Prospectus

4,000,000 shares

Common shares

This is a public offering of shares of common stock of Auspex Pharmaceuticals, Inc. Auspex is offering 3,000,000 shares of common stock and the selling stockholders identified in this prospectus are offering 1,000,000 shares of common stock. We will not receive any proceeds from the sale of shares by the selling stockholders. On January 22, 2015, the last reported sale price of our common stock on The NASDAQ Global Market was $56.90 per share.

Our common stock is listed on The NASDAQ Global Market under the symbol ASPX.

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

	Per share	Total
Public offering price	$56.50	$226,000,000
Underwriting discounts and commissions(1)	$ 3.39	$ 13,560,000
Proceeds, before expenses, to us	$53.11	$159,330,000
Proceeds, before expenses, to selling stockholders	$53.11	$ 53,110,000

(1) We have agreed to reimburse the underwriters for certain expenses. See "Underwriting."

Auspex has granted the underwriters an option for a period of 30 days to purchase up to 600,000 additional shares of common stock.

Investing in our common stock involves a high degree of risk. See "Risk Factors" beginning on page 12.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or passed on the adequacy or accuracy of this prospectus. Any representation to the contrary is a criminal offense.

The underwriters expect to deliver shares of common stock to purchasers on January 28, 2015.

Sole Book-Running Manager

J.P. Morgan

Co-Lead Managers

Stifel **BMO Capital Markets**

Co-Managers

Baird **William Blair**

January 22, 2015

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Neither we, the selling stockholders nor the underwriters have authorized anyone to provide any information or to make any representations other than those contained in this prospectus, any documents incorporated by reference into this prospectus or in any free writing prospectuses prepared by or on behalf of us or to which we have referred you. We and the selling stockholders take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may give you. This prospectus is an offer to sell only the shares offered hereby, but only under circumstances and in jurisdictions where it is lawful to do so. The information contained in this prospectus or in any applicable free writing prospectus is current only as of its date, regardless of its time of delivery or any sale of shares of our common stock. Our business, financial condition, results of operations and prospects may have changed since that date.

For investors outside the United States: We have not, and the underwriters have not, done anything that would permit this offering or possession or distribution of this prospectus in any jurisdiction where action for that purpose is required, other than in the United States. Persons outside the United States who come into possession of this prospectus must inform themselves about, and observe any restrictions relating to, the offering of the shares of common stock and the distribution of this prospectus outside the United States.

Prospectus summary

This summary highlights information contained in other parts of this prospectus or incorporated by reference into this prospectus from our Annual Report on Form 10-K for the year ended December 31, 2013, our Quarterly Report on Form 10-Q for the quarter ended September 30, 2014 and our other filings with the Securities and Exchange Commission listed in the section of the prospectus entitled "Incorporation of Certain Information by Reference. Because it is only a summary, it does not contain all of the information that you should consider before investing in shares of our common stock and it is qualified in its entirety by, and should be read in conjunction with, the more detailed information appearing elsewhere or incorporated by reference in this prospectus. You should read the entire prospectus, the registration statement of which this prospectus is a part, and the information incorporated by reference herein in their entirety before investing in our common stock, including the information in this prospectus and in our Annual Report on Form 10-K for the year ended December 31, 2013 and Quarterly Report on Form 10-Q for the quarter ended September 30, 2014 under the heading "Risk Factors" and our financial statements and the related notes. Unless the context requires otherwise, references in this prospectus to "Auspex," "we," "us" and "our" refer to Auspex Pharmaceuticals, Inc.

Overview

Our company

We are a biopharmaceutical company focused on the development and commercialization of novel medicines for people with movement disorders and other rare diseases, including orphan diseases, which are rare diseases that affect fewer than 200,000 people in the United States. Our pipeline includes product candidates to address unmet medical needs in hyperkinetic movement disorders, such as chorea (abnormal involuntary movements) associated with Huntington's disease, an orphan disease, and tardive dyskinesia and Tourette syndrome, subsets of either of which may be deemed to be orphan diseases, as well as other orphan indications.

In the United States, a majority of the 30,000 Huntington's disease patients manifest chorea, an estimated 500,000 people suffer from tardive dyskinesia and approximately 100,000 children have tics (abnormal involuntary movements or vocalizations) associated with Tourette syndrome. Movement disorders are debilitating medical conditions which can lead to physical injury, social isolation, loss of independence and interference with employment and activities of daily living. These debilitating disorders are poorly addressed and remain largely untreated. We believe that there is a significant need for a safe and efficacious treatment for these serious conditions.

Our lead product candidate, SD-809, is a small molecule inhibitor of vesicular monoamine 2 transporter, or VMAT2, that is designed to regulate the levels of a specific neurotransmitter, dopamine, in the brain. A number of hyperkinetic movement disorders are triggered by abnormal dopamine regulation in the brain. We have recently completed a successful Phase 3 clinical trial of SD-809 for the potential treatment of chorea associated with Huntington's disease. In this trial, which we refer to as First-HD, SD-809 met its primary efficacy endpoint of a statistically significant improvement compared to placebo in the total maximal chorea, or TMC, score on the Unified Huntington's Disease Rating Scale over placebo, as well as showed significant improvements compared to placebo in multiple secondary endpoints including patient global impression of change, or PGIC, clinical global impression of change, or CGIC, and the physical functioning scale of the 36-Item Short-Form Health Survey developed by the RAND Corporation, or the SF-36, a measure of quality of life. Importantly, in First-HD, SD-809 showed a favorable safety and tolerability profile with low rates of adverse events, such as depression, somnolence, akathisia/restlessness and anxiety.

We have also successfully completed the four week switch portion of an open-label clinical trial, which we refer to as ARC-HD Switch, where 37 patients were switched overnight to SD-809 from stable doses of tetrabenazine (marketed as Xenazine® in the United States), approved by the U.S. Food and Drug Administration, or FDA, solely for the treatment of chorea associated with Huntington's disease. In this trial, SD-809 maintained chorea control, with chorea scores declining by approximately one point from baseline at Weeks 1 and 4. In addition, data from 21 patients were available at Week 8 at the time of the analysis; these data demonstrated approximately a two point decline from baseline in chorea scores. Data for the remaining 16 patients will be available at a future date.

We believe that the favorable efficacy and safety data demonstrated in First-HD and ARC-HD, combined with earlier clinical trial results suggest that SD-809, if approved, could become the therapy of choice for treating chorea associated with Huntington's disease, while also suggesting the potential for addressing unmet needs in a variety of other hyperkinetic movement disorders which will need to be confirmed in additional clinical trials. There are no FDA-approved treatments for tardive dyskinesia and there are limited treatment options for treatment of tics associated with Tourette syndrome. A 2007 retrospective chart review conducted by physicians at the Baylor College of Medicine reported that over 75% of patients treated with tetrabenazine had moderate to marked improvement for chorea associated with Huntington's disease, tardive dyskinesia and tics associated with Tourette syndrome. With the same mechanism of action as tetrabenazine, SD-809 may potentially be used to treat these indications. We believe that SD-809 has the potential to change the treatment paradigm and expand the treatment of chorea associated with Huntington's disease, tardive dyskinesia and Tourette syndrome, if the FDA approves SD-809 for these indications.

We have initiated two safety and efficacy clinical trials of SD-809 for the potential treatment of tardive dyskinesia. ARM-TD (Aim to Reduce Movements in Tardive Dyskinesia) is a Phase 2/3 randomized, double-blind, placebo-controlled, dose-titration clinical trial of SD-809 in approximately 90 patients with tardive dyskinesia. Topline results from this trial are expected in mid-2015. Based on feedback we obtained at a meeting with the FDA, the ARM-TD trial may qualify as one of the two pivotal trials needed for a 505(b)(2) New Drug Application, or NDA, filing, subject to FDA review. The AIM-TD (Addressing Involuntary Movements in Tardive Dyskinesia) clinical trial is a Phase 3, randomized, double-blind, placebo-controlled, parallel group trial in approximately 200 people with tardive dyskinesia. We expect topline results from this trial in 2016. Contingent upon successful completion of both studies, we plan to file an NDA for SD-809 for the treatment of tardive dyskinesia in 2016.

In addition to our Huntington's disease and tardive dyskinesia programs, we have initiated an open-label preliminary efficacy, pharmacokinetic and safety Phase 1b clinical trial of SD-809 for the treatment of tics associated with Tourette syndrome. We plan to enroll approximately 20 subjects to evaluate preliminary efficacy and safety, in addition to studying the pharmacokinetics of SD-809 in adolescents. We expect topline data from this trial by mid-2015.

Our market opportunity

Hyperkinetic movement disorders are characterized by abnormal, involuntary movements that can arise from a variety of causes, typically either genetic or drug-induced. Huntington's disease, tardive dyskinesia and Tourette syndrome are three conditions that prominently feature hyperkinetic movements.

In the United States, an estimated 30,000 people have Huntington's disease. One of the most visually prominent symptoms of this disease is chorea, which occurs in 90% of patients, and is moderate to severe in approximately 70% of these patients.

Since its launch in the fourth quarter of 2008, annual tetrabenazine sales in the United States have grown to approximately $250 million for the year ended December 31, 2013, which represents approximately 4,000 patients on tetrabenazine therapy in the United States at the end of 2013. Sales of tetrabenazine for the 12 months ended September 30, 2014 are expected to be approximately $290 million, which represents an estimated $70,000 to $75,000 annual cost of treatment per patient. Based on the 14% price increase of Xenazine effective January 2, 2015, reported by the Red Book Online, the annual cost of treatment in 2015 is estimated to be $80,000 to $85,000 per patient. We believe this limited usage of tetrabenazine is primarily due to its undesirable tolerability and side effect profile (including depression, somnolence, akathisia and anxiety), its dosing frequency, drug interactions, the requirement for genotyping for drug metabolizing enzymes, a warning and precaution in the label regarding corrected QT, or QTc, prolongation and other factors. The distribution of the drug is also highly restricted and is subject to an FDA-mandated Risk Evaluation and Mitigation Strategies, or REMS, program. We believe the foregoing properties of tetrabenazine have contributed to its limited use.

In the United States, an estimated 500,000 patients have tardive dyskinesia. Tardive dyskinesia is a hyperkinetic movement disorder that typically manifests as rapid, repetitive, stereotypic movements that can be induced by certain drugs, such as neuroleptics, which are used for treating psychiatric conditions, as well as by drugs such as metoclopramide, which are used for treating various gastrointestinal disorders. These patients are managed largely by psychiatrists and movement disorder neurologists, and there are no FDA-approved treatments for tardive dyskinesia.

In the United States, an estimated 100,000 children have tics (abnormal involuntary movements or vocalizations) associated with Tourette syndrome, with an estimated 37% categorized as moderate to severe. Peak severity of the disorder is around 12 years of age, with an estimated 13% to 22% of affected children continuing to take medications for tics as adults. There have been no new drugs introduced for treating tics associated with Tourette syndrome in more than 30 years and we believe that physicians consider the two approved neuroleptics to be inadequate. These treatments carry, among other adverse events, the risk of causing permanent neurologic deficits, such as tardive dyskinesia.

