or any combination thereof, as determined by the Committee. The Committee will select the performance criteria based on one or more of the following factors: (i) revenue; (ii) sales; (iii) profit (net profit, gross profit, operating profit, economic profit, profit margins or other corporate profit measures); (iv) earnings (EBIT, EBITDA, earnings per share, or other corporate profit measures); (v) net income (before or after taxes, operating income or other income measures); (vi) cash (cash flow, cash generation or other cash measures); (vii) stock price or performance; (viii) total stockholder return (stock price appreciation plus reinvested dividends divided by beginning share price); (ix) economic value added; (x) return measures (including, but not limited to, return on assets, capital, equity, investments or sales, and cash flow return on assets, capital, equity, or sales); (xi) market share; (xii) improvements in capital structure; (xiii) expenses (expense management, expense ratio, expense efficiency ratios or other expense measures); (xiv) business expansion or consolidation (acquisitions and divestitures); (xv) internal rate of return or increase in net present value; (xvi) working capital targets relating to inventory and/or accounts receivable; (xvii) inventory management; (xviii) service or product delivery or quality; (xix) customer satisfaction; (xx) employee retention; (xxi) safety standards; (xxii) productivity measures; (xxiii) cost reduction measures; and/or (xxiv) strategic plan development and implementation.

Transferability

Each Award may be exercised during the Participant's lifetime only by the Participant or, if permissible under applicable law, by the Participant's guardian or legal representative and may not be otherwise transferred or encumbered by a Participant other than by will or by the laws of descent and distribution. The Committee, however, may permit Awards (other than Incentive Stock Options) to be transferred to family members, a trust for the benefit of such family members, a partnership or limited liability company whose partners or stockholders are the Participant and his or her family members or anyone else approved by it.

Amendment

The Plan will have a term of ten years. The Company's board of directors may amend, suspend or terminate the Plan at any time; however, shareholder approval to amend the Plan may be necessary if the law or SEC so requires. No amendment, suspension or termination will materially and adversely affect the rights of any Participant or recipient of any Award without the consent of the Participant or recipient.

Change in Control

Except to the extent otherwise provided in an Award or required by applicable law, in the event of a Change in Control (as defined in the Plan), upon the occurrence of a Change in Control, the Committee is authorized, but not obligated, to make any of the following adjustments (or any combination thereof) in the terms and conditions of outstanding Awards: (i) continuation or assumption of outstanding Awards by the surviving company; (ii) substitution by the surviving company of equity, equity-based and/or cash awards with substantially the same terms for outstanding Awards; (iii) accelerated exercisability, vesting and/or lapse of restrictions under outstanding Awards immediately prior to the occurrence of the Change in Control; (iv) upon written notice, provide that any outstanding Awards must be exercised, to the extent then exercisable, during a reasonable period determined by the Committee and at the end of such period, any unexercised Awards will terminate;

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and (v) cancellation of all or any portion of outstanding Awards for fair value (in the form of cash, shares or other property) and which value may be zero.

2017 Option Grants

On August 23, 2017, we issued the following options to purchase shares of our common stock and on such vesting terms at a price of $0.41 per share under the Plan to our executive officers, strategic advisors, directors and key employees as follows:

Name	Options Granted	Vesting Schedule
Vimal Mehta, Ph.D.	474,000	Shares shall vest on March 31, 2018.
Krishnan Nandabalan, Ph.D.	474,000	Shares shall vest on March 31, 2018.
Peter Mueller, Ph.D.	86,979	Shares vested in December 2017.
Sheila Gujrathi, Ph.D.	86,979	Options to purchase 29,151 shares shall vest of August 22, 2018 and options to purchase 28,914 shares shall vest on each of August 22, 2019 and August 22, 2020.
Steve Paul, M.D.	86,979	Options to purchase 29,151 shares shall vest of August 22, 2018 and options to purchase 28,914 shares shall vest on each of August 22, 2019 and August 22, 2020.
Peter Mueller, Ph.D.	37,209	Shares vested in December 2017.
Frank Yocca, Ph.D.	149,310	Options to purchase 37,209 shares shall vest on March 31, 2018, and the remaining 112,101 shares vesting monthly over 36 months from August 23, 2018 through August 22, 2021.
Chids Mahadevan	93,141	Options to purchase 23,226 shares shall vest on March 31, 2018, and the remaining 69,915 monthly over 36 months from August 23, 2018 through August 22, 2021.
Vince O'Neill, M.D.	124,425	Options to purchase 31,047 shares shall vest on August 22, 2018, and the remaining 93,378 shares vesting monthly over 36 months from August 23, 2018 through August 22, 2021.
Sandeep Laumas, M.D.	86,979	Options to purchase 29,151 shares shall vest of August 22, 2018 and options to purchase 28,914 shares shall vest on each of August 22, 2019 and August 22, 2020.
Luca Rastelli, Ph.D.	62,094	Options to purchase 15,642 shares shall vest on August 22, 2018 and options to purchase 46,452 shares shall vest over 36 months from August 23, 2018 through August 22, 2021.

On October 2, 2017, we issued Richard Steinhart an option to purchase 83,898 shares of common stock at a price of $5.55 under our Plan. The shares underlying this option vest as follows: 21,093 shares shall vest on October 1, 2018 and the remaining 62,805 options shall vest monthly over 36 months from October 2, 2018 through October 1, 2021.

We have also agreed to issue our advisor Dr. Gujrathi options to purchase 37,209 shares of our common stock upon completion of this offering with an exercise price equal to the initial public offering price of this offering and which shall vest in three installments beginning on the first anniversary of the closing date of this offering.

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CERTAIN RELATIONSHIPS AND RELATED PERSON TRANSACTIONS

The following is a summary of transactions and series of similar transactions, since our inception on March 29, 2017 to which we have been a participant in which the amount involved exceeded or will exceed $120,000 and in which any of our director, executive officer, holder of more than 5% of our capital stock, promotor or certain control person or any member of their immediate family had or will have a direct or indirect material interest.

Amended and Restated Asset Contribution Agreement with BioXcel

We have entered into an asset contribution agreement, effective June 30, 2017, with BioXcel, as amended and restated on November 7, 2017, or the Contribution Agreement, pursuant to which BioXcel agreed to contribute to us, and we agree to acquire from BioXcel, all of BioXcel's rights, title and interest in and to BXCL501, BXCL701, BXCL502 and BXCL702, collectively, the Candidates, and all of the assets and liabilities associated with the Candidates, in consideration for (i) 9,480,000 shares of our common stock, (ii) $1 million upon completion of this offering, (ii) $500,000 upon the later of the 12 month anniversary of this offering and the first dosing of a patient in the bridging bioavailability/bioequivalence study for the BXCL501 program, (iii) $500,000 upon the later of the 12 month anniversary of this offering and the first dosing of a patient in the Phase 2 PoC open label monotherapy or combination trial with Keytruda for the BXCL701 program and (iv) a one-time payment of $5 million within 60 days after the achievement of $50 million in cumulative net sales of any product or combination of products resulting from the development and commercialization of any one of the Candidates or a product derived therefrom.

In addition, pursuant to the Contribution Agreement, upon completion of this offering, BioXcel will grant us a first right to negotiate exclusive rights to any additional product candidates in the fields of neuroscience and immuno-oncology, or the Option Field, that BioXcel may identify on its own, excluding the Candidates, and not in connection with BioXcel's provision of services to us under the Services Agreement as defined and described below. This option for first negotiation shall be valid for a period of five years from the date of this offering. Within 60 days of identifying a potential product candidate in the Option Field, BioXcel shall present such identified candidate to us and we shall then have up to 180 days in which to evaluate such product candidate, or the Evaluation Period. If we wish to negotiate for the exclusive rights to such product candidate, we shall notify BioXcel in writing prior to the end of the Evaluation Period, and upon such notification, we and BioXcel shall negotiate in good faith commercially reasonable terms pursuant to which we can receive BioXcel's rights to such product candidate. If we are unable to mutually agree, in writing, within 90 days after the end of the Evaluation Period to terms regarding our rights to develop and/or commercialize such product candidate, BioXcel shall be free to develop and/or commercialize such product candidate either by itself or with one or more third parties. Prior to the fifth anniversary of this offering, BioXcel has also agreed to not provide product identification collaborative services to third parties in the fields of neuroscience or immuno-oncology when such third parties utilize EvolverAI.

Amended and Restated Separation and Shared Services Agreement

We have entered into a separation and shared services agreement, dated June 30, 2017, or the Effective Date, with BioXcel, as amended and restated on November 7, 2017, or the Services Agreement, pursuant to which BioXcel will provide us with shared office space and equipment, shared services, including the use of EvolverAI, leased employee services and financial support and payment, until the termination of the agreement as described below. In consideration for the use of office space and equipment as well as for general administrative support and payroll services, we have agreed to pay BioXcel a fixed monthly fee of $2,850 as set forth in the Services Agreement. In addition, any services related to intellectual property prosecution and management will be provided at an hourly rate of $250, subject to increase upon completion of the this offering to an hourly rate of $500, for a maximum of 20 hours per month. Any services provided by BioXcel through its subsidiary in India will be provided at

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hourly rates based on the same rates offered to third parties in an arms length transaction as set forth in the Services Agreement. Finally, BioXcel has agreed to provide us the services of Vimal Mehta and Chids Mahadevan, our Chief Executive Officer and Vice President—Finance, respectively, at 90% of their aggregate compensation, which as of the date of this offering is currently $240,000 and $220,000, respectively. We have agreed to pay invoices generated by BioXcel within 60 days of receipt thereof.

On or before December 31, 2019, we shall have the option to enter into a collaborative services agreement with BioXcel pursuant to which BioXcel shall perform product identification and related services for us utilizing EvolverAI. We have agreed that this agreement will be negotiated in good faith and that such agreement will incorporate reasonable market based terms, including consideration for BioXcel reflecting a low, single-digit royalty on net sales and reasonable development and commercialization milestone payments, provided that (i) development milestones shall not exceed $10 million in the aggregate and not be payable prior to proof of concept in humans and (ii) commercialization milestones shall be based on reaching annual net sales levels, be limited to 3% of the applicable net sales level, and not exceed $30 million in the aggregate. BioXcel shall continue to make such product identification and related services available to us for at least 60 months after the Effective Date.

In connection with the Services Agreement, BioXcel agreed to provide us a line of credit, which shall be capped at $1 million, or the Total Funding Amount, pursuant to the terms of the grid note (as discussed below), or the Grid Note. We have also agreed to reimburse BioXcel for its contributed services and support to us in connection with our organization and development prior to the date of the Grid Note in the amount of $562,000, subsequently reduced to $440,000 as of December 31, 2017 which amount shall be payable upon the earlier of (i) 30 days after the completion of this offering and (ii) December 31, 2018.

The parties have agreed that the services and office space provided under the Services Agreement shall decrease over time until the 12 month anniversary of the Effective Date, except for services to be provided by BioXcel through its subsidiary in India, which shall decrease until the 24 to 36 month anniversary of the Effective Date, provided such dates may be extended upon mutual agreement between the parties, collectively, the Term.

The Services Agreement shall terminate at the end of the Term, however, it may be terminated upon the mutual written agreement of the parties. In addition, the Services Agreement may be terminated by the non-defaulting party upon or after the occurrence of a material breach by the other party that is uncured within 30 days after receipt of written notification of such breach. If such breach is not correctable within 30 days, the correction must be initiated within 30 days and thereafter diligently pursued thereafter. Lastly, the shared services agreement may be terminated if either we become bankrupt or insolvent, make any assignment for the benefit of creditors, or if a receive is appointed and such proceeding is not vacated or terminated within 30 days after its commencement or institution.

Grid Note

In connection with the Services Agreement, BioXcel agreed to provide us a line of credit up to the Total Funding Amount pursuant to the terms of the Grid Note. BioXcel shall not be obligated to fund our operations beyond the Total Funding Amount, provided, in the event we determine that we will require additional funding to support our operations and to execute the plan of separation from BioXcel, we and BioXcel will, in good faith, assess increasing the Total Funding Amount, and, shall amend the terms of the Grid Note or execute a new note to reflect any new funding as agreed upon between the parties. The Grid Note shall be payable upon the earlier of (i) the completion of this offering and (ii) December 31, 2018, together with interest on the unpaid balance of each advance, which shall accrue at a rate per annum equal to the applicable federal rate for short-term loans as of the date hereof, in each case calculated based on a 365-day year and actual days elapsed. As of December 31, 2017, we have drawn an amount of $371,000 under the Grid Note.

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Other Transactions

On September 29, 2017, we sold 41,475 shares of our common stock to Peter Mueller, the chairman of our board of directors, at a price of $4.82 share for aggregate gross proceeds to us of $200,000.

On January 3, 2018, we sold 145,518 shares of our common stock to Peter Mueller, the chairman of our board of directors, at a price of $6.88 share for aggregate gross proceeds to us of approximately $1,000,000.

We have granted stock options to members of our board of directors and executive officers. For a description of these stock options, see the section titled "Executive and Director Compensation."

Indemnification Agreements

In connection with this offering, we will enter into indemnification agreements with each of our directors and executive officers. These indemnification agreements will provide the directors and executive officers with contractual rights to indemnification and expense advancement that are, in some cases, broader than the specific indemnification provisions contained under Delaware law. See "Description of Share Capital—Indemnification of Directors and Officers" for additional information regarding indemnification under Delaware law and our amended and restated by-laws.