SD-809, Our product candidate for the treatment of hyperkinetic movement disorders

We have extensive know-how and experience in chemistry involving deuterium, a non-toxic, naturally occurring form of hydrogen. The substitution of deuterium (^{2}H) for hydrogen (^{1}H) in a molecule attenuates the breakdown of the molecule and/or its active metabolites resulting in a differentiated profile as compared to the hydrogen-containing molecule. Selective deuterium substitution maintains the same shape, size, charge, intrinsic potency and target pharmacology of the non-deuterium substituted molecule. Using our deuterium technology, we made chemical modifications at specific positions in the tetrabenazine molecule to create the novel drug candidate SD-809 (deutetrabenazine). We developed SD-809 to have the same intrinsic potency and target pharmacology of tetrabenazine and to improve its tolerability and safety profile. SD-809 is an oral, small molecule product candidate with potential for once-daily or twice-daily dosing. We have successfully completed a Phase 3 clinical trial of SD-809 for the potential treatment of chorea associated with Huntington's disease and are also conducting two clinical trials of SD-809 for the treatment of tardive dyskinesia, as well as one Phase 1b clinical trial for tics associated with Tourette syndrome.

Based on the results of our clinical trials, we plan to submit an NDA to the FDA for SD-809 for the treatment of chorea associated with Huntington's disease by mid-2015 and, if approved, expect to launch commercial sales in 2016. This NDA will use a Section 505(b)(2) regulatory path which we expect will allow us to rely, in our NDA filing, on certain prior nonclinical and clinical safety findings made by the FDA in its approval of the tetrabenazine NDA. We expect the FDA to review data from our own clinical trials as well, including data from First-HD and ARC-HD, as

part of its review of our NDA submission. We are also targeting an NDA submission to the FDA for SD-809 for the treatment of tardive dyskinesia in 2016 pending results from our two ongoing clinical trials.

We intend to commercialize SD-809 for chorea associated with Huntington's disease, if approved, through a small specialty sales force. There are a limited number of neurologists nationwide who treat movement disorders and we believe that such a sales force could effectively address the U.S. market for chorea associated with Huntington's disease. We discovered and developed SD-809 and we retain unencumbered worldwide rights for its development and commercialization. We currently own issued composition of matter patents for SD-809 that are expected to expire in 2031 in the United States and 2029 in Europe, before any patent term extension or equivalent to which we may be entitled in the United States or other jurisdictions where we have issued patents. We have additional patent applications for SD-809 that, if issued, will cover composition of matter, methods of treatment, manufacturing, formulations and other applications and aspects of SD-809, which could potentially extend the patent exclusivity period for SD-809. SD-809 has also been granted an orphan drug designation by the FDA for the treatment of Huntington's disease and an orphan drug designation for the treatment of Tourette syndrome in the pediatric population (defined as zero through 16 years of age).

Additional product candidates

In addition to the issued or allowed patents covering SD-809, we have in our portfolio 60 issued or allowed patents and 60 patent families in prosecution covering, among other things, the deuterium-containing form of 58 drugs. These patents include composition of matter and other patents for SD-560, a deuterium-containing form of pirfenidone, which we are evaluating in a Phase 1 cross-over clinical trial versus pirfenidone, with data expected by mid-2015, as well as for SD-1077, a deuterium-containing form of levodopa, which we will be evaluating in proof of concept clinical trials with data expected in 2016. Our portfolio includes other deuterium-containing compounds that are at various stages of development. We may selectively and opportunistically sell or partner rights for any of these compounds, or acquire one or more additional programs or develop and commercialize them ourselves based on strategic considerations and availability of resources.

Our team

Our management team has extensive experience in drug delivery and development for orphan and central nervous system, or CNS, product candidates. Our Chief Executive Officer, Dr. Pratik Shah, has been involved with the company since 2007, including as the executive chairman, setting the strategy for advancing SD-809 for the treatment of hyperkinetic movement disorders. Dr. David Stamler, our Chief Medical Officer, successfully led the approval of tetrabenazine for chorea associated with Huntington's disease while at Prestwick Pharmaceuticals. Dr. Samuel Saks, our Chief Development Officer, was a co-founder and Chief Executive Officer of Jazz Pharmaceuticals, a specialty CNS pharmaceutical company, and senior vice president, medical affairs and group vice president for ALZA Corporation, or ALZA, a drug delivery-focused pharmaceutical company, prior to its acquisition by Johnson & Johnson. Our Chief Operating Officer, Dr. Bharatt Chowrira, served as Chief Executive Officer of Addex Therapeutics, where he provided leadership to advance its lead program for the treatment of Parkinson's disease, levodopa-induced dyskinesia and dystonia. Our Chief Financial Officer, John Schmid, was previously Chief Financial Officer and co-founder of Trius Therapeutics, Inc., a late-stage antibiotics company acquired by Cubist Pharmaceuticals, Inc. in September 2013.

Our strategy

Our goal is to become a leading biopharmaceutical company focused on the development and commercialization of new medicines for people with movement disorders and other rare diseases, initially targeting hyperkinetic movement disorders. Key elements of our strategy to achieve this goal are to:

- Develop and commercialize SD-809 to be a market leader for the treatment of chorea associated with Huntington's disease, based on its differentiated profile.
- Develop SD-809 for the treatment of additional hyperkinetic movement disorders with unmet medical needs, including tardive dyskinesia and Tourette syndrome.
- Build targeted sales and marketing capabilities in the United States for SD-809, initially focused on movement disorder neurologists.
- Leverage our expertise in deuterium chemistry as well as clinical and regulatory development to derive value from our broad portfolio of proprietary product candidates.

Risks associated with our business

Our business and our ability to implement our business strategy are subject to numerous risks, as more fully described in the section entitled "Risk Factors" immediately following this prospectus summary and in our Annual Report on Form 10-K for the year ended December 31, 2013 and Quarterly Report on Form 10-Q for the quarter ended September 30, 2014, incorporated herein by reference. You should read these risks before you invest in our common stock. We may be unable, for many reasons, including those that are beyond our control, to implement our business strategy. In particular, risks associated with our business include:

- We are highly dependent on the success of SD-809, which is still in clinical development, and we may not be able to successfully obtain regulatory or marketing approval for, or successfully commercialize, this product candidate in any of the indications for which we plan to develop it, which include chorea associated with Huntington's disease, tardive dyskinesia and Tourette syndrome.
- Clinical development is a lengthy and expensive process with an uncertain outcome. Because the results of early clinical trials are not necessarily predictive of future results, SD-809 may not have favorable results in ongoing or later clinical trials or receive regulatory approval.
- If the FDA does not conclude that SD-809 satisfies the requirements for the Section 505(b)(2) regulatory approval pathway, or if the requirements for approval of SD-809 under Section 505(b)(2) are not as we expect, the development and approval of SD-809 will likely take significantly longer, cost significantly more and entail significantly greater complexity and risks than anticipated, and in any case may not be successful.
- We anticipate that SD-809 will require a REMS which could delay the approval of SD-809 and increase the cost, burden and liability associated with the commercialization of SD-809.
- We may experience delays in the commencement or completion of our clinical trials, which could result in increased costs to us and delay our ability to pursue regulatory approval and generate product revenues.
- If we are unable to establish sales and marketing capabilities, we may not be able to effectively market and sell our products and generate product revenue.
- We have incurred significant operating losses since our inception and anticipate that we will continue to incur losses for the foreseeable future and may never be profitable.

- To complete the development and commercialization of SD-809, if approved, for all three planned indications, we will require additional capital. Raising additional funds through debt or equity financing may be dilutive or restrict our operations and raising funds through collaborations or licenses may require us to relinquish rights to our product candidates.
- If we are unable to obtain or protect intellectual property rights related to our product candidates, we may not be able to compete effectively in our market.
- Future sales and issuances of our common stock or rights to purchase common stock by us, including pursuant to our equity incentive plans which provide for an automatic increase in the number of shares of common stock issuable thereunder each calendar year through 2024, could result in additional dilution of the percentage ownership of our stockholders and could cause our stock price to decline.

Corporate and other information

We were incorporated in California in February 2001. In June 2007, we reincorporated in Delaware. Our principal executive offices are located at 3333 N. Torrey Pines Court, Suite 400, San Diego, California, 92037, and our telephone number is (858) 558-2400. Our corporate website address is www.auspexpharma.com. Information contained on or accessible through our website is not a part of this prospectus, and the inclusion of our website address in this prospectus is an inactive textual reference only.

This prospectus contains references to our trademarks and to trademarks belonging to other entities. Solely for convenience, trademarks and trade names referred to in this prospectus, including logos, artwork and other visual displays, may appear without the ® or ™ symbols, but such references are not intended to indicate, in any way, that their respective owners will not assert, to the fullest extent under applicable law, their rights thereto. We do not intend our use or display of other companies' trade names or trademarks to imply a relationship with, or endorsement or sponsorship of us by, any other companies.

Implications of being an emerging growth company

As a company with less than $1.0 billion in revenue during our last fiscal year, we qualify as an "emerging growth company" as defined in the Jumpstart Our Business Startups Act, or JOBS Act, enacted in April 2012. An "emerging growth company" may 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 and only two years of related Management's Discussion and Analysis of Financial Condition and Results of Operations 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, or the Sarbanes-Oxley Act;
- 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 stockholder approval of any golden parachute payments not previously approved.

We may use these provisions until the last day of our fiscal year following the fifth anniversary of our initial public 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 $1.0 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 elected to take advantage of certain of the reduced disclosure obligations in the registration statement of which this prospectus is a part and may elect to take advantage of other reduced reporting requirements in future filings. As a result, the information that we provide to our stockholders may be different than you might receive from other public reporting companies in which you hold equity interests.

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.

The offering

Common stock offered by us	3,000,000 shares
Common stock offered by the selling stockholders	1,000,000 shares
Common stock to be outstanding after this offering	30,482,166 shares
Option to purchase additional shares	The underwriters have a 30-day option to purchase up to a total of 600,000 additional shares of common stock from us.
Use of proceeds	We estimate that the net proceeds to us from this offering will be approximately $158.7 million, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us. The principal purposes of this offering are to increase our public float, increase our financial flexibility, and facilitate an orderly distribution of shares by the selling stockholders. We intend to use the net proceeds to us from this offering to fund building the commercial organization for, and commercial launch of, SD-809, if approved, for the potential treatment of chorea associated with Huntington's disease; for research and development, including, but not limited to funding future clinical and regulatory expenses related to SD-809 and related to the NDA filing and preparation for the commercial launch of SD-809, if approved, for other indications, such as tardive dyskinesia; to support the development of SD-560 and other programs in our pipeline; and for working capital and other general corporate purposes, including making monthly principal and interest payments on our credit facility. We will not receive any of the proceeds from the sale of shares of common stock by the selling stockholders. See "Use of Proceeds."
Risk factors	You should read the "Risk Factors" section of this prospectus for a discussion of certain of the factors to consider carefully before deciding to purchase any shares of our common stock.
NASDAQ Global Market symbol	"ASPX"

The number of shares of our common stock to be outstanding after this offering is based on 27,482,166 shares of common stock outstanding as of September 30, 2014 and excludes:

- 1,960,460 shares of common stock issuable upon the exercise of outstanding stock options as of September 30, 2014, at a weighted-average exercise price of $8.55 per share;
- 708,207 shares of common stock issuable upon the exercise of outstanding warrants as of September 30, 2014, at a weighted-average exercise price of $4.20 per share;

- 1,167,253 shares of common stock reserved for future issuance under our 2014 equity incentive plan, or the 2014 Plan, as of September 30, 2014; and
- 292,105 shares of common stock reserved for future issuance under our 2014 employee stock purchase plan, or the ESPP, as of September 30, 2014.