Related Person Transaction Policy

Prior to this offering, we have not had a formal policy regarding approval of transactions with related parties. We expect to adopt a related person transaction policy that sets forth our procedures for the identification, review, consideration and approval or ratification of related person transactions. The policy will become effective immediately upon the execution of the underwriting agreement for this offering. For purposes of our policy only, a related person transaction is a transaction, arrangement or relationship, or any series of similar transactions, arrangements or relationships, in which we and any related person are, were or will be participants in which the amount involved exceeds $120,000. Transactions involving compensation for services provided to us as an employee or director are not covered by this policy. A related person is any executive officer, director or beneficial owner of more than 5% of any class of our voting securities, including any of their immediate family members and any entity owned or controlled by such persons.

Under the policy, if a transaction has been identified as a related person transaction, including any transaction that was not a related person transaction when originally consummated or any transaction that was not initially identified as a related person transaction prior to consummation, our management must present information regarding the related person transaction to our audit committee, or, if audit committee approval would be inappropriate, to another independent body of our board of directors, for review, consideration and approval or ratification. The presentation must include a description of, among other things, the material facts, the interests, direct and indirect, of the related persons, the benefits to us of the transaction and whether the transaction is on terms that are comparable to the terms available to or from, as the case may be, an unrelated third party or to or from employees generally. Under the policy, we will collect information that we deem reasonably necessary from each director, executive officer and, to the extent feasible, significant shareholder to enable us to identify any existing or potential related-person transactions and to effectuate the terms of the policy. In addition, under our Code of Conduct, our employees and directors will have an affirmative responsibility to disclose any transaction or relationship that reasonably could be expected to give rise to a conflict of interest. In considering related person transactions, our audit committee, or other independent body of our board of directors, will take into account the relevant available facts and circumstances including, but not limited to:

- the risks, costs and benefits to us;

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- the impact on a director's independence in the event that the related person is a director, immediate family member of a director or an entity with which a director is affiliated;
- the availability of other sources for comparable services or products; and
- the terms available to or from, as the case may be, unrelated third parties or to or from employees generally.

The policy requires that, in determining whether to approve, ratify or reject a related person transaction, our audit committee, or other independent body of our board of directors, must consider, in light of known circumstances, whether the transaction is in, or is not inconsistent with, our best interests and those of our shareholders, as our audit committee, or other independent body of our board of directors, determines in the good faith exercise of its discretion.

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PRINCIPAL SHAREHOLDERS

The following table sets forth certain information regarding the beneficial ownership of our common stock as of December 31, 2017 by:

- each of our named executive officers;
- each of our directors;
- all of our current directors and executive officers as a group; and
- each stockholder known by us to own beneficially more than five percent of our common stock.

Beneficial ownership is determined in accordance with the rules of the Securities and Exchange Commission and includes voting or investment power with respect to the securities. Shares of common stock that may be acquired by an individual or group within 60 days of December 31, 2017, pursuant to the exercise of options or warrants, are deemed to be outstanding for the purpose of computing the percentage ownership of such individual or group, but are not deemed to be outstanding for the purpose of computing the percentage ownership of any other person shown in the table. Percentage of ownership is based on 9,907,548 shares of common stock outstanding on December 31, 2017, and 15,362,093 shares of common stock outstanding after the completion of this offering.

Except as indicated in footnotes to this table, we believe that the stockholders named in this table have sole voting and investment power with respect to all shares of common stock shown to be beneficially owned by them, based on information provided to us by such stockholders. Unless otherwise indicated, the address for each director and executive officer listed is: c/o BioXcel Therapeutics, Inc., 780 East Main Street, Branford, CT 06405.

Name of Beneficial Owner	Number of Shares Beneficially Owned Prior to Offering	Percentage of Common Stock Beneficially Owned	
		Before Offering	After Offering[1]
Directors and Executive Officers			
Vimal Mehta, Ph.D.	—	*	*
Peter Mueller, Ph.D.[2]	165,663	1.6%	1.1%
Frank D. Yocca, Ph.D.	—	*	*
Krishnan Nandabalan, Ph.D.[3]	9,480,000	95.7%	61.7%
Sandeep Laumas, M.D.	—	*	*
All current executive officers and directors as a group (7 persons)	9,636,663		
5% or Greater Stockholders	—	*	*
BioXcel Corporation 780 East Main Street, Branford, CT 06405	9,480,000	95.7%	61.7%

* Represents beneficial ownership of less than one percent (1%).

1 Assuming the underwriters do not exercise their option to acquire additional securities, as described in the section "Underwriting" below. If they do exercise in full their option to acquire additional securities, we estimate BioXcel will own approximately 58.6% of our outstanding shares of common stock immediately after this offering.

2 Includes options to purchase 124,188 shares of common stock and excludes 145,518 shares of common stock purchased by Dr. Mueller in January 2018.

3 Dr. Nandabalan owns approximately 43% of the voting stock of BioXcel and may be deemed the beneficial owner of the shares held by BioXcel.

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DESCRIPTION OF CAPITAL STOCK

General

Upon completion of this offering, our authorized capital stock will consist of 50,000,000 shares of common stock, par value $0.001 per share, and 10,000,000 shares of preferred stock, par value $0.001 per share. As of December 31, 2017, there were 9,907,548 shares of common stock, and no shares of preferred stock issued and outstanding.

The following description of our capital stock and provisions of our amended and restated certificate of incorporation and amended and restated bylaws to be effective upon the completion of this offering is only a summary. You should also refer to our amended and restated certificate of incorporation, a copy of which is incorporated by reference as an exhibit to the registration statement of which this prospectus is a part, and our amended and restated bylaws, a copy of which is incorporated by reference as an exhibit to the registration statement of which this prospectus is a part.

Common Stock

We are authorized to issue up to a total of 50,000,000 shares of common stock, par value $0.001 per share. Holders of our common stock are entitled to one vote for each share held on all matters submitted to a vote of our stockholders. Holders of our common stock have no cumulative voting rights.

Further, holders of our common stock have no preemptive or conversion rights or other subscription rights. Upon our liquidation, dissolution or winding- up, holders of our common stock are entitled to share in all assets remaining after payment of all liabilities and the liquidation preferences of any of our outstanding shares of preferred stock. Subject to preferences that may be applicable to any outstanding shares of preferred stock, holders of our common stock are entitled to receive dividends, if any, as may be declared from time to time by our board of directors out of our assets which are legally available. Such dividends, if any, are payable in cash, in property or in shares of capital stock. Each outstanding share of our common stock is, and all shares of common stock to be issued in this offering when they are paid for will be, fully paid and non-assessable.

The holders of a majority of the shares of our capital stock, represented in person or by proxy, are necessary to constitute a quorum for the transaction of business at any meeting. If a quorum is present, an action by stockholders entitled to vote on a matter is approved if the number of votes cast in favor of the action exceeds the number of votes cast in opposition to the action, with the exception of the election of directors, which requires a plurality of the votes cast.

Preferred Stock

Our board of directors has the authority, without further action by the stockholders, to issue up to 10,000,000 shares of preferred stock in one or more series and to fix the designations, powers, preferences, privileges, and relative participating, optional, or special rights as well as the qualifications, limitations, or restrictions of the preferred stock, including dividend rights, conversion rights, voting rights, terms of redemption, and liquidation preferences, any or all of which may be greater than the rights of the common stock. Our board of directors, without stockholder approval, can issue convertible preferred stock with voting, conversion, or other rights that could adversely affect the voting power and other rights of the holders of common stock. Preferred stock could be issued quickly with terms calculated to delay or prevent a change of control or make removal of management more difficult. Additionally, the issuance of preferred stock may have the effect of decreasing the market price of our common stock, and may adversely affect the voting and other rights of the holders of common stock. At present, we have no plans to issue any shares of preferred stock following this offering.

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Options

Our 2017 Equity Incentive Plan, or the Plan, provides for us to sell or issue shares of common stock or restricted shares of common stock, or to grant incentive stock options or nonqualified stock options, stock appreciation rights and restricted stock unit awards for the purchase of shares of common stock, to employees, members of the board of directors and consultants. As of December 31, 2017, options to purchase 2,303,877 common shares were outstanding. For additional information regarding the terms of the Plan, see "Executive and Director Compensation—2017 Equity Incentive Plan."

Piggyback Registration Rights

We have granted one of our stockholders certain piggyback registration rights with respect to their shares of common stock. If we propose to register any of our securities under the Securities Act either for our own account or for the account of other stockholders (other than in connection with this offering), such holder will be entitled to notice of the registration and will be entitled to include their shares of common stock in the registration statement, provided, however, that the Company shall not be required to register the resale of any shares of common stock that are eligible for resale pursuant to Rule 144 under the Securities Act without any requirement for the Company to maintain current public information and without any limitation on volume or manner of sale. 207,375 shares of our common stock are entitled to these piggyback registration rights.

Anti-Takeover Provisions of Delaware Law, our Amended and Restated Certificate of Incorporation and our Amended and Restated Bylaws

Delaware Law

We are governed by the provisions of Section 203 of the Delaware General Corporation Law. In general, Section 203 prohibits a publicly traded Delaware corporation from engaging in a business combination with an interested stockholder for a period of three years after the date of the transaction in which the person became an interested stockholder, unless the business combination is approved in a prescribed manner. A business combination includes mergers, asset sales or other transactions resulting in a financial benefit to the stockholder. An interested stockholder is a person who, together with affiliates and associates, owns (or within three years, did own) 15% or more of the corporation's voting stock, subject to certain exceptions. The statute could have the effect of delaying, deferring or preventing a change in control of our company.

Board of Directors Vacancies

Our amended and restated certificate of incorporation and amended and restated bylaws authorize only our board of directors to fill vacant directorships. In addition, the number of directors constituting our board of directors may be set only by resolution of the majority of the incumbent directors.

Stockholder Action; Special Meeting of Stockholders

Our amended and restated certificate of incorporation and amended and restated bylaws provide that our stockholders may not take action by written consent. Our amended and restated certificate of incorporation and amended and restated bylaws further provide that special meetings of our stockholders may be called by a majority of the board of directors, the Chief Executive Officer, or the Chairman of the board of directors.

Advance Notice Requirements for Stockholder Proposals and Director Nominations

Our amended and restated bylaws provide that stockholders seeking to bring business before our annual meeting of stockholders, or to nominate candidates for election as directors at our annual meeting of stockholders, must provide timely notice of their intent in writing. To be timely, a stockholder's notice must be delivered to the secretary at our principal executive offices not later than

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the close of business on the 90th day nor earlier than the close of business on the 120th day prior to the first anniversary of the preceding year's annual meeting; provided, however, that in the event the date of the annual meeting is more than 30 days before or more than 60 days after such anniversary date, or if no annual meeting was held in the preceding year, notice by the stockholder to be timely must be so delivered not earlier than the close of business on the 120th day prior to such annual meeting and not later than the close of business on the later of the 90th day prior to such annual meeting or the 10th day following the day on which a public announcement of the date of such meeting is first made by us. These provisions may preclude our stockholders from bringing matters before our annual meeting of stockholders or from making nominations for directors at our annual meeting of stockholders.

Authorized but Unissued Shares

Our authorized but unissued shares of common stock and preferred stock are available for future issuance without stockholder approval and may be utilized for a variety of corporate purposes, including future public offerings to raise additional capital, corporate acquisitions and employee benefit plans. The existence of authorized but unissued and unreserved common stock and preferred stock could render more difficult or discourage an attempt to obtain control of us by means of a proxy contest, tender offer, merger or otherwise. If we issue such shares without stockholder approval and in violation of limitations imposed by the Nasdaq Capital Market or any stock exchange on which our stock may then be trading, our stock could be delisted.

Transfer Agent and Registrar

The transfer agent and registrar for our common stock is American Stock Transfer and Trust Company.

Stock Market Listing

We have been approved, subject to notice of issuance, to list our common stock on The Nasdaq Capital Market under the symbol "BTAI."

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SHARES ELIGIBLE FOR FUTURE SALE

Prior to this offering, there has been no public market for our common stock, and a liquid trading market for our common stock may not develop or be sustained after this offering. Future sales of substantial amounts of our common stock in the public market, or the anticipation of these sales, could materially and adversely affect market prices prevailing from time to time, and could impair our ability to raise capital through sales of equity or equity-related securities.

Only a limited number of shares of our common stock will be available for sale in the public market for a period of several months after completion of this offering due to contractual and legal restrictions on resale described below. Nevertheless, sales of a substantial number of shares of our common stock in the public market after such restrictions lapse, or the perception that those sales may occur, could materially and adversely affect the prevailing market price of our common stock. Although we have been approved, subject to notice of issuance, to list our common stock on The Nasdaq Capital Market, we cannot assure you that there will be an active market for our common stock.

Of the shares to be outstanding immediately after the completion of this offering, we expect that the shares to be sold in this offering will be freely tradable without restriction under the Securities Act unless purchased by our "affiliates," as that term is defined in Rule 144 under the Securities Act; provided any shares purchased by participants in our directed share program who purchase $100,000 or more of shares of our common stock will be subject to a 30-day lock-up period. The remaining shares of our common stock outstanding after this offering will be subject to a 180-day lock-up period under the lock-up agreements as described below. These restricted securities may be sold in the public market only if registered or pursuant to an exemption from registration, such as Rule 144 or Rule 701 under the Securities Act.

Rule 144

Affiliate Resales of Restricted Securities

Affiliates of ours must generally comply with Rule 144 if they wish to sell any shares of our common stock in the public market, whether or not those shares are "restricted securities." "Restricted securities" are any securities acquired from us or one of our affiliates in a transaction not involving a public offering. All shares of our common stock issued prior to the closing of the offering made hereby, are considered to be restricted securities. The shares of our common stock sold in this offering are not considered to be restricted securities.