Unless otherwise indicated, all information contained in this prospectus assumes no exercise by the underwriters of their option to purchase up to a total of 600,000 additional shares of our common stock.

Summary financial data

The following table summarizes certain of our financial data. We derived the summary statement of operations data for the years ended December 31, 2011, 2012 and 2013 from our audited financial statements incorporated by reference into this prospectus from our Annual Report on Form 10-K for the year ended December 31, 2013. The summary statement of operations data for the nine months ended September 30, 2013 and 2014 and the summary balance sheet data as of September 30, 2014 were derived from our unaudited financial statements incorporated by reference into this prospectus from our Quarterly Report on Form 10-Q for the quarter ended September 30, 2014. The unaudited financial statements have been prepared on a basis consistent with our audited financial statements incorporated by reference into this prospectus and include, in our opinion, all adjustments, consisting only of normal recurring adjustments, necessary for the fair presentation of the financial information in those statements. Our historical results are not necessarily indicative of the results that may be expected in the future and results of interim periods are not necessarily indicative of the results for the entire year. The summary financial data should be read together with our financial statements and related notes, "Selected Financial Data" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" appearing elsewhere in this prospectus or incorporated by reference herein.

	Year ended December 31,			Nine months ended September 30,	
	2011	2012	2013	2013	2014
				(unaudited)	
				(in thousands, except share and per share data)	
Statement of Operations Data:					
Operating expenses:					
Research and development	$ 4,080	$ 11,741	$ 10,003	$ 6,524	$ 21,365
General and administrative	1,893	1,688	3,189	1,760	8,689
Total operating expenses	5,973	13,429	13,192	8,284	30,054
Gain on sale of assets	3,086	–	–	–	–
Operating loss	(2,887)	(13,429)	(13,192)	(8,284)	(30,054)
Other income (expense)	73	(1,683)	(2,437)	244	(9,479)
Net loss	(2,814)	(15,112)	(15,629)	(8,040)	(39,533)
Net income attributable to non-controlling interest	–	–	–	–	13
Net loss attributed to Auspex common stockholders	$ (2,814)	$ (15,112)	$(15,629)	$ (8,040)	$ (39,546)
Net loss per common share attributable to Auspex common stockholders, basic and diluted(1)	$(21,318)	$(114,485)	$ (371)	$(60,909)	$ (1.92)
Weighted-average shares outstanding used to calculate net loss per common share attributable to Auspex common stockholders, basic and diluted(1)	132	132	42,112	132	20,586,368

(1) See Note 2 to our audited financial statements incorporated by reference into this prospectus from our Annual Report on Form 10-K for the year ended December 31, 2013 for an explanation of the method used to calculate the basic and diluted net loss per common share and the number of shares used in the computation of the per share amounts.

The unaudited pro forma balance sheet data set forth below give effect to our issuance and sale of 3,000,000 shares of our common stock in this offering at the public offering price of $56.50 per share, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us.

We will not receive any proceeds from any sale of shares of our common stock in this offering by the selling stockholders; accordingly, there is no impact upon the pro forma balance sheet for these sales.

	As of September 30, 2014	
	Actual	Pro forma
		(unaudited)
		(in thousands)
Balance Sheet Data:		
Cash, cash equivalents and marketable securities	$159,482	$318,162
Working capital	156,908	315,588
Total assets	166,304	324,984
Long-term debt, including current portion, net of discount	14,550	14,550
Accumulated deficit	105,026	105,026
Total stockholders' equity	143,744	302,424

Risk factors

Investing in our common stock involves a high degree of risk. You should consider carefully the risks described below, together with all of the other information included or incorporated by reference in this prospectus, including the risks and uncertainties discussed under "Risk Factors" in our Annual Report on Form 10-K for the year ended December 31, 2013 and our Quarterly Report on Form 10-Q for the quarter ended September 30, 2014, before deciding whether to invest in our common stock. All of these risk factors are incorporated herein in their entirety. The risks described below and incorporated by reference are material risks currently known, expected or reasonably foreseeable by us. If any of these risks actually materialize, our business, prospects, financial condition, and results of operations could be seriously harmed. This could cause the trading price of our common stock to decline, resulting in a loss of all or part of your investment.

Risks related to our business and industry

We are highly dependent on the success of SD-809, which is still in clinical development, and we may not be able to successfully obtain regulatory or marketing approval for, or successfully commercialize, this product candidate in any of the indications for which we plan to develop it, which include chorea associated with Huntington's disease, tardive dyskinesia and Tourette syndrome.

Our future success will depend almost entirely on our ability to successfully develop, obtain regulatory approval for, and then successfully commercialize SD-809, our lead program, in the United States, which may never occur. We have no significant product candidates beyond Phase 1 clinical trials, in our clinical development pipeline other than SD-809. We currently generate no revenues from sales of any drugs and we may never be able to develop or commercialize a marketable drug.

Although we have successfully completed a Phase 3 clinical trial of SD-809 in patients having chorea associated with Huntington's disease and plan to submit a New Drug Application, or NDA, filing based on the favorable Phase 3 data, we cannot be certain that we will obtain regulatory approval, and even if we do, there can be no assurance that the approval process will not be lengthy. Also, before we can market and sell SD-809 in the United States or foreign jurisdictions, we may need to commence and complete additional clinical trials, and will need to manage clinical, preclinical, and manufacturing activities, obtain necessary regulatory approvals from the FDA in the United States and from similar foreign regulatory agencies in other jurisdictions, obtain manufacturing supply, build a commercial organization or enter into a marketing collaboration with a third party, and in some jurisdictions, obtain reimbursement authorization, among other things. We cannot assure you that we will be able to successfully complete the necessary clinical trials and/or obtain regulatory approvals and develop sufficient commercial capabilities for SD-809. We have not submitted a NDA to the FDA or similar drug approval filings to comparable foreign authorities, for any product candidate. Further, SD-809 may not receive regulatory approval even if it is successful in clinical trials. If we do not receive regulatory approvals, our business, prospects, financial condition and results of operations will be adversely affected. Even if we obtain regulatory approvals, we may never generate significant revenues from any commercial sales of SD-809. If SD-809 is approved and we fail to successfully commercialize it, we may be unable to generate sufficient revenues to sustain and grow our business and our business, prospects, financial condition and results of operations will be adversely affected.

If we are unable to obtain FDA approval of SD-809 in any of the indications for which we plan to develop it, which include chorea associated with Huntington's disease, tardive dyskinesia or Tourette syndrome, or any future product candidates, we will not be able to commercialize them in the United States and our business will be adversely impacted.

If we fail to obtain FDA approval for SD-809 or any future product candidates, we will be unable to market or sell such products in the United States, which will significantly impair our ability to generate any revenues. The clinical development of product candidates is subject to extensive regulation by the FDA in the United States and by comparable regulatory authorities in foreign markets. Product development is a very lengthy and expensive process, and its outcome is inherently uncertain. The product development timeline can vary significantly based upon the product candidate's novelty and complexity.

This regulatory review and approval process, which includes evaluation of preclinical studies and clinical trials of our product candidates as well as the evaluation of our manufacturing processes and our third-party contract manufacturers' facilities, is lengthy, expensive and uncertain. To receive approval, we must, among other things, demonstrate with substantial evidence from clinical trials that the product candidate is both safe and effective for each indication for which approval is sought, and failure can occur in any stage of development. Satisfaction of the approval requirements typically takes many years and the time needed to satisfy them may vary substantially, based on the type, complexity and novelty of the pharmaceutical product. As part of the U.S. Prescription Drug User Fee Act, the FDA has a goal to review and act on a percentage of all submissions in a given time frame. The general review goal for a drug application is ten to twelve months for a Standard Review application and six to eight months for a Priority Review application, depending on whether the drug at issue is a new molecular entity. Priority Review designation is given to drugs that offer major advances in treatment, or provide a treatment where no adequate therapy exists. The FDA's review goals are subject to change, and it is unknown whether the review of our NDA for SD-809, or an NDA filing for any of our other product candidates, will be completed within the FDA's review goals or will be delayed. Moreover, the duration of the FDA's review may depend on the number and types of other NDAs that are submitted to the FDA around the same time period. We cannot predict if, or when, we might receive regulatory approvals for SD-809 or any future product candidates. We intend to seek, where appropriate, Priority Review for our drug candidates but cannot be certain that we will obtain Priority Review, and even if we do, there can be no assurance that the approval process will not be lengthy. Moreover, any approvals that we obtain may contain significant limitations in the form of narrow indications, warnings, precautions or contra-indications with respect to conditions of use, or require as a condition of approval a REMS to ensure that the benefits of the drug outweigh the risks. In such event, our ability to generate revenues from such products could be greatly reduced and our business could be harmed.

The FDA has substantial discretion in the approval process and may refuse to consider our application for substantive review or may form the opinion after review of our data that our application contains deficiencies that prevent approval of a product candidate. If the FDA does not consider or approve our application, it may require that we conduct additional clinical, preclinical or manufacturing validation studies and submit that data before it will reconsider our application. Depending on the extent of these or any other studies, approval of any applications that we submit may be delayed or never approved, or may require us to expend more resources than we have available. It is also possible that additional studies, if performed and completed, may not be successful or considered sufficient by the FDA to support approval. If any of these outcomes occur, we may be forced to abandon one or more of our applications for approval, which might significantly harm our business, prospects, financial condition and results of operations.

The FDA or other comparable foreign regulatory authorities can delay, limit, or deny approval of a product candidate for many reasons, including:

- such 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 other regulatory authorities that a product candidate is safe and effective for any indication;
- such authorities may not accept clinical data from trials which are conducted at clinical facilities or in countries where the standard of care is potentially different from the United States;
- we may be unable to demonstrate that a product candidate's clinical and other benefits outweigh its safety risks;
- such authorities may disagree with our interpretation of data from preclinical studies or clinical trials;
- the results of our clinical trials may not demonstrate the safety or efficacy required by such authorities for approval;
- such authorities may find deficiencies in 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 such authorities may significantly change in a manner rendering our clinical data insufficient for approval.

Even if we do receive regulatory approval to market a product candidate, any such approval may be subject to limitations on the indicated uses for which we may market the product. It is possible that SD-809 and any of the product candidates we may seek to develop in the future may never obtain the appropriate regulatory approvals necessary for us to commence product sales. Any delay in obtaining, or an inability to obtain, applicable regulatory approvals would prevent us from commercializing our product candidates, generating revenues and achieving and sustaining profitability.

Clinical development is a lengthy and expensive process with an uncertain outcome. Because the results of early clinical trials are not necessarily predictive of future results, SD-809 may not have favorable results in ongoing or later clinical trials or receive regulatory approval.

Clinical development is expensive, takes many years to complete and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process and SD-809 is subject to the risks of failure inherent in drug development. Success in early clinical trials does not mean that later clinical trials will be successful. Product candidates in later-stage clinical trials may fail to demonstrate sufficient safety or efficacy despite having progressed through initial clinical trials, even at statistically significant levels. Companies frequently suffer significant setbacks in late-stage clinical trials due to lack of efficacy or adverse safety profiles, even after earlier clinical trials have shown promising results. Our ongoing and future clinical trials may not be successful.

The planned, ongoing and recently completed clinical trials of SD-809 may not be appropriately designed to support submission of an NDA to the FDA or demonstrate safety or efficacy at the level required by the FDA for product approval.

In December 2014, we announced positive topline results from our First-HD trial, a placebo-controlled trial of SD-809 in 90 patients with chorea associated with Huntington's disease and our ARC-HD Switch trial where patients with chorea associated with Huntington's disease on stable doses of tetrabenazine were switched overnight to SD-809 (at about half the tetrabenazine dose) and monitored for safety and efficacy for eight weeks, with an analysis at one, four and eight weeks. Patients from First-HD and ARC-HD Switch are eligible to roll into our ARC-HD Rollover trial, which is a long-term safety study. These trials are being conducted at sites in the United States, Canada and Australia.

We have also initiated two clinical trials for the treatment of tardive dyskinesia that are being conducted at sites in the United States and Europe. The first trial, referred to as ARM-TD, is a placebo-controlled trial of

SD-809 in approximately 92 patients with tardive dyskinesia and we expect to announce topline data from this trial in mid-2015. The second trial, referred to as AIM-TD, is a placebo-controlled trial of SD-809 in approximately 200 patients with tardive dyskinesia and we expect to announce topline data from this trial in 2016. In addition to the Huntington's disease and tardive dyskinesia programs, we initiated an open-label preliminary efficacy, pharmacokinetic and safety Phase 1b clinical trial for the treatment of tics associated with Tourette syndrome. We plan to enroll approximately 20 subjects to evaluate preliminary efficacy and safety, in addition to studying the pharmacokinetics of SD-809 in adolescents. These additional data are expected to further support the dosing assumptions and design elements of a randomized, controlled trial in this patient population and accelerate the development of SD-809 in this indication. We plan to announce topline data from this trial by mid-2015.

Although we achieved positive results on the endpoints from our First-HD and ARC-HD Switch clinical trials, and even if we achieve positive results from our other ongoing or any future clinical trials, the FDA may disagree that the clinical trials are adequate to show safety or efficacy in the indication being sought or with our interpretation of the data and deem the results insufficient to demonstrate efficacy at the level required by the FDA for product approval. While we do not have any current plans to do so, it is possible that we may make modifications to the clinical trial protocols or designs of one or more of our ongoing clinical trials that delay enrollment or completion of such clinical trials and could delay regulatory approval of SD-809. Any failure to obtain approval for SD-809 on the timeline that we currently anticipate, or at all, would have a material and adverse impact on our business, prospects, financial condition and results of operations.

If the FDA does not conclude that SD-809 satisfies the requirements for the Section 505(b)(2) regulatory approval pathway, or if the requirements for approval of SD-809 under Section 505(b)(2) are not as we expect, the development and approval of SD-809 will likely take significantly longer, cost significantly more and entail significantly greater complexity and risks than anticipated, and in any case may not be successful.

We intend to seek FDA approval through the Section 505(b)(2) regulatory pathway for SD-809. Section 505(b)(2) of the Federal Food, Drug, and Cosmetic Act, or the FDCA, 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 and for which the applicant has not obtained a right of reference. In the case of SD-809, we intend to file a Section 505(b)(2) NDA that relies on certain of the FDA's prior findings of safety for the approved drug, tetrabenazine (marketed as Xenazine in the United States). Our ability to rely on certain of the FDA's safety findings with regard to tetrabenazine will depend on our ability to demonstrate to the FDA's satisfaction that the dose range of SD-809 employed in our Phase 3 program exposes patients to similar levels of key active metabolites as the approved dose range of tetrabenazine. In our initial Phase 1 clinical trial, the sum of two key metabolites more than doubled at an equi-milligram dose of SD-809 as tetrabenazine, indicating that SD-809 exposes patients to similar levels of active metabolites at half the dose of tetrabenazine. Even with such a showing, the FDA has indicated that controlled safety data for SD-809 is required. We have generated such controlled safety data for SD-809 in our First-HD trial, however there can be no assurance that the FDA will be satisfied with these data. With regard to efficacy, our First-HD trial is similar in design to the successful tetrabenazine clinical trial that, along with the confirmatory evidence from a second, failed clinical trial, provided the basis for the finding of efficacy of tetrabenazine. We have not discussed with the FDA specifically whether our First-HD trial alone is adequate to establish the efficacy of SD-809. We expect to discuss this matter along with the topline positive results from the First-HD trial at a pre-NDA meeting with the FDA; however, we can provide no assurance that the FDA will not require additional clinical trials of SD-809.

By pursuing the Section 505(b)(2) regulatory pathway for SD-809, our reliance on the prior findings of safety for Xenazine may require any approved labeling for SD-809 to include certain safety information that is included in the labeling of Xenazine. For example, we have completed a thorough QT study of SD-809 in healthy

subjects to further evaluate the potential effect of SD-809 on cardiac repolarization, specifically the QT-interval, and although the results are favorable, the trial design was not discussed with FDA and it is possible that the labeling for SD-809, if approved, may contain information pertaining to the Xenazine QT interval. Topline results from this thorough QT study demonstrated that at two different dose levels, SD-809 had no clinically significant effect on cardiac repolarization as assessed by the QT interval. A tetrabenazine arm was included for comparison and demonstrated an increase in QTc that is consistent with the effect reported in the FDA labeling for Xenazine. The clinical trial's assay sensitivity was established by the observation of characteristic QTc prolongation following dosing with moxifloxacin. If the FDA disagrees with our position that reliance on the safety data for Xenazine is appropriate, or if the data required for approval of our Section 505(b)(2) NDA are different than 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 SD-809 would likely substantially increase. Moreover, the inability to pursue the Section 505(b)(2) regulatory pathway could result in new competitive products reaching the market faster than SD-809, which could materially adversely impact our competitive position and prospects. Even if the Section 505(b)(2) regulatory pathway is deemed appropriate 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 may file citizens' petitions with the FDA in an attempt to persuade the FDA that our products, or the clinical trials 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).

SD-809 or our other product candidates may cause undesirable side effects or have other properties that may delay or prevent their regulatory approval or commercialization or limit their commercial potential.

Undesirable side effects caused by SD-809 or our other product candidates could cause us or regulatory authorities to interrupt, delay, suspend or terminate clinical trials and could result in a more restrictive label or the delay or denial of marketing approval by the FDA or other regulatory authorities. This, in turn, could limit or prevent us from commercializing SD-809, or our other product candidates, and generating revenues from their sale. While the topline data from the First-HD and ARC-HD Switch clinical trials showed a favorable safety and tolerability profile with low rates of adverse events, such as depression, somnolence, akathisia and anxiety, the most common adverse events observed in patients who received SD-809 were insomnia, somnolence, fatigue, diarrhea, irritability and dry mouth. In First-HD, there was one patient with two serious adverse events (cholecystitis and agitated depression) in the SD-809 group, and one patient with one serious adverse event (exacerbation of chronic obstructive pulmonary disease, or COPD) in the placebo group. The same patient experiencing the serious adverse events in the SD-809 group also reported suicidal ideation, which was not considered a serious adverse event. In ARC-HD Switch, one patient was hospitalized for pneumonia and another patient was hospitalized overnight for dehydration. Neither of these adverse events was considered related to study medication and both of these events resolved. In the ongoing open label ARC-HD Rollover trial, which is not a placebo controlled trial, one patient was briefly hospitalized as a precaution for worsening anxiety, depression and suicidal ideation, events considered possibly related to study medication. Another subject developed depression and suicidal ideation, events considered possibly related to study medication. A third subject was briefly hospitalized for dehydration and confusion, events considered not related to study medication. All of these adverse events resolved. Results from our ongoing or future clinical trials could reveal a high and unacceptable severity and prevalence of these or other side effects. In such an event, our trials could be suspended or terminated and the FDA or comparable foreign regulatory authorities could order us to cease further development of or deny approval of SD-809 or our other product candidates for their targeted indications. Further, such side effects could affect patient recruitment or the ability of enrolled patients to

complete the trial or result in potential product liability claims. Any of these occurrences may have a material and adverse impact on our business, prospects, financial condition and results of operations.

In addition, if SD-809 or any of our other product candidates receives marketing approval and we or others later identify undesirable side effects caused by such product candidate, a number of significant negative consequences could result, including:

- the FDA may withdraw its approval of such product candidate;
- the FDA may require that we demonstrate a larger clinical benefit by conducting additional clinical trials for approval to offset the risk;
- the FDA may require the addition of labeling statements or warnings that could diminish the usage of the product or otherwise limit the commercial success of such product candidate;
- the FDA may make the requirements of any REMS more restrictive;
- we may be required to change the way such product candidate is administered;
- we may choose to recall, withdraw or discontinue sale of such product candidate;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any one or a combination of these events could prevent us from achieving or maintaining market acceptance of the affected product candidate or could substantially increase the costs and expenses of commercializing such product candidate, which in turn could delay or prevent us from generating any revenues from the sale of the product, which could significantly harm our business, prospects, financial condition and results of operations.

We anticipate that SD-809 will require a REMS which could delay the approval of SD-809 and increase the cost, burden and liability associated with the commercialization of SD-809.

The FDA Amendments Act of 2007 implemented safety-related changes to product labeling and provided the FDA with expanded authority to require the adoption of a REMS to assure safe use of the product candidates, either as a condition of product candidate approval or on the basis of new safety information. Given that tetrabenazine is subject to a REMS, we anticipate that approval of SD-809, if obtained, will be conditioned on the requirement to implement a REMS, and it is possible that our other product candidates may require a REMS. The REMS may include, among other things, medication guides for patients, special communication plans to health care professionals or elements to assure safe use, such as restricted distribution methods, patient registries and/or other risk minimization tools. We cannot predict the specific REMS to be required as part of the FDA's approval of our product candidates. While the topline data from the First-HD and ARC-HD Switch clinical trials showed a favorable safety and tolerability profile with low rates of depression, somnolence, akathisia, anxiety and suicidal ideation, the most common adverse events observed in patients who received SD-809 were insomnia, somnolence, fatigue, diarrhea, irritability and dry mouth. In First-HD, there was one patient with two serious adverse events (cholecystitis and agitated depression) in the SD-809 group, and one patient with one serious adverse event (exacerbation of COPD) in the placebo group. The same patient experiencing the serious adverse events in the SD-809 group also reported suicidal ideation, which was not considered a serious adverse event. In ARC-HD Switch, one patient was hospitalized for pneumonia and another patient was hospitalized overnight for dehydration. Neither of these adverse events was considered related to study medication and both of these events resolved. In the ongoing open label ARC-HD Rollover trial which is not a placebo controlled trial, one patient was briefly hospitalized as a precaution for worsening anxiety, depression and suicidal ideation, events considered possibly related to study medication. Another subject

developed depression and suicidal ideation, events considered possibly related to study medication. A third subject was briefly hospitalized for dehydration and confusion, events considered not related to study medication. All of these adverse events resolved. We would likely expect the elements of the REMS for SD-809, if approved, to be similar to the REMS for tetrabenazine, which includes a communication plan to healthcare providers to provide information about the increased risk of drug-associated depression and suicidality, proper titration and dosing, and the risk of drug-drug interactions with strong CYP2D6 inhibitors in patients taking the drug and the need to test for CYP2D6 enzyme activity. Any limitations on approval or marketing could restrict the commercial promotion, distribution, prescription or dispensing of our product candidates, if approved. Depending on the extent of the REMS requirements, these requirements may significantly increase our costs to commercialize these product candidates. Furthermore, risks of our product candidates that are not adequately addressed through proposed REMS for such product candidates may also prevent or delay their approval for commercialization.