Non-Affiliate Resales of Restricted Securities

Any person or entity who is not an affiliate of ours and who has not been an affiliate of ours at any time during the three months preceding a sale is only required to comply with Rule 144 in connection with sales of restricted shares of our common stock. Subject to the lock-up agreements described below, those persons may sell shares of our common stock that they have beneficially owned for at least one year without any restrictions under Rule 144 immediately following the effective date of the registration statement of which this prospectus is a part.

Further, beginning 90 days after the effective date of the registration statement of which this prospectus is a part, a person who is not an affiliate of ours at the time such person sells shares of our common stock, and has not been an affiliate of ours at any time during the three months preceding such sale, and who has beneficially owned such shares of our common stock, as applicable, for at least six months but less than a year, is entitled to sell such shares so long as there is adequate current public information, as defined in Rule 144, available about us.

Resales of restricted shares of our common stock by non-affiliates are not subject to the manner of sale, volume limitation or notice filing provisions of Rule 144, described above.

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Rule 701

Rule 701 generally allows a stockholder who purchased shares of our common stock pursuant to a written compensatory plan or contract and who is not deemed to have been an affiliate of ours during the immediately preceding 90 days to sell these shares in reliance upon Rule 144, but without being required to comply with the public information, holding period, volume limitation, or notice provisions of Rule 144.

Rule 701 also permits affiliates of ours to sell their Rule 701 shares under Rule 144 without complying with the holding period requirements of Rule 144. All holders of Rule 701 shares, however, are required to wait until 90 days after the date of this prospectus before selling such shares pursuant to Rule 701 and until expiration of the 180-day lock-up period described below.

Equity Incentive Awards

We intend to file a registration statement on Form S-8 under the Securities Act after the closing of this offering to register the shares of common stock that are issuable pursuant to our Plan. The registration statement is expected to be filed and become effective as soon as practicable after the completion of this offering. Accordingly, shares registered under the registration statement will be available for sale in the open market following its effective date, subject to Rule 144 volume limitations and the lock-up arrangement described above, if applicable.

Lock-Up Agreements

We, each of our directors and executive officers, and the holders of all of our outstanding shares of common stock prior to this offering, have agreed that, without the prior written consent of Barclays Capital Inc., UBS Securities LLC and BMO Capital Markets Corp. on behalf of the underwriters, we and they will not, subject to limited exceptions, during the period ending 180 days after the date of this prospectus, subject to extension in specified circumstances:

- offer, pledge, sell or contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, lend, or otherwise transfer or dispose of, directly or indirectly, any shares of common stock or any securities convertible into or exercisable or exchangeable for common stock;
- enter into any swap or other arrangement that transfers to another, in whole or in part, any of the economic consequences of ownership of our common stock or any securities convertible into or exchangeable or exercisable for shares of our common stock, whether such transaction is to be settled by delivery of shares of our common stock or such other securities, in cash or otherwise;
- make any demand for or exercise any right with respect to the registration of any shares of our common stock or any securities convertible into or exchangeable or exercisable for shares of our common stock; or
- publicly announce an intention to do any of the foregoing.

The lock-up restrictions, specified exceptions and the circumstances under which the lock-up period may be extended are described in more detail under the caption "Underwriting."

Participants in the directed share program who purchase $100,000 or more of shares of our common stock will be subject to a 30-day lock-up with respect to any shares sold to them pursuant to that program. This lock-up will have similar restrictions to the lock-up agreements described in this section. Any shares sold in the directed share program to our directors or executive officers will be subject to the lock-up agreements described in this section.

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MATERIAL U.S. FEDERAL INCOME TAX CONSEQUENCES TO NON-U.S. HOLDERS OF OUR COMMON STOCK

The following is a summary of the material U.S. federal income tax consequences to non-U.S. holders (as defined below) of the ownership and disposition of our common stock but does not purport to be a complete analysis of all the potential tax considerations relating thereto. This summary is based upon the provisions of the Internal Revenue Code of 1986, as amended, or the Internal Revenue Code, Treasury regulations promulgated thereunder, administrative rulings and judicial decisions, all as of the date hereof. These authorities may be changed, possibly retroactively, so as to result in U.S. federal income tax consequences different from those set forth below. No ruling on the U.S. federal, state, or local tax considerations relevant to our operations or to the purchase, ownership or disposition of our shares, has been requested from the IRS or other tax authority. No assurance can be given that the IRS would not assert, or that a court would not sustain, a position contrary to any of the tax consequences described below.

This summary also does not address the tax considerations arising under the laws of any non-U.S., state or local jurisdiction, or under U.S. federal gift and estate tax laws, except to the limited extent set forth below. In addition, this discussion does not address tax considerations applicable to an investor's particular circumstances or to investors that may be subject to special tax rules, including, without limitation:

- banks, insurance companies or other financial institutions, regulated investment companies or real estate investment trusts;
- persons subject to the alternative minimum tax or Medicare contribution tax on net investment income;
- tax-exempt organizations or governmental organizations;
- controlled foreign corporations, passive foreign investment companies and corporations that accumulate earnings to avoid U.S. federal income tax;
- brokers or dealers in securities or currencies;
- traders in securities that elect to use a mark-to-market method of accounting for their securities holdings;
- persons that own, or are deemed to own, more than five percent of our capital stock (except to the extent specifically set forth below);
- U.S. expatriates and certain former citizens or long-term residents of the United States;
- partnerships or entities classified as partnerships for U.S. federal income tax purposes or other pass-through entities (and investors therein);
- persons who hold our common stock as a position in a hedging transaction, "straddle," "conversion transaction" or other risk reduction transaction or integrated investment;
- persons who hold or receive our common stock pursuant to the exercise of any employee stock option or otherwise as compensation;
- persons who do not hold our common stock as a capital asset within the meaning of Section 1221 of the Internal Revenue Code; or
- persons deemed to sell our common stock under the constructive sale provisions of the Internal Revenue Code.

You are urged to consult your tax advisor with respect to the application of the U.S. federal income tax laws to your particular situation, as well as any tax consequences of the purchase,

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ownership and disposition of our common stock arising under the U.S. federal estate or gift tax rules or under the laws of any state, local, non-U.S., or other taxing jurisdiction or under any applicable tax treaty.

Non-U.S. Holder Defined

For purposes of this discussion, you are a non-U.S. holder (other than a partnership) if you are any holder other than:

- an individual citizen or resident of the United States (for U.S. federal income tax purposes);
- a corporation or other entity taxable as a corporation created or organized in the United States or under the laws of the United States, any state thereof, or the District of Columbia, or other entity treated as such for U.S. federal income tax purposes;
- an estate whose income is subject to U.S. federal income tax regardless of its source; or
- a trust (x) whose administration is subject to the primary supervision of a U.S. court and which has one or more "U.S. persons" (within the meaning of Section 7701(a)(30) of the Internal Revenue Code) who have the authority to control all substantial decisions of the trust or (y) which has made a valid election to be treated as a U.S. person.

In addition, if a partnership or entity classified as a partnership for U.S. federal income tax purposes holds our common stock, the tax treatment of a partner generally will depend on the status of the partner and upon the activities of the partnership. Accordingly, partnerships that hold our common stock, and partners in such partnerships, should consult their tax advisors.

Distributions

As described in "Dividend Policy," we have never declared or paid cash dividends on our common stock and do not anticipate paying any dividends on our common stock in the foreseeable future. However, if we do make distributions on our common stock, those payments will constitute dividends for U.S. tax purposes to the extent paid from our current or accumulated earnings and profits, as determined under U.S. federal income tax principles. To the extent those distributions exceed both our current and our accumulated earnings and profits, they will constitute a return of capital and will first reduce your basis in our common stock, but not below zero, and then will be treated as gain from the sale of stock as described below under "—Gain on Disposition of Common Stock."

Subject to the discussion below on effectively connected income, backup withholding and foreign accounts, any dividend paid to you generally will be subject to U.S. withholding tax either at a rate of 30% of the gross amount of the dividend or such lower rate as may be specified by an applicable income tax treaty. In order to receive a reduced treaty rate, you must provide us with an IRS Form W-8BEN, IRS Form W-8BEN-E or other appropriate version of IRS Form W-8 certifying qualification for the reduced rate. A non-U.S. holder of shares of our common stock eligible for a reduced rate of U.S. withholding tax pursuant to an income tax treaty may obtain a refund of any excess amounts withheld by timely filing an appropriate claim for refund with the IRS. If the non-U.S. holder holds the stock through a financial institution or other agent acting on the non-U.S. holder's behalf, the non-U.S. holder will be required to provide appropriate documentation to the agent, which then will be required to provide certification to us or our paying agent, either directly or through other intermediaries.

Dividends received by you that are effectively connected with your conduct of a U.S. trade or business (and, if required by an applicable income tax treaty, attributable to a permanent establishment maintained by you in the United States) are generally exempt from such withholding tax. In order to obtain this exemption, you must provide us with an IRS Form W-8ECI or other applicable IRS

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Form W-8 properly certifying such exemption. Such effectively connected dividends, although not subject to withholding tax, are taxed at the same graduated rates applicable to U.S. persons, net of certain deductions and credits. In addition, if you are a corporate non-U.S. holder, dividends you receive that are effectively connected with your conduct of a U.S. trade or business may also be subject to a branch profits tax at a rate of 30% or such lower rate as may be specified by an applicable income tax treaty. You should consult your tax advisor regarding any applicable tax treaties that may provide for different rules.

Gain on Disposition of Common Stock

Subject to the discussion below regarding backup withholding and foreign accounts, you generally will not be required to pay U.S. federal income tax on any gain realized upon the sale or other disposition of our common stock unless:

- the gain is effectively connected with your conduct of a U.S. trade or business (and, if required by an applicable income tax treaty, the gain is attributable to a permanent establishment maintained by you in the United States);
- you are a non-resident alien individual who is present in the United States for a period or periods aggregating 183 days or more during the taxable year in which the sale or disposition occurs and certain other conditions are met; or
- our common stock constitutes a United States real property interest by reason of our status as a "United States real property holding corporation," or USRPHC, for U.S. federal income tax purposes at any time within the shorter of (i) the five-year period preceding your disposition of our common stock, or (ii) your holding period for our common stock.

We believe that we are not currently and will not become a USRPHC for U.S. federal income tax purposes, and the remainder of this discussion so assumes. However, because the determination of whether we are a USRPHC depends on the fair market value of our U.S. real property relative to the fair market value of our other business assets, there can be no assurance that we will not become a USRPHC in the future. Even if we become a USRPHC, however, as long as our common stock is regularly traded on an established securities market, such common stock will be treated as U.S. real property interests only if you actually or constructively hold more than five percent of such regularly traded common stock at any time during the shorter of the five-year period preceding your disposition of, or your holding period for, our common stock.

If you are a non-U.S. holder described in the first bullet above, you will be required to pay tax on the net gain derived from the sale under regular graduated U.S. federal income tax rates, and a corporate non-U.S. holder described in the first bullet above also may be subject to the branch profits tax at a 30% rate, or such lower rate as may be specified by an applicable income tax treaty. If you are an individual non-U.S. holder described in the second bullet above, you will be required to pay a flat 30% tax (or such lower rate specified by an applicable income tax treaty) on the gain derived from the sale, which gain may be offset by U.S. source capital losses for the year (provided you have timely filed U.S. federal income tax returns with respect to such losses). You should consult any applicable income tax or other treaties that may provide for different rules.

Federal Estate Tax

Our common stock beneficially owned by an individual who is not a citizen or resident of the United States (as defined for U.S. federal estate tax purposes) at the time of their death will generally be includable in the decedent's gross estate for U.S. federal estate tax purposes, unless an applicable estate tax treaty provides otherwise. The test for whether an individual is a resident of the United States for U.S. federal estate tax purposes differs from the test used for U.S. federal income tax

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purposes. Some individuals, therefore, may be non-U.S. holders for U.S. federal income tax purposes, but not for U.S. federal estate tax purposes, and vice versa.

Backup Withholding and Information Reporting

Generally, we must report annually to the IRS the amount of dividends paid to you, your name and address and the amount of tax withheld, if any. A similar report will be sent to you. Pursuant to applicable income tax treaties or other agreements, the IRS may make these reports available to tax authorities in your country of residence.

Payments of dividends or of proceeds on the disposition of stock made to you may be subject to information reporting and backup withholding at a current rate of 28% unless you establish an exemption, for example, by properly certifying your non-U.S. status on an IRS Form W-8BEN, IRS Form W-8BEN-E or another appropriate version of IRS Form W-8.

Backup withholding is not an additional tax; rather, the U.S. federal income tax liability of persons subject to backup withholding will be reduced by the amount of tax withheld. If withholding results in an overpayment of taxes, a refund or credit may generally be obtained from the IRS, provided that the required information is furnished to the IRS in a timely manner.

Foreign Account Tax Compliance

The Foreign Account Tax Compliance Act, or FATCA, imposes withholding tax at a rate of 30% on dividends on and gross proceeds from the sale or other disposition of our common stock paid to "foreign financial institutions" (as specially defined under these rules), unless such institution enters into an agreement with the U.S. government to withhold on certain payments and to collect and provide to the U.S. tax authorities substantial information regarding the U.S. account holders of such institution (which includes certain equity and debt holders of such institution, as well as certain account holders that are foreign entities with U.S. owners) or otherwise establishes an exemption. FATCA also generally imposes a U.S. federal withholding tax of 30% on dividends on and gross proceeds from the sale or other disposition of our common stock paid to a "non-financial foreign entity" (as specially defined for purposes of these rules) unless such entity provides the withholding agent with a certification identifying certain substantial direct and indirect U.S. owners of the entity, certifies that there are none or otherwise establishes an exemption. The withholding provisions under FATCA generally apply to dividends on our common stock, and under current transition rules, are expected to apply with respect to the gross proceeds from the sale or other disposition of our common stock on or after January 1, 2019. An intergovernmental agreement between the United States and an applicable foreign country may modify the requirements described in this paragraph. Non-U.S. holders should consult their tax advisors regarding the possible implications of this legislation on their investment in our common stock.

Each prospective investor should consult its tax advisor regarding the particular U.S. federal, state and local and non-U.S. tax consequences of purchasing, holding and disposing of our common stock, including the consequences of any proposed change in applicable laws.

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UNDERWRITING

Barclays Capital Inc., UBS Securities LLC and BMO Capital Markets Corp. are acting as the representatives of the underwriters and the book-running managers of this offering. Under the terms of an underwriting agreement, which is filed as an exhibit to the registration statement, each of the underwriters named below has severally agreed to purchase from us the respective number of shares of common stock shown opposite its name below:

Underwriters	Number of Shares
Barclays Capital Inc.	1,909,092
UBS Securities LLC	1,636,363
BMO Capital Markets Corp.	1,363,636
Canaccord Genuity Inc.	545,454
Total	5,454,545

The underwriting agreement provides that the underwriters' obligation to purchase shares of common stock depends on the satisfaction of the conditions contained in the underwriting agreement including:

- the representations and warranties made by us to the underwriters are true;
- there is no material change in our business or the financial markets; and
- we deliver customary closing documents to the underwriters.

Commissions and Expenses

The following table summarizes the underwriting discounts and commissions we will pay to the underwriters. These amounts are shown assuming both no exercise and full exercise of the underwriters' option to purchase additional shares. The underwriting fee is the difference between the initial price to the public and the amount the underwriters pay to us for the shares.

	No Exercise	Full Exercise
Per Share	$ 0.77	$ 0.77
Total	$ 4,199,999.65	$ 4,829,999.00

The representatives have advised us that the underwriters propose to offer the shares of common stock directly to the public at the public offering price on the cover of this prospectus and to selected dealers, which may include the underwriters, at such offering price less a selling concession not in excess of $0.46200 per share. After the offering, the representatives may change the offering price and other selling terms.

Pursuant to an financial advisory services agreement, we have retained H.C. Wainwright & Co., LLC ("Wainwright"), a FINRA member, to act as our financial advisor in connection with this offering. We have agreed to pay Wainwright, upon the completion of this offering, a fee of $150,000. The services provided by Wainwright included customary business and financial analysis. Wainwright is not acting as an underwriter and will not sell or offer to sell any securities and will not identify, solicit or engage directly with any public or institutional investors. In addition, Wainwright will not underwrite or purchase any of our shares of common stock or warrants to purchase shares of common stock in this offering or otherwise participate in any such undertaking.

The expenses of this offering that are payable by us are estimated to be approximately $1,600,000 (excluding estimated underwriting discounts and commissions). We have also agreed to reimburse the underwriters for certain of their expenses, in an amount up to $250,000, incurred in connection with this offering.

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Option to Purchase Additional Shares

We have granted the underwriters an option exercisable for 30 days after the date of this prospectus, to purchase, from time to time, in whole or in part, up to an aggregate of 818,181 shares from us at the public offering price less underwriting discounts and commissions. To the extent that this option is exercised, each underwriter will be obligated, subject to certain conditions, to purchase its pro rata portion of these additional shares based on the underwriter's percentage underwriting commitment in this offering as indicated in the table at the beginning of this Underwriting Section.

Directed Share Program

At our request, the underwriters have reserved up to 5% of the common stock being offered by this prospectus for sale at the initial public offering price to our directors, officers, employees and other individuals associated with us and members of their families. The sales will be made by UBS Financial Services Inc., a selected dealer affiliated with UBS Securities LLC, an underwriter of this offering, through a directed share program. We do not know if these persons will choose to purchase all or any portion of these reserved shares, but any purchases they do make will reduce the number of shares available to the general public. Any reserved shares not so purchased will be offered by the underwriters to the general public on the same terms as the other shares of common stock. Participants in the directed share program who purchase $100,000 or more of shares of our common stock will be subject to a 30-day lock-up with respect to any shares sold to them pursuant to the program. This lockup will have similar restrictions to the lock-up agreements described below. Any shares sold in the directed share program to our directors or executive officers will be subject to the lock-up agreements described in "—Lock-Up Agreements" below.

Lock-Up Agreements

We, all of our directors, executive officers, and holders of all of our outstanding stock have agreed that, for a period of 180 days after the date of this prospectus subject to certain limited exceptions, we and they will not directly or indirectly, without the prior written consent of each of Barclays Capital Inc., UBS Securities LLC and BMO Capital Markets Corp., (i) offer for sale, sell, pledge, or otherwise dispose of (or enter into any transaction or device that is designed to, or could be expected to, result in the disposition by any person at any time in the future of) any shares of common stock (including, without limitation, shares of common stock that may be deemed to be beneficially owned by us or them in accordance with the rules and regulations of the SEC and shares of common stock that may be issued upon exercise of any options or warrants) or securities convertible into or exercisable or exchangeable for common stock, (ii) enter into any swap or other derivatives transaction that transfers to another, in whole or in part, any of the economic benefits or risks of ownership of shares of common stock, whether any such transaction described in clause (i) or (ii) above is to be settled by delivery of common stock or other securities, in cash or otherwise, (iii) make any demand for or exercise any right or file or cause to be filed a registration statement, including any amendments thereto, with respect to the registration of any shares of common stock or securities convertible into or exercisable or exchangeable for common stock or any of our other securities, or (iv) publicly disclose the intention to do any of the foregoing.

Barclays Capital Inc., UBS Securities LLC and BMO Capital Markets Corp., in their sole discretion, may release the common stock and other securities subject to the lock-up agreements described above in whole or in part at any time. When determining whether or not to release common stock and other securities from lock-up agreements, Barclays Capital Inc., UBS Securities LLC and BMO Capital Markets Corp. will consider, among other factors, the holder's reasons for requesting the release, the number of shares of common stock and other securities for which the release is being requested and market conditions at the time.

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Offering Price Determination

Prior to this offering, there has been no public market for our common stock. The initial public offering price was negotiated between the representatives and us. In determining the initial public offering price of our common stock, the representatives considered:

- the history and prospects for the industry in which we compete;
- our financial information;
- the ability of our management and our business potential and earning prospects;
- the prevailing securities markets at the time of this offering; and
- the recent market prices of, and the demand for, publicly traded shares of generally comparable companies.

Indemnification

We have agreed to indemnify the underwriters against certain liabilities, including liabilities under the Securities Act, and to contribute to payments that the underwriters may be required to make for these liabilities.

Stabilization, Short Positions and Penalty Bids

The representatives may engage in stabilizing transactions, short sales and purchases to cover positions created by short sales, and penalty bids or purchases for the purpose of pegging, fixing or maintaining the price of the common stock, in accordance with Regulation M under the Exchange Act:

- Stabilizing transactions permit bids to purchase the underlying security so long as the stabilizing bids do not exceed a specified maximum.
- A short position involves a sale by the underwriters of shares in excess of the number of shares the underwriters are obligated to purchase in the offering, which creates the syndicate short position. This short position may be either a covered short position or a naked short position. In a covered short position, the number of shares involved in the sales made by the underwriters in excess of the number of shares they are obligated to purchase is not greater than the number of shares that they may purchase by exercising their option to purchase additional shares. In a naked short position, the number of shares involved is greater than the number of shares in their option to purchase additional shares. The underwriters may close out any short position by either exercising their option to purchase additional shares and/or purchasing shares in the open market. In determining the source of shares to close out the short position, the underwriters will consider, among other things, the price of shares available for purchase in the open market as compared to the price at which they may purchase shares through their option to purchase additional shares. A naked short position is more likely to be created if the underwriters are concerned that there could be downward pressure on the price of the shares in the open market after pricing that could adversely affect investors who purchase in the offering.
- Syndicate covering transactions involve purchases of the common stock in the open market after the distribution has been completed in order to cover syndicate short positions.
- Penalty bids permit the representatives to reclaim a selling concession from a syndicate member when the common stock originally sold by the syndicate member is purchased in a stabilizing or syndicate covering transaction to cover syndicate short positions.

These stabilizing transactions, syndicate covering transactions and penalty bids may have the effect of raising or maintaining the market price of our common stock or preventing or retarding a decline in the market price of the common stock. As a result, the price of the common stock may be higher than

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the price that might otherwise exist in the open market. These transactions may be effected on The Nasdaq Capital Market or otherwise and, if commenced, may be discontinued at any time.

Neither we nor any of the underwriters make any representation or prediction as to the direction or magnitude of any effect that the transactions described above may have on the price of the common stock. In addition, neither we nor any of the underwriters make any representation that the representatives will engage in these stabilizing transactions or that any transaction, once commenced, will not be discontinued without notice.

Electronic Distribution

A prospectus in electronic format may be made available on the Internet sites or through other online services maintained by one or more of the underwriters and/or selling group members participating in this offering, or by their affiliates. In those cases, prospective investors may view offering terms online and, depending upon the particular underwriter or selling group member, prospective investors may be allowed to place orders online. The underwriters may agree with us to allocate a specific number of shares for sale to online brokerage account holders. Any such allocation for online distributions will be made by the representatives on the same basis as other allocations.

Other than the prospectus in electronic format, the information on any underwriter's or selling group member's web site and any information contained in any other web site maintained by an underwriter or selling group member is not part of the prospectus or the registration statement of which this prospectus forms a part, has not been approved and/or endorsed by us or any underwriter or selling group member in its capacity as underwriter or selling group member and should not be relied upon by investors.

Listing on The Nasdaq Capital Market

We have been approved, subject to notice of issuance, to list our common stock on The Nasdaq Capital Market under the symbol "BTAI".

Discretionary Sales

The underwriters have informed us that they do not expect to sell more than 5% of the common stock in the aggregate to accounts over which they exercise discretionary authority.

Stamp Taxes

If you purchase shares of common stock offered in this prospectus, you may be required to pay stamp taxes and other charges under the laws and practices of the country of purchase, in addition to the offering price listed on the cover page of this prospectus.

Other Relationships

The underwriters and certain of their affiliates are full service financial institutions engaged in various activities, which may include securities trading, commercial and investment banking, financial advisory, investment management, investment research, principal investment, hedging, financing and brokerage activities. The underwriters and certain of their affiliates have, from time to time, performed, and may in the future perform, various commercial and investment banking and financial advisory services for the issuer and its affiliates, for which they received or may in the future receive customary fees and expenses.

In the ordinary course of their various business activities, the underwriters and certain of their affiliates may make or hold a broad array of investments and actively trade debt and equity securities (or related derivative securities) and financial instruments (including bank loans) for their own account and for the accounts of their customers, and such investment and securities activities may involve

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securities and/or instruments of the issuer or its affiliates. If the underwriters or their affiliates have a lending relationship with us, they routinely hedge their credit exposure to us consistent with their customary risk management policies. The underwriters and their affiliates may hedge such exposure by entering into transactions which consist of either the purchase of credit default swaps or the creation of short positions in our securities or the securities of our affiliates, including potentially the shares of common stock offered hereby. Any such credit default swaps or short positions could adversely affect future trading prices of the shares of common stock offered hereby. The underwriters and certain of their affiliates may also communicate independent investment recommendations, market color or trading ideas and/or publish or express independent research views in respect of such securities or instruments and may at any time hold, or recommend to clients that they acquire, long and/or short positions in such securities and instruments.

Selling Restrictions

This prospectus does not constitute an offer to sell to, or a solicitation of an offer to buy from, anyone in any country or jurisdiction (i) in which such an offer or solicitation is not authorized, (ii) in which any person making such offer or solicitation is not qualified to do so or (iii) in which any such offer or solicitation would otherwise be unlawful. No action has been taken that would, or is intended to, permit a public offer of the shares of common stock or possession or distribution of this prospectus or any other offering or publicity material relating to the shares of common stock in any country or jurisdiction (other than the United States) where any such action for that purpose is required. Accordingly, each underwriter has undertaken that it will not, directly or indirectly, offer or sell any shares of common stock or have in its possession, distribute or publish any prospectus, form of application, advertisement or other document or information in any country or jurisdiction except under circumstances that will, to the best of its knowledge and belief, result in compliance with any applicable laws and regulations and all offers and sales of shares of common stock by it will be made on the same terms.

European Economic Area

In relation to each Member State of the European Economic Area which has implemented the Prospectus Directive (each, a "Relevant Member State") an offer to the public of any common stock which are the subject of the offering contemplated herein may not be made in that Relevant Member State, except that an offer to the public in that Relevant Member State of any common stock may be made at any time under the following exemptions under the Prospectus Directive, if they have been implemented in that Relevant Member State:

- to legal entities which are qualified investors as defined under the Prospectus Directive;
- by the underwriters to fewer than 100, or, if the Relevant Member State has implemented the relevant provisions of the 2010 PD Amending Directive, 150, natural or legal persons (other than qualified investors as defined in the Prospectus Directive), as permitted under the Prospectus Directive, subject to obtaining the prior consent of the representatives of the underwriters for any such offer; or
- in any other circumstances falling within Article 3(2) of the Prospectus Directive,

provided that no such offer of common stock shall result in a requirement for us or any underwriter to publish a prospectus pursuant to Article 3 of the Prospectus Directive or supplement a prospectus pursuant to Article 16 of the Prospectus Directive.