We may experience delays in the commencement or completion of our clinical trials, which could result in increased costs to us and delay our ability to pursue regulatory approval and generate product revenues.

Delays in the commencement or completion of clinical trials could significantly impact our product development costs and could result in the need for additional financing. We do not know whether our ongoing and planned clinical trials of SD-809 will be completed on time, or at all, or whether any clinical trials will need to be redesigned, enroll patients on time or be completed on schedule, if at all. The commencement or completion of clinical trials can be delayed for a variety of reasons, including delays in or related to:

- raising sufficient capital to fund the clinical trials;
- obtaining regulatory feedback on trial design necessary to commence a clinical trial;
- identifying, recruiting and training suitable clinical investigators;
- identifying, recruiting and enrolling suitable patients to participate in a clinical 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 clinical trial sites;
- obtaining sufficient quantities of drug product for use in clinical trials;
- obtaining institutional review board, or IRB, approval to conduct a clinical trial at a prospective site;
- adding new clinical trial sites;
- clinical trial sites deviating from trial protocol or dropping out of a trial;
- failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols;
- failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions;
- unforeseen safety issues or any determination that a clinical trial presents unacceptable health risks;
- retaining patients who have initiated a clinical trial but may withdraw due to adverse side effects from the therapy, insufficient efficacy, fatigue with the clinical trial process or personal issues; and
- catastrophic loss of drug product due to shipping delays or delays in customs in connection with delivery of drug product to or from foreign countries for use in clinical trials.

In addition, the FDA may also put a clinical trial on clinical hold at any time during product candidate development.

Patient enrollment, a significant factor in the timing of clinical trials, 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, competing clinical trials and clinicians' and patients' perceptions as to the potential benefits of the product candidate being studied in relation to other available therapies, including any new therapies that may be approved for the indications we are investigating. Our ongoing AIM-TD clinical trial of SD-809 for the treatment of tardive dyskinesia will seek to enroll significantly more patients than we have enrolled in any single clinical trial of SD-809 to date and we may not be able to do so successfully. Furthermore, we rely on CROs and clinical trial sites to ensure the proper and timely conduct of our clinical trials and, while we have agreements governing their committed activities, we have limited influence over their actual performance.

We could encounter delays if physicians encounter unresolved ethical issues associated with enrolling patients in clinical trials of our product candidates in lieu of prescribing existing treatments that have established safety and efficacy profiles. Principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive cash compensation in connection with such services. If these relationships exceed certain financial thresholds, they must be reported to the FDA at the time of NDA submission. Such payments made to any investigator or the investigator's institution that exceeds $25,000 during the time the clinical investigator is carrying out the study and for one year following completion of the study must be reported to the FDA at the time of NDA submission. In addition, disclosable financial interests include: (1) any compensation to the investigator by the sponsor in which the value could be affected by study outcome; (2) a proprietary interest in the tested product; and (3) any equity interest in the sponsor of the covered clinical study, including any ownership interest, stock options, or other financial interest whose value cannot be readily determined through reference to public prices. In addition to disclosing the financial interest of an investigator, the NDA applicant must describe any steps taken to minimize the risk of bias, which could include factors such as multiple study sites, the use of appropriate blinding and randomization procedures, and the assessment of objective study points. We expect to disclose a financial arrangement, including a grant to an investigator's institution, for at least one investigator and submit this information in our NDA. In addition, individuals associated with our CROs or any other entity that manages or is involved with our clinical trials, including principal investigators, may serve as consultants to us from time to time and receive cash compensation in connection with such services. If these relationships and any related compensation to the clinical investigator carrying out the study, or to any entity that manages or is involved with our clinical trials or to individuals associated with that entity, result in perceived or actual conflicts of interest, or the FDA concludes that the financial relationship may have affected interpretation of the study, the integrity of the data generated at the applicable clinical trial site may be questioned and the utility of the clinical trial itself may be jeopardized, which could result in the delay or rejection of our NDA by the FDA. Any such delay or rejection could prevent us from commercializing SD-809.

Further, we could encounter delays if a clinical trial is suspended or terminated by us, the IRBs in the institutions in which such trials are being conducted, the Data Safety Monitoring Board for such trial, or by the FDA or other regulatory authorities 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 our product candidate, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. If we experience termination of, or delays in the completion of, any clinical trial of our product candidate, the commercial prospects of our product candidate will be harmed, and our ability to generate product revenues will be delayed. In addition, any delays in completing our clinical trials will increase our costs, slow down our product development and approval process and jeopardize our ability to commence product sales and generate revenues. Any of these occurrences may harm our business, prospects, financial condition

and results of operations significantly. Furthermore, 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.

Changes in regulatory requirements and guidance also may occur and we may need to amend clinical trial protocols to reflect these changes. Amendments may require us to resubmit our clinical trial protocols to IRBs for re-examination, which may impact the costs, timing and successful completion of a clinical trial. If we experience delays in the completion of, or if we must terminate, any clinical trial of SD-809, our ability to obtain regulatory approval will be delayed and the commercial prospects, if any, for SD-809 may be harmed. If we ultimately commercialize SD-809 or any of our other product candidates, other therapies for the same indications may have been introduced to the market during the period we have been delayed and such therapies may have established a competitive advantage over our product candidates.

We may not obtain orphan drug exclusivity for any of our product candidates.

Our business strategy focuses on the development and commercialization of novel medicines for the treatment of orphan diseases. In the United States, under the Orphan Drug Act, the FDA may grant orphan designation to a drug intended to treat a rare disease or condition. Such diseases and conditions are those that affect fewer than 200,000 individuals in the United States or, if they affect more than 200,000 individuals in the United States, there is no reasonable expectation that the cost of developing and making a drug product available in the United States for these types of diseases or conditions will be recovered from sales of the product. Orphan drug designation must be requested before submitting an NDA. The applicant will not be required to pay the NDA fee if the orphan designation has been granted, but will be required to pay the NDA fee if the designation is still pending at the time the NDA is submitted, although the FDA could still grant a waiver of the application fee for the first drug application as a small business (section 736(d) of the FDCA) if the criteria for a small business are met. If the FDA grants orphan drug designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by that agency. Orphan drug designation does not convey any advantage in or shorten the duration of the regulatory review and approval process, but it can lead to financial incentives, such as opportunities for grant funding toward clinical trial costs, tax advantages in-lieu of R&D tax credits and user-fee waivers. If a product that has orphan drug designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan drug marketing exclusivity for a period of seven years. Orphan drug marketing exclusivity generally prevents the FDA from approving another application, including a full NDA, to market the same drug for the same indication for seven years, except in limited circumstances, including if the FDA concludes that the later drug is safer, more effective or makes a major contribution to patient care. The FDA defines “same drug” as a drug that contains the same active chemical entity and is intended for the same use as the drug in question. A designated orphan drug may not receive orphan drug marketing exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation or if a third party first obtains approval of the same drug for the orphan designated indication (unless FDA concludes that the later drug is safer, more effective or makes a major contribution to patient care). Orphan drug marketing exclusivity rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition. We received an orphan drug designation of SD-809 for the treatment of Huntington’s disease from the FDA in November 2014, and an orphan drug designation of SD-809 for the treatment of Tourette syndrome in the pediatric population (defined as zero through 16 years of age) from the FDA in January 2015.

We face significant competition from other pharmaceutical and biotechnology companies, and our operating results will suffer if we fail to compete effectively.

The biotechnology and pharmaceutical industries are intensely competitive and subject to rapid and significant technological change. We have competitors both in the United States and international markets, including major multinational pharmaceutical companies, biotechnology companies and universities and other research institutions. Many of our competitors have substantially greater financial, technical and other resources, such as larger research and development staff, experienced marketing and manufacturing organizations and well-established sales forces. Additional mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated in our competitors. Competition may increase further 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, easier to administer or less costly than SD-809 or our other product candidates.

We anticipate that, if approved, SD-809 will compete primarily against Xenazine and, potentially in the future, generic tetrabenazine for the treatment of chorea associated with Huntington's disease. In addition, there are several product candidates in clinical development for the treatment of Huntington's disease. These include Huntexil (prodipidine) and Nerventra (laquinimod), which are both being developed by Teva Pharmaceutical Industries; PBT2, which is being developed by Prana Biotechnology Ltd.; SEN0014196 (selisistat), which is being developed by Siena Biotech S.p.A.; Procysbi (cysteamine), which is approved for the treatment of nephropathic cystinosis and is being developed for Huntington's disease by Raptor Pharmaceuticals, Inc.; OMS824, which is being developed by Omeros Corporation; PF-2545920, which is being developed by Pfizer Inc. and BN82451 which is being developed by Ipsen.

There are currently no approved drugs for the treatment of tardive dyskinesia, but we believe that tetrabenazine is prescribed off-label for this indication. We are aware of several product candidates in clinical development for treatment of tardive dyskinesia including: NBI-98854 (valine ester substituted analog of a single stereoisomer of alpha), which is being developed by Neurocrine Biosciences Inc.; SNC-102 (acamprosate calcium), which is being developed by Synchroneuron Inc.; and Tardoxal (pyridoxine hydrochloride), which is being developed by Medicure Inc.

There are currently two FDA-approved drugs for the treatment of Tourette syndrome: haloperidol and pimozide, which are generic neuroleptics. These drugs were approved for this indication by the FDA in 1967 and 1983, respectively. We believe that tetrabenazine and guanfacine are prescribed off-label for this indication as well. We are aware of several product candidates in clinical development for the treatment of Tourette syndrome including: ABILIFY (aripiprazole), which is being developed by Otsuka Pharmaceutical Group; NBI-98854 (a valine ester-substituted analog of alpha), which is being developed by Neurocrine Biosciences, Inc.; ecopipam (a synthetic benzazepine derivative), which is being developed by Psyadon Pharmaceuticals Inc.; AZD5213, which is being developed by AstraZeneca plc; CPP-109, which is being developed by Catalyst Pharmaceutical Partners; and EPI-754, which is being developed by Edison Pharmaceuticals, Inc.

The availability and price of our competitors' products could limit the demand, and the price we are able to charge, for SD-809 or our other product candidates. We may not be able to successfully execute on our business objectives if the market acceptance of SD-809 or our other product candidates is inhibited by significant price competition from Xenazine, or any generic tetrabenazine that may be available in the future, or if physicians are reluctant to switch from existing products to SD-809 or our other product candidates, or if physicians switch to other new products or choose to reserve SD-809 or our other product candidates for use in limited patient populations. Xenazine is expected to lose its market exclusivity related to its orphan drug status for the treatment of chorea associated with Huntington's disease in August 2015 and generic versions of

tetrabenazine may potentially be introduced into the market after such time. In addition, established pharmaceutical companies may invest heavily to accelerate discovery and development of novel compounds or to in-license and develop novel compounds that could make SD-809 or our other product candidates obsolete.

While comparative safety or efficacy are not required for FDA approval, and we do not intend to test SD-809 against Xenazine in our ongoing Phase 3 clinical trials, 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, obtaining FDA approval or discovering, developing and commercializing products before we do, which would have a material adverse impact on our business. The inability to compete with existing products or subsequently introduced products would have a material adverse impact on our business, prospects, financial condition and results of operations.

Even if we receive regulatory approval for SD-809 or any future product candidates, 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.