Each person in a Relevant Member State who receives any communication in respect of, or who acquires any common stock under, the offers contemplated here in this prospectus will be deemed to have represented, warranted and agreed to and with each underwriter and us that:

- it is a qualified investor as defined under the Prospectus Directive; and

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- in the case of any common stock acquired by it as a financial intermediary, as that term is used in Article 3(2) of the Prospectus Directive, (i) the common stock acquired by it in the offering have not been acquired on behalf of, nor have they been acquired with a view to their offer or resale to, persons in any Relevant Member State other than qualified investors, as that term is defined in the Prospectus Directive, or in the circumstances in which the prior consent of the representatives of the underwriters has been given to the offer or resale or (ii) where common stock have been acquired by it on behalf of persons in any Relevant Member State other than qualified investors, the offer of such common stock to it is not treated under the Prospectus Directive as having been made to such persons.

For the purposes of this representation and the provision above, the expression an "offer of common stock to the public" in relation to any common stock in any Relevant Member State means the communication in any form and by any means of sufficient information on the terms of the offer and any common stock to be offered so as to enable an investor to decide to purchase or subscribe for the common stock, as the same may be varied in that Relevant Member State by any measure implementing the Prospectus Directive in that Relevant Member State, the expression "Prospectus Directive" means Directive 2003/71/EC (and amendments thereto, including the 2010 PD Amending Directive, to the extent implemented in the Relevant Member State), and includes any relevant implementing measure in each Relevant Member State and the expression "2010 PD Amending Directive" means Directive 2010/73/EU.

United Kingdom

This prospectus has only been communicated or caused to have been communicated and will only be communicated or caused to be communicated as an invitation or inducement to engage in investment activity (within the meaning of Section 21 of the Financial Services and Markets Act of 2000 (the "FSMA")) as received in connection with the issue or sale of the common stock in circumstances in which Section 21(1) of the FSMA does not apply to us. All applicable provisions of the FSMA will be complied with in respect to anything done in relation to the common stock in, from or otherwise involving the United Kingdom.

Notice to Residents of Canada

The securities may be sold only to purchasers purchasing, or deemed to be purchasing, as principal that are accredited investors, as defined in National Instrument 45-106 Prospectus Exemptions or subsection 73.3(1) of the Securities Act (Ontario), and are permitted clients, as defined in National Instrument 31-103 Registration Requirements, Exemptions and Ongoing Registrant Obligations. Any resale of the securities must be made in accordance with an exemption from, or in a transaction not subject to, the prospectus requirements of applicable securities laws.

Securities legislation in certain provinces or territories of Canada may provide a purchaser with remedies for rescission or damages if this prospectus (including any amendment thereto) contains a misrepresentation, provided that the remedies for rescission or damages are exercised by the purchaser within the time limit prescribed by the securities legislation of the purchaser's province or territory. The purchaser should refer to any applicable provisions of the securities legislation of the purchaser's province or territory for particulars of these rights or consult with a legal advisor.

Pursuant to section 3A.3 (or, in the case of securities issued or guaranteed by the government of a non-Canadian jurisdiction, section 3A.4) of National Instrument 33-105 Underwriting Conflicts (NI 33-105), the underwriters are not required to comply with the disclosure requirements of NI 33-105 regarding underwriter conflicts of interest in connection with this offering.

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LEGAL MATTERS

The validity of the issuance of the common stock offered by us in this offering will be passed upon for us by Sheppard, Mullin, Richter & Hampton LLP, New York, New York. Certain legal matters in connection with this offering will be passed upon for the underwriters by Latham & Watkins LLP.

EXPERTS

The financial statements of BioXcel Therapeutics, Inc. as of December 31, 2017 and 2016 and for each of the years then ended included in this Registration Statement, of which this Prospectus forms a part, have been so included in reliance on the report of BDO USA, LLP, an independent registered public accounting firm (the report on the financial statements contains an explanatory paragraph regarding the Company's ability to continue as a going concern) appearing elsewhere herein, given on the authority of said firm as experts in auditing and accounting. The balance sheet of BioXcel Therapeutics, Inc. as of December 31, 2016, and the related statements of operations, changes in net Parent investment, and cash flows for the period January 1, 2017 through June 30, 2017 are the carved-out operations of certain assets and liabilities of BioXcel Corporation.

WHERE YOU CAN FIND MORE INFORMATION

We have filed with the Securities and Exchange Commission a registration statement on Form S-1 under the Securities Act with respect to the common stock offered by this prospectus. This prospectus, which is part of the registration statement, omits certain information, exhibits, schedules and undertakings set forth in the registration statement. For further information pertaining to us and our common stock, reference is made to the registration statement and the exhibits and schedules to the registration statement. Statements contained in this prospectus as to the contents or provisions of any documents referred to in this prospectus are not necessarily complete, and in each instance where a copy of the document has been filed as an exhibit to the registration statement, reference is made to the exhibit for a more complete description of the matters involved.

You may read and copy all or any portion of the registration statement without charge at the public reference room of the Securities and Exchange Commission at 100 F Street, N.E., Washington, D.C. 20549. Copies of the registration statement may be obtained from the Securities and Exchange Commission at prescribed rates from the public reference room of the Securities and Exchange Commission at such address. You may obtain information regarding the operation of the public reference room by calling 1-800-SEC-0330. In addition, registration statements and certain other filings made with the Securities and Exchange Commission electronically are publicly available through the Securities and Exchange Commission's website at *http://www.sec.gov*. The registration statement, including all exhibits and amendments to the registration statement, has been filed electronically with the Securities and Exchange Commission.

Upon completion of this offering, we will become subject to the information and periodic reporting requirements of the Securities Exchange Act of 1934, as amended, and, accordingly, will be required to file annual reports containing financial statements audited by an independent public accounting firm, quarterly reports containing unaudited financial data, current reports, proxy statements and other information with the Securities and Exchange Commission. You will be able to inspect and copy such periodic reports, proxy statements and other information at the Securities and Exchange Commission's public reference room, and the website of the Securities and Exchange Commission referred to above.

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FINANCIAL STATEMENTS

BioXcel Therapeutics, Inc.

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REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

The Board of Directors and Shareholders
of BioXcel Therapeutics, Inc.

Opinion on the Financial Statements

We have audited the accompanying balance sheets of BioXcel Therapeutics, Inc. (the "Company") as of December 31, 2017 and 2016, and the related statements of operations, changes in stockholder's deficit/net Parent investment, and cash flows for the years then ended, and the related notes (collectively referred to as the "financial statements"). The balance sheet as of December 31, 2016, and the related statements of operations, changes in net Parent investment, and cash flows for the period January 1, 2017 through June 30, 2017 are the carved-out operations of certain assets and liabilities of BioXcel Corporation. In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2017 and 2016, and the results of its operations and its cash flows for the years then ended, in conformity with accounting principles generally accepted in the United States of America.

Going Concern

The accompanying financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note 2 to the financial statements, the Company has incurred significant operating losses and negative cash flows from operations. The Company also had a working capital deficiency of $1.45 million and an accumulated deficit of $4.45 million at December 31, 2017. The Company is dependent on obtaining necessary funding in order to continue its operations. These conditions raise substantial doubt about its ability to continue as a going concern. Management's plans regarding those matters also are described in Note 2.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) ("PCAOB") and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ BDO USA LLP

We have served as the Company's auditor since 2017
Woodbridge, New Jersey
February 12, 2018, except for Note 12 c, which is March 5, 2018 and Note 12 d, which is March 8, 2018

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BIOXCEL THERAPEUTICS, INC.

BALANCE SHEETS

(amount in thousands, except shares and per share data)

	December 31, 2017 (A)	December 31, 2016 (A)
ASSETS		
Current assets		
Cash	$ 887	$ —
Prepaid expenses and other assets	3	2
Total current assets	890	2
Deferred offering expenses	461	—
Equipment, net	4	5
Total assets	**$ 1,355**	**$ 7**
LIABILITIES AND STOCKHOLDERS' DEFICIT / NET PARENT INVESTMENT		
Current liabilities		
Accounts payable	444	279
Accrued expenses	1,015	52
Payable to Parent for services	67	—
Notes payable to Parent	371	—
Due to Parent	440	—
Total current liabilities	2,337	331
Total liabilities	2,337	331
Commitments and contingencies	—	—
Stockholders' deficit / Net Parent investment		
Preferred stock, $0.001 par value, 10,000,000 shares authorized; no shares issued or outstanding	—	—
Common stock, $0.001 par value, 50,000,000 shares authorized; 9,907,548 shares issued and outstanding as of December 31, 2017 (see Note 12d)	10	—
Additional paid-in-capital	3,458	—
Net Parent investment		
Net liabilities assumed from Parent	—	(324)
Total net Parent investment	—	(324)
Accumulated deficit	(4,450)	—
Total stockholders deficit and net Parent investment	(982)	(324)
Total liabilities and stockholders' deficit / net Parent investment	**$ 1,355**	**$ 7**

(A) See Note 2 to the financial statements

The accompanying notes are an integral part of these financial statements

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BIOXCEL THERAPEUTICS, INC.

STATEMENTS OF OPERATIONS

(amount in thousands, except share and per share data)

	Years Ended	
	2017 (A)	2016 (A)
Revenues	$ —	$ —
Operating costs and expenses		
Research and development	2,690	1,399
General and administrative	1,847	721
Total operating expenses	4,537	2,120
Loss from operations	(4,537)	(2,120)
Other expense		
Interest expense	(2)	—
Net loss	$ (4,539)	$ (2,120)
Net loss per share attributable to common stockholder/Parent, basic and diluted	$ (.47)	$ (.22)
Weighted average common shares outstanding, basic and diluted	9,685,005	9,480,000

(A) See Note 2 to the financial statements

The accompanying notes are an integral part of these financial statements.

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BIOXCEL THERAPEUTICS, INC.

STATEMENTS OF CHANGES IN STOCKHOLDERS' DEFICIT/NET PARENT INVESTMENT

(amount in thousands, except shares and per share data)

	Common Stock		Net Parent Investment	Additional Paid in Capital	Accumulated Deficit	Total
	Shares	Amount				
Balance as of January 1, 2016	—	$ —	$ (173)	$ —	$ —	$ (173)
Investment from Parent			1,969			1,969
Net loss			(2,120)			(2,120)
Balance as of December 31, 2016	**—**	**—**	**(324)**	**—**	**—**	**(324)**
Investment from Parent			539			539
Net loss[(A)]			(529)			(529)
Balance as of March 29, 2017 (date of incorporation)	**—**	**—**	(314)	**—**	**—**	(314)
Issuance of common shares (see Note 12d)	9,907,548	10		2,051		2,061
Liabilities assumed from Parent			(126)			(126)
Transfer to accumulated deficit			440		(440)	—
Stock-based compensation				1,407		1,407
Net loss[(A)]					(4,010)	(4,010)
Balance as of December 31, 2017	**9,907,548**	**$ 10**	**$ —**	**$ 3,458**	**$ (4,450)**	**$ (982)**

(A) Combined net loss for the period ended December 31, 2017 is $ 4,539

The accompanying notes are an integral part of these financial statements.

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BIOXCEL THERAPEUTICS, INC.

STATEMENTS OF CASH FLOWS

(amount in thousands, except shares and per share data)

	Years Ended December 31,	
	2017 (A)	2016 (A)
CASH FLOWS FROM OPERATING ACTIVITIES:		
Net loss	**$ (4,539)**	**$ (2,120)**
Reconciliation of net loss to net cash (used in) provided by operating activities:		
Depreciation and amortization	1	—
Stock-based compensation expense	1,606	671
Changes in operating assets and liabilities:		
Prepaid expenses	(1)	(1)
Accounts payable and accrued expenses	737	156
Net cash used in operating activities	(2,196)	(1,294)
CASH FLOWS FROM INVESTING ACTIVITIES:		
Purchase of fixed assets	—	(4)
Net cash used in investing activities	—	(4)
CASH FLOWS FROM FINANCING ACTIVITIES:		
Deferred offering expense	(70)	—
Net Parent investment	214	1,298
Payable to Parent for services	67	—
Due to Parent	440	—
Proceeds from note payable—Parent	371	—
Proceeds from issuance of common stock	2,061	—
Net cash provided by financing activities	3,083	1,298
Net increase in cash	887	—
Cash, beginning of the year	—	—
Cash, end of the year	$ 887	$ —
Non-cash financing activities:		
Deferred offering expenses, unpaid as of 12/31/17	$ 391	—
Supplemental disclosure		
Reclassification of net parental investment in the Company to accumulated deficit	$ 440	$ —

(A) See Note 2 to the financial statements

The accompanying notes are an integral part of these financial statements.

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BIOXCEL THERAPEUTICS, INC.

NOTES TO FINANCIAL STATEMENTS

(amounts in thousands, except share and per share data)

Note 1. Organization and Principal Activities

BioXcel Therapeutics, Inc. (the "Company" or "BTI") is a clinical stage biopharmaceutical company focused on novel artificial intelligence-based drug development to identify the next wave of medicines across neuroscience and immuno-oncology. The Company's drug re-innovation approach leverages existing approved drugs and/or clinically validated product candidates together with big data and proprietary machine learning algorithms to identify new therapeutic indices. The Company is a wholly-owned subsidiary of BioXcel Corporation ("BioXcel" or "Parent") and was incorporated under the laws of the State of Delaware on March 29, 2017—see note 2 basis of presentation for further discussion. The Company's principal office is in Branford, Connecticut.

The Company's primary activities have been the development of a clinical plan and pre-clinical research and development of two advanced programs: BXCL501, a sublingual thin film formulation of dexmedetomidine designed for acute treatment of agitation resulting from neurological and psychiatric disorders, and BXCL701, an immuno-oncology agent designed for treatment of a rare form of prostate cancer and for treatment of pancreatic cancer. These two programs and two emerging programs BXCL502 and BXCL702 (together, "the BTI Business") programs have been contributed to the Company from the parent company BioXcel.