Any regulatory approvals that we receive for our product candidates will contain approved indicated uses, and we will be required to market any approved products in accordance with the indicated uses and our approved labeling. In addition, any regulatory approvals may contain conditions for approval or requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the safety and efficacy of the product candidate. In addition, if the FDA or a comparable foreign regulatory authority approves any of our product candidates, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion, import, export and recordkeeping for the product will be subject to extensive and ongoing regulatory requirements. These requirements include submissions of safety and other post-marketing information and reports, registration, as well as continued compliance with current good manufacturing practices, or cGMPs, and current good clinical practices, or cGCPs, for any clinical trials that we conduct post-approval. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in, among other things:

- restrictions on the marketing or manufacturing of the product, withdrawal of the product from the market, or voluntary or mandatory product recalls;
- fines, warning or untitled letters or holds on clinical trials;
- refusal by the FDA to approve pending applications or supplements to approved applications filed, or suspension or revocation of product approvals;
- product seizure or detention, or refusal to permit the import or export of products; and
- injunctions, the imposition of civil penalties or criminal prosecution.

We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. If we are not able to maintain regulatory compliance or if we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, regulatory sanctions may be applied or 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.

We rely on third parties to conduct our clinical trials. If these third parties do not successfully carry out their contractual 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 agreements with third-party CROs to conduct or monitor and manage data for our ongoing clinical programs, including SD-809. We rely heavily on these parties for execution of our clinical trials, and we control only certain aspects of their activities. Nevertheless, we are responsible for ensuring that each of our trials is conducted in accordance with applicable protocol, legal, regulatory and scientific standards, and our reliance on our CROs does not relieve us of our regulatory responsibilities. We and our CROs are required to comply with federal regulations and cGCPs, which are guidelines enforced by the FDA and comparable foreign regulatory authorities for all of our product candidates in clinical development. Regulatory authorities enforce these cGCPs through periodic inspections of trial sponsors, principal investigators and trial sites. If we or any of these CROs fail to comply with applicable regulations and cGCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA 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, such regulatory authorities will determine that any of our clinical trials comply with the applicable regulations and cGCPs. In addition, our clinical trials must be conducted with drug product produced under applicable regulations and cGMP and will require a large number of trial subjects. Our or our respective CROs' failure to comply with these regulations or to recruit a sufficient number of patients may require us to repeat clinical trials, which would delay the regulatory approval process. Moreover, our business may be implicated if any of our CROs violate federal or state fraud and abuse or false claims laws and regulations or healthcare privacy and security laws.

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 ongoing preclinical, clinical and nonclinical programs. These CROs 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, which could harm our competitive position. If our 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 or regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated and we may not be able to complete development of, obtain regulatory approval for or successfully commercialize our product candidates. As a result, our financial results and the commercial prospects for SD-809 and other future product candidates would be harmed, our costs could increase and our ability to generate revenues could be delayed.

Switching or adding CROs involves substantial cost and requires extensive 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, prospects, financial condition and results of operations.

We rely on third parties to manufacture supplies of SD-809, and we intend to rely on third parties to manufacture commercial supplies of SD-809, if and when it is approved. The development and commercialization of SD-809 could be stopped or delayed if any such third party fails to provide us with sufficient quantities of product or fails to do so at acceptable quality levels or prices or fails to maintain or achieve satisfactory regulatory compliance.

We do not currently have nor do we plan to acquire the infrastructure or capability internally to manufacture our clinical drug supplies for use in the conduct of our clinical trials, and we lack the resources and the

capability to manufacture SD-809 or our other product candidates on a clinical or commercial scale. Instead, we rely on our third-party manufacturing partners for the production of the active pharmaceutical ingredient, or API, and drug formulation of SD-809. The facilities used by our third-party manufacturers to manufacture SD-809 and any other potential product candidates that we may develop in the future must be successfully inspected by the applicable regulatory authorities, including the FDA, after we submit our NDA to the FDA. We are currently completely dependent on our third-party manufacturers for the production of SD-809 in accordance with cGMPs, which include, among other things, quality control, quality assurance and the maintenance of records and documentation.

Although we have entered into an agreement for the manufacture of clinical supplies and initial commercial supplies of SD-809, our third-party manufacturers may not perform as agreed, may be unable to comply with these cGMP requirements and with FDA, state and foreign regulatory requirements or may terminate its agreement with us. If any of our third-party manufacturers cannot successfully manufacture material that conforms to our specifications and the applicable regulatory authorities' strict regulatory requirements, or pass regulatory inspection, our NDA will not be approved. In addition, although we are ultimately responsible for ensuring product quality, we have no direct day-to-day control over our third-party manufacturers' ability to maintain adequate quality control, quality assurance and qualified personnel. If our third-party manufacturers are unable to satisfy the regulatory requirements for the manufacture of our products, or if our suppliers or third-party manufacturers decide they no longer want to manufacture our products, we may need to find alternative manufacturing facilities, which would be time-consuming and significantly impact our ability to develop, obtain regulatory approval for or market our products. We might be unable to identify manufacturers for long-term commercial supply on acceptable terms or at all. Manufacturers are subject to ongoing periodic announced and unannounced inspection by the FDA and other governmental authorities to ensure compliance with government regulations. Currently, our contract manufacturer for the API for SD-809 is located outside the United States and the FDA has recently increased the number of foreign drug manufacturers that it inspects as well as the frequency of such inspections. As a result, our third-party manufacturers may be subject to increased scrutiny.

If we were to experience an unexpected loss of SD-809 supply for clinical development or commercialization, we could experience delays in our ongoing or planned clinical trials as our third-party manufacturers would need to manufacture additional SD-809 and we may not be able to provide sufficient lead time to enable our third-party manufacturers to schedule a manufacturing slot, or to produce the necessary replacement quantities. This could result in delays in progressing our clinical development activities and achieving regulatory approval for our products, which could materially harm our business.

The manufacture of pharmaceutical products is complex and requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. We and our contract manufacturers must comply with cGMP regulations and guidelines. Manufacturers of pharmaceutical products often encounter difficulties in production, particularly in scaling up and validating initial production. These problems include difficulties with production costs and yields, quality control, including stability of the product, quality assurance testing, operator error, shortages of qualified personnel, as well as compliance with strictly enforced federal, state and foreign regulations. Furthermore, if microbial, viral or other contaminations are discovered in our products or in the manufacturing facilities in which our products are made, such manufacturing facilities may need to be closed for an extended period of time to investigate and remedy the contamination. We cannot assure you that any stability or other issues relating to the manufacture of any of our products will not occur in the future. Additionally, our manufacturers may experience manufacturing difficulties due to resource constraints or as a result of labor disputes or unstable political environments. If our manufacturers were to encounter any of these difficulties, or otherwise fail to comply with their contractual obligations, our ability to provide any product candidates to patients in clinical trials would be

jeopardized. Any delay or interruption in the supply of clinical trial supplies could delay the completion of clinical trials, increase the costs associated with maintaining clinical trial programs and, depending upon the period of delay, require us to commence new clinical trials at additional expense or terminate clinical trials completely.

Any adverse developments affecting clinical or commercial manufacturing of our products may result in shipment delays, inventory shortages, lot failures, product withdrawals or recalls, or other interruptions in the supply of our products or product candidates. We may also have to take inventory write-offs and incur other charges and expenses for products or product candidates that fail to meet specifications, undertake costly remediation efforts or seek more costly manufacturing alternatives. Accordingly, failures or difficulties faced at any level of our supply chain could materially adversely affect our business and delay or impede the development and commercialization of any of our products or product candidates and could have a material adverse effect on our business, prospects, financial condition and results of operations.

We plan to rely on third-party specialty channels to distribute SD-809 to patients, to successfully commercialize SD-809, if approved. If we are unable to effectively establish and manage this distribution process, the commercial launch and sales of SD-809 may be delayed or compromised.

We plan to contract with and rely on third-party specialty pharmacies to distribute SD-809 to patients. A specialty pharmacy is a pharmacy that specializes in the dispensing of medications for complex or chronic conditions, which require a high level of patient education and ongoing management. This distribution network will require significant attention from our management team. If we are unable to effectively establish and manage this distribution process, the commercial launch and sales of SD-809 will be delayed or compromised and our results of operations may be harmed.

In addition, the use of specialty pharmacies involves certain risks, including, but not limited to, risks that these organizations will:

- not provide us with accurate or timely information regarding their inventories, the number of patients who are using SD-809, or complaints regarding SD-809;
- not effectively sell or support SD-809;
- reduce or discontinue their efforts to sell or support SD-809;
- not devote the resources necessary to sell SD-809 in the volumes and within the time frames that we expect;
- be unable to satisfy financial obligations to us or others; or
- cease operations.

Any such events may result in decreased sales and lower revenue, which could have a material adverse effect on our business, prospects, financial condition and results of operations.

Our ability to generate revenues from SD-809 or our other product candidates will be subject to attaining significant market acceptance among physicians, patients and healthcare payors.

Neither SD-809 nor any of our other product candidates, if approved, may attain market acceptance among physicians, patients, healthcare payors or the medical community. We believe that the degree of market acceptance and our ability to generate revenues from SD-809 and our other product candidates will depend on a number of factors, including:

- timing of market introduction of our products as well as competitive drugs;
- efficacy and safety of our product candidates;

- the clinical indication(s), if any, for which SD-809 or our other product candidates are approved;
- with respect to SD-809, the size of the markets for the treatment of chorea associated with Huntington's disease, tardive dyskinesia and Tourette syndrome;
- acceptance by patients, primary care specialists and key specialists, including neurologists and psychiatrists;
- potential or perceived advantages or disadvantages of SD-809 or our other product candidates over other alternative treatments, including cost of treatment and relative convenience and ease of administration and length of sustained benefits from treatment;
- strength of sales, marketing and distribution support;
- the price of our product candidates, both in absolute terms and relative to alternative treatments;
- the effect of current and future healthcare laws;
- availability of coverage and adequate reimbursement from government and other third-party payors;
- product labeling requirements of the FDA or other regulatory authorities; and
- the requirements of the REMS likely to be imposed by the FDA.

While we believe that the reduced interpatient variability and lower dosing frequency of SD-809 relative to Xenazine will allow us to differentiate SD-809 from Xenazine in the market, if approved, because we do not intend to conduct a Phase 3 clinical trial comparing SD-809 to Xenazine, we will not be able to make direct comparative claims regarding the safety of SD-809 and Xenazine either in labeling or in our promotional materials for SD-809 from such trials. If SD-809 or any of our other product candidates is approved but fails to attain market acceptance by physicians, health care payors, or patients, we may not be able to generate significant revenue to achieve or sustain profitability, which would have a material adverse effect on our business, prospects, financial condition and results of operations.

Coverage and reimbursement may not be available, or may be available at only limited levels, for SD-809 or our other product candidates, which could make it difficult for us to sell our product candidates profitably.

Market acceptance and sales of SD-809 or our other product candidates will depend in large part on global reimbursement policies and may be affected by future healthcare reform measures. Successful commercialization of SD-809 or our other product candidates will depend in part on the availability of governmental and third-party payor reimbursement for the cost of our product candidates. Government authorities, private health insurers and other organizations establish coverage and reimbursement policies for new products, including product candidates like SD-809. In particular, in the United States, the Medicare and Medicaid programs increasingly are used as models for how private payors and other governmental payors develop their coverage and reimbursement policies for drugs and other medical products and services, particularly for new and innovative products and therapies, which has resulted in lower average selling prices. Further, the increased emphasis on managed healthcare in the United States will put additional pressure on product pricing, coverage, reimbursement and utilization, which may adversely affect our product sales and results of operations. These pressures can arise from policies and practices of managed care groups, judicial decisions and governmental laws and regulations related to Medicare, Medicaid and healthcare reform, coverage and reimbursement policies and pricing in general.