Note 2. Basis of Presentation and Liquidity

Basis of Presentation

The financial statements of the Company are derived by carving out the historical results of operations and historical cost basis of the, assets and liabilities associated with product candidates BXCL501, BXCL701, BXCL502 and BXCL702 that have been contributed to the Company by BioXcel (the "BTI Business") from the BioXcel's financial statements.

These results reflect amounts specifically attributable to the BTI Business, which include expenses, assets and liabilities of BioXcel relating to the candidates that were contributed to the Company by BioXcel under a contribution agreement, effective June 30, 2017, as amended and restated on November 7, 2017, or the Contribution Agreement, for the period from January 1, 2015 until the formation of the Company on March 29, 2017 (date of incorporation) and further until June 30, 2017. The Company has entered into a separation and shared services agreement with BioXcel that took effect on June 30, 2017, as amended and restated on November 7, 2017, or the Services Agreement, pursuant to which BioXcel provides the Company with certain general and administrative and development support services effective June 30, 2017. However, consistent with accounting regulations, it has been assumed that the Company was a separate business since January 1, 2015 and accordingly the assets, liabilities and expenses relating to the BTI Business have been separated from the Company in the financial statements for periods prior to and post incorporation through June 30, 2017. The financial statements as of December 31, 2016 and for the period January 1, 2017 through June 30, 2017 include reasonable allocations for assets and liabilities and expenses attributable to the BTI Business.

Accordingly, the historical financial information for the fiscal year ended December 31, 2016 has been "carved-out" of the financial statements of BioXcel, and such financial information is limited to business activities, assets and liabilities of the BTI Business. For the year ended December 31, 2017 results include carve-out amounts from BioXcel for the period January 1, 2017 through June 30, 2017 and as a standalone entity for the period July 1, 2017 through December 31, 2017.

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BIOXCEL THERAPEUTICS, INC.

NOTES TO FINANCIAL STATEMENTS (Continued)

(amounts in thousands, except share and per share data)

Note 2. Basis of Presentation and Liquidity (Continued)

All assets and liabilities contributed by BioXcel to the Company have been recorded at historical book value. The historical financial statements derived during the years ended December 31, 2016 and for the period January 1, 2017 through June 30, 2017 have been presented on a basis that includes the results attributable to the BTI Business as if the Company owned the business for these periods.

The carve-out financial information includes both direct and indirect expenses. The historical direct expenses consist primarily of salaries of research and development employees directly involved in the BTI Business activities, stock based compensation for such employees, preclinical and clinical trial related expenses, research expenses and fees paid to scientific advisors. The indirect expenses consist of allocated employee costs, stock-based compensation, legal, professional and consulting fees attributable to the BTI Business and general and administrative overhead charged back to the BTI Business in proportion to the time spent by employees directly involved in the BTI Business, compared to the total time spent by all the employees.

Prepaid expenses, other current assets, fixed assets, accounts payable, accrued wages and salaries and accrued liabilities are presented using the allocation method whereby assets and liabilities directly related to the BTI Business were allocated at 100% to the Company. For compensation related matters, the allocation was based on time spent by employees directly involved in the BTI Business compared to the total time spent by all employees. All other allocations were based on management estimates.

The Company believes that the assumptions underlying the allocations of direct and indirect expenses in the carve-out financial information are reasonable, however, the financial position, results of operations and cash flows may have been materially different if the Company had operated as a stand-alone entity as of and for the years ended December 31, 2017 and 2016, respectively.

The Company has calculated its income tax amounts using a separate return methodology and it has presented these amounts as if it were a separate taxpayer from BioXcel for the period since the date of incorporation (March 29, 2017). BioXcel is a standalone S corporation and its tax obligations were passed through to its shareholders and were not a liability of the S corporation. As a result, BioXcel does not require a tax provision for federal or state purposes and on the same lines no taxes have been allocated to the financials of the Company which is derived from a carve-out process from the financials of BioXcel. Pursuant to our incorporation as a C corporation, BioXcel became the Company's sole owner and contributed the BTI Business in a tax free transaction. From the date of incorporation, the Company has been a standalone C corporation subject to corporate income tax and the deferred tax assets have been calculated accordingly.

Liquidity and Going Concern

The Company incurred net losses of $(4,539) and $(2,120) during the years ended December 31, 2017 and 2016, respectively. The Company has a working capital deficit of $(1,447) as of December 31, 2017 and $(329) as of December 31, 2016. The Company had a net Parent Investment of $324 as of December 31, 2016, There was no net Parent investment as of December 31, 2017 as all amounts were transferred to accumulated deficit. The Company has not yet developed its own funding sources and is largely dependent on BioXcel for funding. These matters raise substantial doubt about the Company's ability to continue as a going concern. Under the Agreement, BioXcel has agreed to provide a line of

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BIOXCEL THERAPEUTICS, INC.

NOTES TO FINANCIAL STATEMENTS (Continued)

(amounts in thousands, except share and per share data)

Note 2. Basis of Presentation and Liquidity (Continued)

credit to the Company in the amount of up to $1,000 (which can be increased based on a mutual agreement) for working capital purposes.

The Company is obligated to repay BioXcel the amounts drawn down under the Grid Note upon the closing of an initial public offering or 18 months from the date of the note whichever is earlier. As on December 31, 2017 the Company had drawn $371. For the period March 29, 2017 through December 31, 2017 the Parent paid certain expenses on the Company's behalf prior to when the Grid Note was available totaling approximately $562 of which $122 has been repaid as of December 31, 2017. This is to be repaid the earliest to occur of: (x) thirty days after an initial public offering (y) ten (10) days after the Company receives funding of at least $5,000 other than through the IPO; and (z) December 31, 2018.

In addition, the Company needs to raise additional capital from either its Parent or from external sources in order to sustain its operations while continuing the longer-term efforts contemplated under its business plan. The Company expects to continue incurring losses for the foreseeable future and must raise additional capital to pursue its product development initiatives, conduct clinical trials and continue as a going concern. The Company cannot provide any assurance that it will raise additional capital. Management believes that the Company has access to capital resources through possible public or private equity offerings, debt financings, corporate collaborations or other means; however, the Company has not secured any commitment for new financing at this time nor can it provide any assurance that new financing will be available on commercially acceptable terms, if at all. If the Company is unable to secure additional capital, it may be required to curtail its research and development initiatives and clinical trials and take additional measures to reduce costs in order to conserve available cash in amounts sufficient to sustain operations and meet its obligations. These measures could cause significant delays in the Company's research and development, clinical trials and regulatory efforts, which is critical to the realization of its business plan and the future operations of the Company. The Company is currently exploring external financing alternatives which will be needed by the Company to fund its operations. The accompanying financial statements do not include any adjustments that may be necessary should the Company be unable to continue as a going concern.

Note 3. Summary of Significant Accounting Policies

Use of Estimates

The Company's financial statements are prepared in accordance with U.S. GAAP. The preparation of Bioxcel's financial statements requires the Company to make estimates and assumptions that impact the reported amounts of assets, liabilities and expenses in our financial statements and accompanying notes. The most significant estimates in the financial statements relate to the fair value of equity awards, the valuation of the Parent's common stock, allocation of expenses, assets and liabilities from the Parent and valuation allowance related to the Company's deferred tax assets and liabilities. Although these estimates are based on the Company's knowledge of current events and actions it may undertake in the future, actual results may ultimately materially differ from these estimates.

Cash

Cash is in accounts held at leading U.S. financial institutions that are insured by the Federal Deposit Insurance Corporation (FDIC) up to $250. Cash balances could exceed insured amounts at any given

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BIOXCEL THERAPEUTICS, INC.

NOTES TO FINANCIAL STATEMENTS (Continued)

(amounts in thousands, except share and per share data)

Note 3. Summary of Significant Accounting Policies (Continued)

time; however, the Company has not experienced any such losses and believes the risk of loss is minimal.

Equipment

Equipment consist of computers that are stated at cost and depreciated using the straight-line method over estimated useful life of 5 years.

The Company follows the guidance provided by FASB ASC Topic 360-10, *Property, Plant, and Equipment*. Long-lived assets are reviewed for impairment whenever events or changes in circumstances indicate that the carrying amount of an asset may not be recoverable. Recoverability of assets to be held and used is measured by a comparison of the carrying amount of an asset to future net cash flows expected to be generated. Impairment charges are recognized at the amount by which the carrying amount of an asset exceeds the fair value of the asset. Assets to be disposed of are reported at the lower of the carrying amount or the fair value less costs to sell.

Since its inception the Company has not recognized any impairment or disposition of long lived assets.

Stock-Based Compensation

The Company accounts for stock-based compensation in accordance with ASC 718, "*Compensation—Stock Compensation*", which requires the measurement and recognition of compensation expense based on estimated fair market values for all share-based awards made to employees and directors, including stock options. The Company's stock based compensation plan was adopted and became effective in August 2017. Prior to the Company adopting its stock based compensation plan the Parent granted stock options to its employees. As a result related stock-based compensation expense has been allocated to the Company over the required service period over which these BioXcel stock option awards vest in the same manner salary costs of employees have been allocated to the BTI Business in the carve-out process.

Both BioXcel and the Company's stock option awards are valued at fair value on the date of grant and that fair value is recognized over the requisite service period. The estimated fair value of stock option awards was determined using the Black-Scholes option pricing model on the date of grant. Significant judgment and estimates were used to estimate the fair value of these awards, as they are not publicly traded.

ASC 718 requires companies to estimate the fair value of share-based awards on the date of grant using an option-pricing model. Both BioXcel and the Company use the Black-Scholes option-pricing model as its method of determining fair value. This model is affected by both BioXcel and the Company's stock price as well as assumptions regarding a number of subjective variables. These subjective variables include, but are not limited to, both the Company's and BioXcel's expected stock price volatility over the term of the awards, and actual and projected employee stock option exercise behaviors. The value of the portion of the award that is ultimately expected to vest is recognized as an expense in the statement of operations over the requisite service period. The periodic expense is then determined based on the valuation of the options, and at that time an estimated forfeiture rate, if any, is used to reduce the expense recorded. The Parent's estimates of pre-vesting forfeitures is primarily based on the its historical experience and is adjusted to reflect actual forfeitures as the options vest.

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BIOXCEL THERAPEUTICS, INC.

NOTES TO FINANCIAL STATEMENTS (Continued)

(amounts in thousands, except share and per share data)

Note 3. Summary of Significant Accounting Policies (Continued)

We have adopted FASB ASU 2016-09 and account for forfeitures as they occur, by reversing compensation cost when the award is forfeited.

Research and Development Costs

Research and development expenses include wages, benefits, facilities, supplies, external services, clinical study and manufacturing costs and other expenses that are directly related to its research and development activities. At the end of the reporting period, the Company compares payments made to third party service providers to the estimated progress toward completion of the research or development objectives. Such estimates are subject to change as additional information becomes available. Depending on the timing of payments to the service providers and the progress that the Company estimates has been made as a result of the service provided, the Company may record net prepaid or accrued expense relating to these costs. The Company expenses research and development costs as it incurs them.

Patent Costs

Costs related to filing and pursuing patent applications are recorded as general and administrative expense and expensed as incurred since recoverability of such expenditures is uncertain.

Income Taxes

The Company accounts for income taxes under Accounting Standards Codification ("ASC") 740 Income Taxes ("ASC 740"). Under ASC 740, deferred tax assets and liabilities are determined based on the differences between the financial reporting and tax bases of assets and liabilities and net operating loss and credit carryforwards using enacted tax rates in effect for the year in which the differences are expected to impact taxable income. Valuation allowances are established when necessary to reduce deferred tax assets to the amounts expected to be realized.

ASC 740 also clarifies the accounting for uncertainty in income taxes recognized in an enterprise's financial statements and prescribes a recognition threshold and measurement process for financial statement recognition and measurement of a tax position taken or expected to be taken in a tax return.

Tax benefits claimed or expected to be claimed on a tax return are recorded in the Company's financial statements. A tax benefit from an uncertain tax position is only recognized if it is more likely than not that the tax position will be sustained on examination by the taxing authorities, based on the technical merits of the position. The tax benefits recognized in the financial statements from such a position are measured based on the largest benefit that has a greater than fifty percent likelihood of being realized upon ultimate resolution. Uncertain tax positions have had no impact on the Company's financial condition, results of operations or cash flows.

Fair Value Measurements

ASC 820 "*Fair Value Measurements*" defines fair value, establishes a framework for measuring fair value in GAAP and expands disclosures about fair value measurements. ASC 820 defines fair value as the price that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date. ASC 820 establishes a fair value hierarchy that

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BIOXCEL THERAPEUTICS, INC.

NOTES TO FINANCIAL STATEMENTS (Continued)

(amounts in thousands, except share and per share data)

Note 3. Summary of Significant Accounting Policies (Continued)

distinguishes between (1) market participant assumptions developed based on market data obtained from independent sources (observable inputs) and (2) an entity's own assumptions about market participant assumptions developed based on the best information available in the circumstances (unobservable inputs). The fair value hierarchy consists of three broad levels, which gives the highest priority to unadjusted quoted prices in active markets for identical assets or liabilities (Level 1) and the lowest priority to unobservable inputs (Level 3). The three levels of the fair value hierarchy under ASC 820 are described below:

- Level 1—Quoted prices (unadjusted) in active markets that are accessible at the measurement date for assets or liabilities. The fair value hierarchy gives the highest priority to Level 1 inputs.

- Level 2—Directly or indirectly observable inputs as of the reporting date through correlation with market data, including quoted prices for similar assets and liabilities in active markets and quoted prices in markets that are not active. Level 2 also includes assets and liabilities that are valued using models or other pricing methodologies that do not require significant judgment since the input assumptions used in the models, such as interest rates and volatility factors, are corroborated by readily observable data from actively quoted markets for substantially the full term of the financial instrument.