In March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively, PPACA, became law in the United States. PPACA substantially changes the way healthcare is financed by both governmental and private insurers and significantly affects the pharmaceutical industry. Among the provisions of PPACA of greatest importance to the pharmaceutical

industry are the following: (1) an annual, nondeductible fee on any entity that manufactures or imports certain branded prescription drugs and biologic agents, apportioned among these entities according to their market share in certain government healthcare programs; (2) an increase in the rebates a manufacturer must pay under the Medicaid Drug Rebate Program to 23.1% and 13.0% of the average manufacturer price for branded and generic drugs, respectively; (3) extension of manufacturers' Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations; (4) expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to additional individuals and by adding new mandatory eligibility categories for certain individuals with income at or below 133% of the Federal Poverty Level in 2014, thereby potentially increasing manufacturers' Medicaid rebate liability; (5) expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program; (6) expansion of health care fraud and abuse laws, including the False Claims Act and the Anti-Kickback Statute, new government investigative powers, and enhanced penalties for noncompliance; (7) a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts to negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D; and (8) a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

In addition, other legislative changes have been proposed and adopted in the United States since PPACA was enacted. On August 2, 2011, the Budget Control Act of 2011 among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions of Medicare payments to providers of 2% per fiscal year, which went into effect on April 1, 2013 and will stay in effect through 2024 unless additional Congressional action is taken. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors.

We expect to experience pricing pressures in connection with the sale of SD-809 and our other product candidates, if approved, and any other products that we may develop, due to the trend toward managed healthcare, the increasing influence of health maintenance organizations and additional legislative proposals. If we fail to successfully secure and maintain adequate coverage and reimbursement for our products or are significantly delayed in doing so, we will have difficulty achieving market acceptance of our products and expected revenue and profitability which would have a material adverse effect on our business, prospects, financial condition and results of operations.

We may also be subject to healthcare laws, regulation and enforcement and our failure to comply with those laws could have a material adverse effect on our results of operations and financial conditions.

Although we currently do not have any products on the market, if SD-809 or any future product candidates are approved, once we begin commercializing our products, we may be subject to additional healthcare regulation and enforcement by the federal government and the states and foreign governments in which we conduct our business. The laws that may affect our ability to operate include:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and willfully soliciting, receiving, offering or paying remuneration, directly or indirectly, to induce, or in return for, the purchase or recommendation of an item or service reimbursable under a federal healthcare program, such as the Medicare and Medicaid programs;

- federal civil and criminal false claims laws and civil monetary penalty laws, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment from Medicare, Medicaid, or other third-party payors that are false or fraudulent;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created new federal criminal statutes that prohibit executing a scheme to defraud any healthcare benefit program and making false statements relating to healthcare matters;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act and its implementing regulations, which imposes certain requirements relating to the privacy, security, and transmission of individually identifiable health information;
- the federal Physician Payment Sunshine Act, which requires certain manufacturers of drugs, devices, biologics, and medical supplies to report annually to the Centers for Medicare & Medicaid Services information related to payments and other transfers of value to physicians, other healthcare providers, and teaching hospitals, and ownership and investment interests held by physicians and other healthcare providers and their immediate family members;
- state law equivalents of each of the above federal laws, such as anti-kickback and false claims laws that may apply to items or services reimbursed by any third-party payor, including commercial insurers;
- state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the applicable compliance guidance promulgated by the federal government, or otherwise restrict payments that may be made to healthcare providers and other potential referral sources;
- state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures;
- state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts;
- state laws governing pharmaceutical distribution that require application and registration with state boards of pharmacy; and
- state requirements related to cGMP inspections of pharmaceutical manufacturing facilities operating within their state.

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. In addition, recent health care reform legislation has strengthened these laws. For example, PPACA, among other things, amends the intent requirement of the federal anti-kickback and criminal healthcare fraud statutes. A person or entity no longer needs to have actual knowledge of the statute or specific intent to violate it. Moreover, PPACA 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 False Claims Act.

If our operations are found to be in violation of any of such laws or any other governmental regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, the curtailment or restructuring of our operations, the exclusion from participation in federal and state healthcare programs and imprisonment, any of which could adversely affect our ability to operate our business and our financial results.

If we are unable to establish sales and marketing capabilities, we may not be able to effectively market and sell our products and generate product revenue.

We are developing SD-809 for specific patient populations served by neurologists as well as psychiatrists. We do not currently have an organization for the sale, marketing or distribution of SD-809 or any of our other product candidates and we must build this organization, or enter into a marketing collaboration with a third party, in order to commercialize SD-809 and any future product candidates. We intend to establish an initial internal specialty sales force to sell SD-809, if approved, for the treatment of chorea associated with Huntington's disease. In addition, we intend to enter into contractual relationships with specialty pharmacies for the distribution of SD-809, if approved. We may partner with third parties to commercialize SD-809 if it is approved for other indications, including tardive dyskinesia and Tourette syndrome.

The establishment and development of our own sales force in the United States to market SD-809 will be expensive and time consuming and could delay any product launch, and we cannot be certain that we would be able to successfully develop this capacity. If we are unable to establish our sales and marketing capability or any other non-technical capabilities necessary to commercialize any products we may develop, we will need to contract with third parties to market and sell such products in the United States. We currently possess limited resources and may not be successful in establishing our own internal sales force or in establishing arrangements with third parties, including with specialty pharmacies, on acceptable terms, if at all.

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, 2014, we had 35 full-time employees. In addition, we have engaged part-time employees and individual consultants to assist us with a number of activities, including finance, clinical, regulatory and quality. We will need to expand our managerial, operational, financial and other resources in order to manage our operations and clinical trials, continue our research and development activities, commercialize SD-809, if approved, and transition to operating as a public company. Our management and scientific personnel, systems and facilities currently in place may not be adequate to support this expected growth. Our need to effectively manage our operations, growth and various projects requires that we:

- manage our clinical trials effectively, including our three ongoing clinical trials of SD-809;
- manage our internal development efforts effectively while carrying out our contractual obligations to 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.

Because we rely on numerous consultants, effectively outsourcing many key functions of our business, we will 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 candidates 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 candidates and, accordingly, may not achieve our research, development and commercialization goals.

If we are not successful in attracting and retaining highly qualified personnel, we may not be able to successfully implement our business strategy.

Our ability to compete in the highly competitive biotechnology and pharmaceuticals industries depends upon our ability to attract and retain highly qualified managerial, scientific and medical personnel. In order to retain valuable employees and consultants at our company, in addition to salary and other cash incentives, we provide incentive stock options and restricted stock grants that vest over time. The value to employees and consultants of stock options and restricted stock grants that vest over time will be significantly affected by movements in our stock price that are beyond our control, and may at any time be insufficient to counteract more lucrative offers from other companies.

Our scientific team in particular has expertise in many different aspects of drug discovery and development and may be difficult to retain or replace. We conduct our operations at our facilities in San Diego, California and this region is headquarters to many other pharmaceutical companies and many academic and research institutions and therefore we face increased competition for personnel in this location. Competition for skilled personnel in our market is very intense and competition for experienced scientists may limit our ability to hire and retain highly qualified personnel on acceptable terms.

Despite our efforts to retain valuable employees, members of our management and scientific and development teams may terminate their employment with us on short notice. Although we have written employment arrangements with our employees, these employment arrangements provide for at-will employment, which means that our employees can leave our employment at any time, with or without notice. The loss of the services of any of our executive officers or other key employees and our inability to find suitable replacements could potentially harm our business, financial condition and prospects. We do not maintain "key man" insurance policies on the lives of these individuals or the lives of any of our other employees.

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and management resources, we focus on a limited number of research programs and product candidates and are currently focused principally on SD-809. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial drugs or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable drugs. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through future collaboration, licensing or other arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights.

The terms of our term loan require us to meet certain operating and financial covenants and place restrictions on our operating and financial flexibility. If we raise additional capital through debt financing, the terms of any new debt could further restrict our ability to operate our business.

In December 2013, we entered into a term loan facility with Oxford Finance LLC, or Oxford, and its assignees, collectively referred to as the lenders, for an aggregate amount of $15.0 million, which was funded at closing, and in December 2014, we amended the facility to extend the interest-only payment period. Our obligations under the term loan facility are secured, subject to customary permitted liens and other agreed upon exceptions, by perfected first priority interest in substantially all of our tangible personal property, excluding

our intellectual property. Our intellectual property is subject to a negative pledge. $5.0 million of the proceeds from the term loan were used to repay our Square 1 credit facility. The term loan bears interest at a fixed rate equal to 8.99% per annum and matures on January 1, 2018. We have been making interest-only payments pursuant to the terms of the loan facility and, pursuant to the terms of the December 2014 amendment, we are required to make a total of 24 monthly interest only payments through January 1, 2016 followed by 24 equal monthly payments against the outstanding principal and interest. However, if we complete an NDA submission of SD-809 for chorea associated with Huntington's disease, and we are otherwise in compliance with the loan facility, as of June 30, 2015, the interest only period will extended to 30 months, followed by 18 equal monthly payments against the outstanding principal and interest. Upon repayment of the term loan, we are required to make a final payment to the lender equal to 3% of the original amount of the term loan.

The loan and security agreement governing the credit facility contains customary affirmative and negative covenants, indemnification provisions and events of default. The affirmative covenants include, among others, covenants requiring us to maintain our legal existence and governmental approvals, deliver certain financial reports and maintain insurance. The negative covenants include, among others, restrictions on dispositions, changes in business, management, ownership or business locations, mergers or acquisitions, indebtedness, encumbrances, distributions, investments, transactions with affiliates and subordinated debt. If we default under the credit facility, Oxford may accelerate all of our repayment obligations and take control of our pledged assets, potentially requiring us to renegotiate our agreement on terms less favorable to us or to immediately cease operations. Further, if we are liquidated, Oxford's right to repayment would be senior to the rights of the holders of our common stock to receive any proceeds from the liquidation. Oxford could declare a default under the credit facility upon the occurrence of an event of default, which includes any event that Oxford interprets as a material adverse change as defined under the loan and security agreement, thereby requiring us to repay the loan immediately or to attempt to reverse the declaration of default through negotiation or litigation. Any declaration by Oxford of an event of default could significantly harm our business and prospects and could cause the price of our common stock to decline. If we raise any additional debt financing, the terms of such additional debt could further restrict our operating and financial flexibility.

Our limited operating history makes evaluating our business and future prospects difficult, and may increase the risk of any investment in our common stock.

We were formed as a California corporation in February 2001. In June 2007 we reincorporated in Delaware. Our operations to date have been limited to developing SD-809 and our other product candidates. In addition, as an early stage company, we have limited experience and have not yet demonstrated an ability to successfully overcome many of the risks and uncertainties frequently encountered by companies in new and rapidly evolving fields, particularly in the pharmaceutical area. Nor have we demonstrated an ability to obtain regulatory approval for or to commercialize a product candidate. Consequently, any predictions about our future performance may not be as accurate as they could be if we had a history of successfully developing and commercializing a significant number of pharmaceutical products.