- Level 3—Unobservable inputs that are supported by little or no market activity and reflect the use of significant management judgment. These values are generally determined using pricing models for which the assumptions utilize management's estimates of market participant assumptions.

In determining fair value, the Company utilizes valuation techniques that maximize the use of observable inputs and minimize the use of unobservable inputs to the extent possible as well as considers counterparty credit risk in its assessment of fair value.

The carrying amounts of cash, accounts payable and accrued expenses approximate fair value due to the short-term nature of these instruments.

Net Loss per Share

The Company computes basic net loss per share by dividing net loss per share available to common stockholders by the weighted average number of common shares outstanding for the period and excludes the effects of any potentially dilutive securities. Diluted earnings per share, if presented, would include the dilution that would occur upon the exercise or conversion of all potentially dilutive securities into common stock using the "treasury stock" and/or "if converted" methods as applicable. The Company did not have any potentially diluted securities outstanding in any period presented in the accompanying financial statements. The Company was incorporated on March 29, 2017 and loss per common share was calculated for the years ended December 31, 2017 and 2016 respectively, assuming the shares issued to the Parent at formation were outstanding for all periods presented.

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BIOXCEL THERAPEUTICS, INC.

NOTES TO FINANCIAL STATEMENTS (Continued)

(amounts in thousands, except share and per share data)

Note 3. Summary of Significant Accounting Policies (Continued)

Recent Accounting Pronouncements

In May 2014, the Financial Accounting Standards Board, or FASB, issued *ASU 2014-09 Revenue form Contracts with Customers.* Under this guidance on the recognition of revenue from customers. Under this guidance, an entity will recognize revenue when it transfers promised goods or services to customers in an amount that reflects what the entity expects to receive in exchange for the goods or services. This new guidance also requires more detailed disclosures to enable users of the financial statements to understand the nature, amount, timing and uncertainty of revenue and cash flows arising from contracts with customers. The Company will adopt this guidance beginning on January 1, 2018. The guidance allows the selection of one of two methods of adoption, either the full retrospective approach, meaning the guidance would be applied to all periods presented, or modified retrospective approach, meaning the cumulative effect of applying the guidance would be recognized as an adjustment to opening accumulated deficit balance. Since the Company has no revenues to date, the Company does not believe the adoption of ASU-214-09 will have a material impact on its financial statements.

In August 2014, the FASB issued *ASU 2014-15 Disclosures of Uncertainties around an Entity's Ability to Continue as a Going Concern.* This ASU requires management to determine whether substantial doubt exists regarding the entity's going concern presumption, which generally refers to an entity's ability to meets its obligations as they become due. If substantial doubt exists but is not alleviated by management's plan, the footnotes must specifically state that "there is substantial doubt about the entity's ability to continue as a going concern within one year after the financial statements are issued." In addition, if substantial doubt exists, regardless of whether such doubt was alleviated, entities must disclose (a) principal conditions or events that raise substantial doubt about the entity's ability to continue as a going concern (before consideration of management's plans, if any); (b) management's evaluation of the significance of those conditions or events in relation to the entity's ability to meet its obligations; and (c) management's plans that are intended to mitigate the conditions or events that raise substantial doubt, or that did alleviate substantial doubt, about the entity's ability to continue as a going concern. If substantial doubt has not been alleviated, these disclosures should become more extensive in subsequent reporting periods as additional information becomes available. In the period that substantial doubt no longer exists (before or after considering management's plans), management should disclose how the principal conditions and events that originally gave rise to substantial doubt have been resolved. The Company has adopted the provisions of ASU 2014-15 beginning January 1, 2016.

In February 2016, the FASB issued *ASU 2016-02 Lease Accounting Topic 842*. This ASU requires us to record all leases longer than one year on our balance sheet. Under the new guidance, when the Company records leases on its balance sheet under it will record a liability with a value equal to the present value of payments it will make over the life of the lease and an asset representing the underlying leased asset. The new accounting guidance requires the Company to determine if its leases are operating or financing leases, similar to current accounting guidance. The Company will record expense for operating type leases on a straight-line basis as an operating expense and it will record expense for finance type leases as interest expense. The new lease standard is effective for annual and interim periods beginning after December 15, 2018, with early adoption permitted. The Company must adopt the new standard on a modified retrospective basis, which requires it to reflect its leases on its

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BIOXCEL THERAPEUTICS, INC.

NOTES TO FINANCIAL STATEMENTS (Continued)

(amounts in thousands, except share and per share data)

Note 3. Summary of Significant Accounting Policies (Continued)

balance sheet for the earliest comparative period presented. The Company is currently assessing the timing of adoption as well as the effects it will have on its financial statements and disclosures.

In March 2016, the FASB ASU 2016-09, *Compensation- Stock Compensation* simplifying certain aspects of share-based payment accounting. Under the amended guidance, the Company will recognize excess tax benefits and tax deficiencies as income tax expense or benefit in its statement of operations on a prospective basis. As the Company has a valuation allowance, this change will impact the Company's net operating loss carryforward and the valuation allowance disclosures. Additionally, the Company will classify excess tax benefits as an operating activity and classify amounts the Company withholds in shares for the payment of employee taxes as a financing activity on the statement of cash flows for each period presented. The amended guidance allows the Company to account for forfeitures when they occur or continue to estimate them. The Company will continue to estimate its forfeitures. The amended share-based payment standard is effective for annual and interim periods beginning after December 15, 2016, with early adoption permitted in any interim or annual period. The Company adopted this guidance on January 1, 2017 and does not believe the amended guidance will have a material impact on its financial results.

The SEC staff issued Staff Accounting Bulletin ("SAB") 118, which provides guidance on accounting for the tax effects of the U.S. tax reform announced on December 22, 2017 by the U.S. Government commonly referred to as the Tax Cuts and Jobs Act. SAB 118 provides a measurement period that should not extend beyond one year from the U.S. tax reform enactment date for companies to complete the accounting under Accounting Standards Codification ("ASC") 740. In accordance with SAB 118, a company must reflect the income tax effects of those aspects of the U.S. tax reform for which the accounting under ASC 740 is complete. Specifically, the Company will be required to revalue its U.S. deferred tax assets and liabilities due to the federal income tax rate reduction from 35 percent to 21 percent. Since the Company has provided a full valuation allowance against its deferred tax assets, the revaluation of the deferred tax assets did not have a material impact on any period presented.

Note 4. Transactions with BioXcel

The Company has entered into an asset contribution agreement, effective June 30, 2017, with BioXcel, as amended and restated on November 7, 2017, or the Contribution Agreement, pursuant to which BioXcel agreed to contribute BioXcel's rights, title and interest in BXCL501, BXCL701, BXCL502 and BXCL702, and all of the assets and liabilities associated in consideration for (i) 9,480,000 shares of our common stock, (ii) $1 million upon completion of an initial public offering, (iii) $500 upon the later of the 12 month anniversary of an initial public offering and the first dosing of a patient in the bridging bioavailability/ bioequivalence study for the BXCL501 program, (iv) $500 upon the later of the 12 month anniversary of an initial public offering and the first dosing of a patient in the Phase 2 PoC open label monotherapy or combination trial with Keytruda for the BXCL701 program and (v) a one-time payment of $5,000 within 60 days after the achievement of $50,000 in cumulative net sales of any product or combination of products resulting from the development and commercialization of any one of the Candidates or a product derived therefrom.

The Company has also entered into a separation and shared services agreement with BioXcel that took effect on June 30, 2017, as amended and restated on November 7, 2017, or the Services Agreement,

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BIOXCEL THERAPEUTICS, INC.

NOTES TO FINANCIAL STATEMENTS (Continued)

(amounts in thousands, except share and per share data)

Note 4. Transactions with BioXcel (Continued)

pursuant to which BioXcel will allow the Company to continue to use the office space, equipment, services and leased employees based on the agreed upon terms and conditions for a payment of defined monthly and/or hourly fees.

In connection with the Services Agreement, BioXcel agreed to provide the Company a line of credit, which shall be capped at $1,000, or the Total Funding Amount, pursuant to the terms of a grid note, or the Grid Note. The Grid Note shall be payable upon the earlier of (i) the completion of an initial public offering and (ii) December 31, 2018, together with interest on the unpaid balance of each advance made under the Grid Note, which shall accrue at a rate per annum equal to the applicable federal rate for short-term loans as of the date hereof, in each case calculated based on a 365-day year and actual days elapsed. As of December 31, 2017, has drawn down $371 under the Grid Note.

The Parent has made investments of approximately $2,971 commencing January 1, 2015 through March 29, 2017 (the date of incorporation of the Company) that relate to the BTI Business which was offset by total losses from the BTI Business of $3,285 resulting in net Parent investment of $(314) as on March 29, 2017. Furthermore, the net value of the assets and liabilities amounting to net liabilities of $126 which pertain to the BTI Business were allocated to the Company have also been classified under net Parent investment. As the Company became a substantive operating entity beginning June 30, 2017, the net Parent investment account totaling an amount of $440 was reclassified into accumulated deficit for the period ended June 30, 2017.

For the period March 29, 2017 through June 30, 2017, BioXcel paid for expenses on the Company's behalf totaling approximately $562. This amount has been reduced to $440 as of December 31, 2017. The Company has agreed to reimburse BioXcel for this amount upon the earlier of (i) 30 days after the completion of an initial public offering and (ii) December 31, 2018.

Note 5. Equipment

Equipment consist of the following	December 31, 2017	December 31, 2016
Computers	$ 5	$ 5
Accumulated depreciation and amortization	(1)	—
	$ 4	$ 5

Note 6. Commitments and Contingencies

The Company is required to pay to BioXcel the amount of $5,000 within 60 days after the achievement of $50,000 in cumulative net sales of any product or combination of products resulting from the development and commercialization of any one of the candidates BXCL501, BXCL701, BXCL502, and BXCL702 or a product derived therefrom.

The Company is also required to pay to BioXcel the amount of $2,000 in connection with an initial public offering ("IPO"), (x) the first $1,000 of which the Company shall pay to BioXcel in a lump-sum payment within thirty (30) days after closing of the IPO and (y) the second $1,000, (i) $500 of which is payable upon the later of the 12 month anniversary of an offering and the first dosing of a patient in

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BIOXCEL THERAPEUTICS, INC.

NOTES TO FINANCIAL STATEMENTS (Continued)

(amounts in thousands, except share and per share data)

Note 6. Commitments and Contingencies (Continued)

the bridging bioavailability/bioequivalence study for the BXCL501 program and (ii) $500 of which is payable upon the later of the 12 month anniversary of an offering and the first dosing of a patient in the Phase 2 PoC open label monotherapy or combination trial with Keytruda for the BXCL701 program.

The employment agreements for Frank Yocca, the Chief Scientific Officer and Luca Rastelli, the Vice President—Oncology R&D have been contributed to the Company by the Parent as a part of the Agreement. The employment agreements provide, among other things, for the payment of three and four months respectively of severance compensation for terminations under certain circumstances. With respect to these agreement, at December 31, 2017, potential severance payout amounted to $104 and aggregated annual salaries amounted to $340.

The Company has entered into a contract with a clinical research organization in order to conduct its first human clinical trial in BXCL501. The contract totals approximately $1 million, to be incurred during 2018.

Note 7. Accrued Expenses

Accrued expenses consist of the following

	December 31, 2017	December 31, 2016
Accrued salaries and benefits	$ 79	$ 27
Professional and consultant fees	120	15
Legal Expenses	413	10
Materials and clinical trial expenses	403	—
	$ 1,015	$ 52

Note 8. Stockholders' Deficit / Net Parent Investment

The Parent has made investments of approximately $539 and $1,969 during the years ended, December 31 2017 and 2016, respectively, which were offset by net losses of $(4,539) and $(2,120) during the corresponding periods resulting in net Parent investment of $0 and $(324) as on December 31, 2017 and 2016. There was no net Parent investment as of December 31, 2017 as all amounts were transferred to accumulated deficit as of June 30, 2017.

For the period for January 1, 2017 to the date of incorporation (March 29, 2017), the Parent has made an investment of $539 which was offset by net loss of $(529). Furthermore, at June 30, 2017, the net value of the assets and liabilities amounting to net liabilities of $126 which pertain to the BTI Business were allocated to the Company have also been classified under net Parent investment. As the Company became a substantive operating entity beginning June 30, 2017, the net Parent investment account

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BIOXCEL THERAPEUTICS, INC.

NOTES TO FINANCIAL STATEMENTS (Continued)

(amounts in thousands, except share and per share data)

Note 8. Stockholders' Deficit / Net Parent Investment (Continued)

totaling an amount of $440 was reclassified into accumulated deficit for the period ended June 30, 2017.

	Net Parent Investment
Balance, January 1, 2015	**$ —**
Investment from Parent	463
Net loss	(636)
Balance as of December 31, 2015	**(173)**
Investment from Parent	1,969
Net loss	(2,120)
Balance as of December 31, 2016	**(324)**
Investment from Parent	539
Net loss	(529)
Balance as of March 29, 2017 (date of incorporation)	**(314)**
For the six months period ended June 30, 2017	
Liabilities assumed from Parent	(126)
Transfer to accumulated deficit	440
Balance as of June 30, 2017	**$ —**

Authorized Capital

The Company is authorized to issue up to 10,000,000 preferred shares with a par value of $0.001 per share. No preferred shares are issued and outstanding.

The Company is authorized to issue up to 50,000,000 shares of common stock with a par value of $0.001 per share. 9,480,000 shares were issued to BioXcel pursuant to the Contribution Agreement—see Note 4.

Description of Common Stock

Each share of common stock has the right to one vote. The holders of common stock are entitled to dividends when funds are legally available and when declared by the board of directors.

On September 29, 2017, the Company entered into a Common Stock Purchase Agreement under which, the Company agreed to sell to the Purchasers, for cash, 155,709 shares of Common stock, par value $001. per share, for the purchase price of $4.82 per share. Gross and net proceeds totaled $751. In October 2017 the Company sold an additional 271,839 shares of Common stock with gross and net proceeds of $1,311. In January and February 2018, the Company sold 283,452 shares of common stock at $6.88 per share for total gross and net proceeds of approximately $1,950.

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BIOXCEL THERAPEUTICS, INC.

NOTES TO FINANCIAL STATEMENTS (Continued)

(amounts in thousands, except share and per share data)

Note 8. Stockholders' Deficit / Net Parent Investment (Continued)

Registration Rights

Certain shareholders have demand registration rights with respect to these securities, as set forth in the stock Purchase Agreement. These registration rights would require the Company to give notice to each Investor and include in such registration statement, other than in an S-1 filing all or any part of the Shares that the Purchaser requests to be registered; however, that the Company shall not be required to register the resale of any shares that are eligible for resale under Rule 144 of the Securities Act without any requirement for the Company to maintain current public information and without any limitation on volume or manner of sale.

Anti Dilution Protection

In the event that prior to an IPO, and between September 30, 2017 and September 30, 2018, the Company issues additional securities below $4.82 per share of common stock, the Company shall issue the investors additional shares of common stock based on a specific formula which would average the price of the shares sold under these stock purchase agreements with shares sold at a lower purchase price.

Note 9 Stock-Based Compensation

Stock Options

The Company's 2017 Stock Incentive Plan (the "2017 Stock Plan") became effective in August 2017 and will expire in August 2027. Under the 2017 Stock Plan, the Company may grant incentive stock options, non-statutory stock options, restricted stock awards and other stock-based awards.

As of December 31, 2017, there were 2,962,500 shares of the Company's common stock authorized for issuance under the 2017 Stock Plan. Options granted under the 2017 Stock Plan have a term of ten years with vesting determined by the board of directors, generally over a four-year term.

The fair value of options at date of grant was estimated using the Black-Scholes option-pricing model with the following assumptions. Stock-based awards to non-employees are re-measured at fair value each financial reporting date until performance is complete:

Employees

	For the Year Ended December 31, 2017
Exercise price per share	$.41 - $5.55
Expected stock price volatility	76.61% - 77.65%
Risk-free rate of interest	1.78% - 2.17%
Fair value of grants per share	$.26 - $3.95
Expected Term (years)	5.2 - 7.0

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BIOXCEL THERAPEUTICS, INC.

NOTES TO FINANCIAL STATEMENTS (Continued)

(amounts in thousands, except share and per share data)

Note 9 Stock-Based Compensation (Continued)

Non-Employees

	For the Year Ended December 31, 2017
Exercise price per share	$ .41
Expected stock price volatility	77.50%
Risk-free rate of interest	2.39%
Fair value of grants per share	$ 8.08
Expected Term (years)	9.6

The Company does not have a history of market prices of its common stock as it is not a public company and, as such, volatility was estimated using historical volatilities of similar public companies. The expected life of the employee awards is estimated based on the simplified method, which calculates the expected life based upon the midpoint of the term of the award and the vesting period. The Company uses the simplified method because it does not have sufficient option exercise data to provide a reasonable basis upon which to estimate the expected term. The expected term of non-employee awards represents the awards contractual term. The expected dividend yield is 0% as the Company has no history of paying dividends nor does management expect to pay dividends over the contractual terms of these options. The risk-free interest rates are based on the United States Treasury yield curve in effect at the time of grant, with maturities approximating the expected life of the stock options.

The following table summarizes information about stock option activity during the period the Plan was in effect (in thousands, except share and per share data):

Employee Options

(Dollars thousands, expect shares and per share amounts)	Number of Shares	Weighted Average Exercise Price per Share	Total Intrinsic Value	Weighted Average Remaining Contractual Life (in years)
Employee options granted	1,813,524	$ .65	$ 13,892	9.7
Outstanding as of December 31, 2017	1,813,524	$ .65	$ 13,892	9.7
Options vested and exercisable as of December 31, 2017	124,188	$ .41	$ 981	9.6

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BIOXCEL THERAPEUTICS, INC.

NOTES TO FINANCIAL STATEMENTS (Continued)

(amounts in thousands, except share and per share data)

Note 9 Stock-Based Compensation (Continued)

Non-employee Options

(Dollars thousands, expect shares and per share amounts)	Number of Shares	Weighted Average Exercise Price per Share	Total Intrinsic Value	Weighted Average Remaining Contractual Life (in years)
Non-employee options granted	496,515	$ .41	$ 3,921	9.6
Outstanding as of December 31, 2017	496,515	$ .41	$ 3,921	9.6
Options vested and exercisable as of December 31, 2017	—	$ —	$ —	—

The Company granted 2,310,039 options to purchase shares of common stock during the year ended December 31, 2017. No options were exercised during the year ended December 31, 2017 and 652,461 shares remain available for grant as of December 31, 2017. On December 28, 2017 the board of directors accelerated the vesting of options to purchase 124,188 shares of common stock previously granted to our chairman of the board of directors because of the unique scientific and business skills and guidance he has provided to the Company, which has resulted in an IND Exemption for BXCL501 and a clinical development plan for BXCL701. As a result, under ASC 718 this is considered a type 1 probable to probable modification of a vesting condition and was accounted for under ASC 718 by expensing the balance of the award during the period ending December 31, 2017. The board has no plans to accelerate any other stock options granted by the Company. This accelerated vesting resulted in charge to general and administrative expenses of approximately $30.

Compensation costs associated with the Company's stock options are recognized, based on the grant-date fair values of these options, over the requisite service period. Accordingly, the Company recognized stock based compensation expense of $1.2 million for the year ended December 31, 2017. There was no corresponding charge for corresponding period ending December 31, 2016 as the plan did not exist.

Unrecognized compensation expense related to unvested awards as of December 31, 2017 was $3.1 million for non-employees and $498 for employees and will be recognized over the remaining vesting periods of the underlying awards. The weighted-average period over which such compensation is expected to be recognized is 1.8 years for non-employees and 9 months for employees.

BioXcel Charges

The financial statements include certain expenses of BioXcel, the parent, including stock-based compensation, that were carved-out of the historical financial results of BioXcel based on the percentage of the expense attributable to BTI related activities.

BioXcel, has granted stock options to its employees under its own Equity Incentive Plan ("BioXcel Plan"). Stock-based compensation expense from the BioXcel Plan is allocated to the Company over the period over which those stock option awards vest and are based the on the percentage of time spent on Company activities compared to BioXcel activities, which is the same basis used for allocation of salary costs. The BioXcel stock option awards are valued at fair value on the date of grant and that fair value is recognized over the requisite service period. The estimated fair value of these BioXcel stock option

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BIOXCEL THERAPEUTICS, INC.

NOTES TO FINANCIAL STATEMENTS (Continued)

(amounts in thousands, except share and per share data)

Note 9 Stock-Based Compensation (Continued)

awards was determined using the Black Scholes option pricing model on the date of grant. Significant judgment and estimates were used to estimate the fair value of these awards, as they are not publicly traded.

For the years ended December 31, 2017 and 2016 share-based compensation expense recognized by the Company in its statements of operations related to BioXcel equity awards totaled $439 and $671 respectively. For the year ending December 31, 2017 $199 of the $439 share based compensation is part of the net loss for the period January 1, 2017 to June 30, 2017 which was transferred to accumulated deficit as explained in Note 8.

Total share based compensation charges for the years ending December 31, 2017 and 2016 were $1,606 and $671 respectively.

Note 10. Income Taxes

The Parent is a standalone S corporation and its tax obligations were passed through to its shareholders and were not a liability of the S corporation. As a result, BioXcel does not require a tax provision for federal or state purposes.

Pursuant to incorporation of the Company as a C corporation on March 29, 2017, BioXcel became the sole owner of BioXcel Thereapeutics, Inc., and contributed certain assets to the Company in a tax free transaction. From the date of incorporation, the Company is a standalone C corporation subject to corporate income tax and the deferred taxes of the Company have been calculated accordingly.

The significant components of the Company's net deferred tax assets at December 31, 2017 are shown below. In determining the realizability of the Company's net deferred tax asset, the Company considered numerous factors, including historical profitability, estimated future taxable income, and the industry in which it operates. Based on this information the Company has provided a valuation allowance for the full amount of its net deferred tax asset because the Company has determined that it is more likely than not that it will not be realized.

	2017
Federal net operating losses	$ 627
State net operating losses	212
Stock based compensation	268
Federal tax credit	42
Accrued expense	9
Total gross deferred tax assets	**1,158**
Less: valuation allowance	$ (1,158)
Net deferred tax assets	**$ —**

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BIOXCEL THERAPEUTICS, INC.

NOTES TO FINANCIAL STATEMENTS (Continued)

(amounts in thousands, except share and per share data)

Note 10. Income Taxes (Continued)

A reconciliation between the Company's effective tax rate and the federal statutory rate for the period from inception to December 31, 2017 is as follows:

	2017	
Federal Statutory Rate	$ (1,543)	34%
Change in Federal Rate	517	–11.38%
Permanent Differences	192	–4.24%
Research and Development	(42)	(0.92)%
State Taxes	—	–0%
Valuation Allowance	876	–19.30%
Effective Tax Rate	—	0%

At December 31, 2017, the Company had approximately $2.986 million of gross federal and state net operating loss carry-forwards. If not utilized, the federal and state net operating loss carry-forwards will begin to expire in 2037. The utilization of such net operating loss carry-forwards and realization of tax benefits in future years depends predominantly upon having taxable income. The Company also has approximately $42 of federal research and development credits which will begin to expire in 2037 if not utilized.

Utilization of the NOL and research tax credit carryforwards may be subject to a substantial annual limitation due to ownership change limitations that has occurred or that could occur in the future, as required by Section 382 of the Code, as well as similar state and foreign provisions. These ownership changes may limit the amount of NOL and research tax credit carryforwards that can be utilized annually to offset future taxable income and tax, respectively. In general, an "ownership change" as defined by Section 382 of the Code results from a transaction or series of transactions over a three-year period resulting in an ownership change of more than 50 percentage points of the outstanding stock of a company by certain stockholders or public groups. To date, the Company's NOL's have not been subject to Section 382 limitation.

Entities are also required to evaluate, measure, recognize and disclose any uncertain income tax provisions taken on their income tax returns. The Company has analyzed its tax positions and has concluded that as of December 31, 2017 there were no uncertain positions. Interest and penalties, if any, as they relate to income taxes assessed, are included in the income tax provision. There was no income tax related interest and penalties included in the income tax provision.

For the year ended December 31, 2017, the Company revised its estimated annual effictive rate to reflect a change in the federal statutory rate from 35% to 21%, resulting from legislation that was enacted on December 22, 2017. The rate change is effective beginning of our calendar year 2018.

In addition, to reflect the new corporate tax rate beginning January 1, 2018, we recognized a tax benefit in our tax provision for the period related to adjusting our deferred tax balance to reflect the new corporate tax rate. As a result, income tax expense reported for the year was adjusted to reflect the change in the tax law and resulted in a decrease in the income tax expense of $470 for the year.

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BIOXCEL THERAPEUTICS, INC.

NOTES TO FINANCIAL STATEMENTS (Continued)

(amounts in thousands, except share and per share data)

Note 11. Deferred Offering Costs

The Company capitalizes certain legal and other third-party fees that are directly associated with in-process equity financings as deferred offering costs until such financings are consummated. After consummation of the equity financing, these costs are recorded as a reduction of the proceeds generated as a result of the offering. Should the planned equity financing be abandoned, the deferred offering costs will be expensed immediately as a charge to operating expense in the statements of operations. Deferred offering costs amounted to $461 at December 31, 2017. There were no deferred offering costs at December 31, 2016.

Note 12. Subsequent Events

a. In January and February 2018, the Company entered into a series of common stock purchase agreements under which, the Company agreed to sell to the purchasers, for cash 283,452 shares of common stock, par value $.001 per share, for the purchase price of $6.88 per share. Gross and net proceeds totaled approximately $1,950.

b. On February 5, 2018, the Company entered into a contract to manufacture BXCL 701 for approximately $267.

c. On March 4, 2018, the Board of Directors and shareholders of the Company approved an increase of 500,070 common stock options under the Company's 2017 Equity Incentive Plan. The total number of awards authorized for issuance under the 2017 Equity Incentive Plan now totals 3,462,570, an increase from 2,962,500.

d. On February 25, 2018, the Company approved an amended and restated certificate of incorporation to increase the authorized shares of capital stock from 100,000 shares to 60 million shares of which 50 million will be common shares and 10 million will be preferred shares. The par value of such shares is $0.001 per share. In addition, the Company approved a 237 for 1 stock split. The amended and restated certificate of incorporation and the stock split were contingent upon the successful completion of the Company's IPO. On March 7, 2018, the Prospectus was declared effective by the Securities and Exchange Commission and the contingent measures were met. The financial statements reflect the effects of these actions for all years presented. The Company's common shares commenced trading on NASDAQ on March 8, 2018.

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5,454,545 Shares

BIOXCEL THERAPEUTICS, INC.

Common Stock

Prospectus

March 7, 2018

Joint Book-Running Managers

Barclays

UBS Investment Bank

BMO Capital Markets

Lead Manager

Canaccord Genuity

Until April 1, 2018 (25 days after the date of this prospectus), all dealers that effect transactions in these securities, whether or not participating in this offering, may be required to deliver a prospectus. This delivery requirement is in addition to the dealers' obligation to deliver a prospectus when acting as an underwriter and with respect to their unsold allotments or subscriptions.