Our business and operations would suffer in the event of system failures.

Despite the implementation of security measures, our internal computer systems and those of our current and any future CROs and other contractors and consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While we have not experienced any such material system failure, accident or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our development programs and our business operations. 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 rely on third parties to manufacture SD-809 and conduct

clinical trials, and similar events relating to their computer systems 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.

Business interruptions could seriously harm our future revenue and financial condition and increase our costs and expenses.

Our operations could be subject to earthquakes, power shortages, telecommunications failures, systems failures, water shortages, floods, hurricanes, typhoons, fires, extreme weather conditions, medical epidemics and other natural or man-made disasters or business interruptions. The occurrence of any of these business interruptions could seriously harm our business and financial condition and increase our costs and expenses. Our management operates in our principal executive offices located in San Diego, California. If our offices were affected by a natural or man-made disaster, particularly those that are characteristic of the region, such as wildfires and earthquakes, or other business interruption, our ability to manage our domestic and foreign operations could be impaired, which could materially and adversely affect our results of operations and financial condition. We currently rely, and intend to rely in the future, on our third-party manufacturers, to produce our supply of SD-809. Our ability to obtain supplies of SD-809 could be disrupted, and our results of operations and financial condition could be materially and adversely affected if the operations of our third-party manufacturers were affected by a man-made or natural disaster or other business interruption. The ultimate impact of such events on us, our significant suppliers and our general infrastructure is unknown.

Our business involves the use of hazardous materials, and we and our third-party manufacturers must comply with environmental laws and regulations, which can be expensive and restrict how we do business.

Our third-party manufacturers' activities involve the controlled storage, use and disposal of hazardous materials owned by us, including the components of SD-809 and other hazardous compounds. We and our manufacturers are subject to federal, state and local as well as foreign laws and regulations governing the use, manufacture, storage, handling and disposal of these hazardous materials. Although we believe that the safety procedures utilized by our third-party manufacturers for handling and disposing of these materials comply with the standards prescribed by these laws and regulations, we cannot eliminate the risk of accidental contamination or injury from these materials. In the event of an accident, state, federal or foreign authorities may curtail the use of hazardous materials and interrupt our business operations. We do not currently maintain hazardous materials insurance coverage. If we are subject to any liability as a result of our third-party manufacturers' activities involving hazardous materials, our business and financial condition may be adversely affected. In the future we may seek to establish longer term third-party manufacturing arrangements, pursuant to which we would seek to obtain contractual indemnification protection from such third-party manufacturers potentially limiting this liability exposure.

If product liability lawsuits are brought against us, we may incur substantial liabilities and may be required to limit commercialization of our products.

We face an inherent risk of product liability as a result of the clinical trials and, if approved, the commercialization of SD-809 or our other product candidates. For example, we may be sued if any product we develop allegedly causes injury or is found to be otherwise unsuitable during clinical trials, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability or a breach of warranties. Claims could also be asserted under state or foreign consumer protection acts. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of our product candidate. Even a successful defense would require significant

financial and management resources. Regardless of the merits or eventual outcome, liability claims may result in:

- decreased demand for SD-809 or other product candidates that we may develop in the future;
- injury to our reputation;
- withdrawal of clinical trial participants;
- initiation of investigations by regulators;
- costs to defend the related litigation;
- a diversion of management's time and our resources;
- substantial monetary awards to clinical trial participants or patients;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;
- loss of revenue;
- exhaustion of any available insurance and our capital resources;
- the inability to commercialize our products or product candidates; and
- a decline in our stock price.

Our inability to obtain and retain sufficient product liability insurance at an acceptable cost to protect against potential product liability claims could prevent or inhibit the commercialization of products we develop. We currently carry product liability insurance covering our clinical trials in the amount of $5.0 million in the aggregate. Although we maintain such insurance, 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. If we determine that it is prudent to increase our product liability coverage due to the commercial launch of SD-809 or any of our other product candidates, we may be unable to obtain such increased coverage on acceptable terms or at all. Our insurance policies also have various exclusions, and we may be subject to a product liability claim for which we have no coverage. 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.

Our employees, independent contractors, principal investigators, CROs, consultants and vendors 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 that our employees, independent contractors, principal investigators, CROs, consultants and vendors may engage in fraudulent conduct or other illegal activity. Misconduct by these parties could include intentional, reckless and/or negligent conduct or disclosure of unauthorized activities to us that violates: (1) FDA regulations, including those laws requiring the reporting of true, complete and accurate information to the FDA, (2) manufacturing standards, (3) federal and state healthcare fraud and abuse laws and regulations, or (4) laws that require the reporting of financial information or data accurately. Specifically, 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, sales commission, customer incentive programs and other business arrangements. Activities subject to these laws 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 and ethics, but it is not always possible to identify and deter misconduct by employees and other third parties, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a

significant impact on our business, including the imposition of civil, criminal and administrative penalties, damages, monetary fines, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

Risks related to our financial position and capital requirements

We have incurred significant operating losses since our inception and anticipate that we will continue to incur losses for the foreseeable future and may never be profitable.

We are a development stage company with limited operating history. To date, we have focused primarily on developing SD-809 and our other product candidates. SD-809 will require substantial additional development time and resources before we will be able to receive regulatory approvals, implement commercialization strategies and begin generating revenue from product sales, as will our other product candidates, and there can be no assurance that any of our product candidates will ever achieve regulatory approval or generate any revenue. We do not know when we will generate any revenue, if ever, from sales of SD-809 or any of our other product candidates. We have incurred losses each year since our inception, including net losses of $15.6 million and $15.1 million for the years ended December 31, 2013 and 2012, respectively. Moreover, for the nine months ended September 30, 2014, our net loss is $39.5 million and as of September 30, 2014, we had an accumulated deficit of $105.0 million.

We have devoted most of our financial resources to product development. To date, we have financed our operations primarily through the sale of equity and debt securities. The size of our future net losses will depend, in part, on the rate of future expenditures and our ability to generate revenue. To date, we do not have any product candidates that have been commercialized, and if SD-809 is not successfully developed or commercialized in either Huntington's disease, tardive dyskinesia or Tourette syndrome, or if none of our other product candidates are successfully developed or commercialized, or if revenue is insufficient following marketing approval, we may not achieve profitability and our business may fail.

Because of the numerous risks and uncertainties associated with pharmaceutical product development, we are unable to fully predict the timing or amount of our increased expenses, but we expect to continue to incur substantial expenses, which we expect to increase as we expand our development activities and build a specialty sales force and commercialization infrastructure. Our expenses could increase beyond expectations if we are required by the FDA to perform studies in addition to those that we currently anticipate. As a result of the foregoing, we expect to continue to incur significant and increasing losses and negative cash flows for the foreseeable future, which may increase compared to past periods. Even if we are able to generate revenue from the sale of any approved products, we may not become profitable and may need to obtain additional funding to continue operations.

To complete the development and commercialization of SD-809, if approved, for all three planned indications, we will require additional capital. Raising additional funds through debt or equity financing may be dilutive or restrict our operations and raising funds through collaborations or licenses may require us to relinquish rights to our product candidates.

Our operations have consumed substantial amounts of cash since inception. From inception to September 30, 2014, we have raised net cash proceeds of approximately $232.8 million from the sale of common stock, convertible preferred stock, convertible notes and warrants, the exercise of stock options and warrants and ESPP purchases. For example, we raised net proceeds of $86.7 million in connection with our initial public offering in February 2014 and net proceeds of $65.0 million in connection with our follow-on offering in July 2014. Additionally, we have raised proceeds of $3.3 million from the sale and license of certain patent rights

and the sale of equipment. We have also borrowed $15.0 million under our credit facility with Oxford, of which we used $5.0 million to repay borrowings under a credit facility with Square 1 Bank, and the reminder of which we are using for working capital.

We expect to continue to spend substantial amounts to continue clinical development of SD-809, including the conduct of our ongoing clinical trials, planned clinical trials and any future required clinical development, seek regulatory approval for SD-809, launch and commercialize SD-809, if approved, and repay our existing debt.

We expect that the net proceeds to us from this offering and our existing cash, cash equivalents and marketable securities, together with interest thereon, will be sufficient to fund our operations at least through 2017, including through the completion of our ongoing and planned clinical trials and the filing of an NDA and commercial launch of SD-809, if approved, for the treatment of chorea associated with Huntington's disease. However, changing circumstances may cause us to consume capital significantly faster than we currently anticipate, and we may need to spend more money than currently expected because of circumstances beyond our control. For example, our ongoing clinical trials may encounter technical or other issues that could cause our development costs to increase more than we expected. In any event, we expect that we will require additional capital to complete the development of SD-809 for other indications.

We expect to finance future cash needs through public or private equity offerings, debt financings, as well as through interest income earned on cash balances. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our existing stockholders will be diluted, and the terms may include liquidation or other preferences that adversely affect the rights of our stockholders. Debt financings may be coupled with an equity component, such as warrants to purchase shares, which could also result in dilution of our existing stockholders' ownership. The incurrence of indebtedness would result in increased fixed payment obligations and could also result in certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. In addition, if we raise additional funds through collaboration or license arrangements, it may be necessary to relinquish potentially valuable rights to our product candidates or grant licenses on terms that are not favorable to us.

We cannot be certain that additional funding will be available on acceptable terms, or at all. Subject to limited exceptions, the credit facility also prohibits us from incurring indebtedness without the prior written consent of Oxford. If we are unable to raise additional capital in sufficient amounts or on terms acceptable to us we may have to significantly delay, scale back or discontinue the development or commercialization of SD-809. We also could be required to: (1) significantly delay, scale back or discontinue the development or commercialization of our other product candidates; (2) relinquish or license on unfavorable terms our rights to our product candidates that we otherwise would seek to develop or commercialize ourselves; or (3) significantly curtail or cease operations altogether.

Risks related to our intellectual property

If we are unable to obtain or protect intellectual property rights related to our product candidates, we may not be able to compete effectively in our market.

We rely upon a combination of patents, trade secret protection and confidentiality agreements to protect the intellectual property related to SD-809 and our other product candidates. The strength of patents in the biotechnology and pharmaceutical field involves complex legal and scientific questions and can be uncertain. The patent applications that we own may fail to result in issued patents with claims that cover the products in the United States or in other countries. If this were to occur, early generic competition could be expected against product candidates in development. There is no assurance that all of the potentially relevant prior art

relating to our patents and patent applications, which can invalidate a patent or prevent a patent from issuing based on a pending patent application, has been found.

Even if patents do successfully issue, third parties may challenge their validity, enforceability or scope, which may result in such patents being narrowed or invalidated. Any adverse outcome in these types of matters could result in one or more generic versions of our products being launched before the expiration of our Orange Book listed patents, which could adversely affect our ability to establish market share or successfully execute our business strategy to increase sales of our products and would negatively impact our financial condition and results of operations, including causing a significant decrease in our revenues and cash flows.

Composition of matter patents on the chemical API are generally considered to be the strongest form of intellectual property protection for pharmaceutical products, as such patents provide protection without regard to any method of use. Our SD-809 patent portfolio currently includes one issued composition of matter patent in the United States (US 8,524,733), one in Europe (EP 2326643B), one in Japan (JP 5616345) and one in China (CN 102186848), one in New Zealand (591615), and one patent application for which we have received a notice of allowance in the United States, and several pending patent applications in the United States and other countries that, if issued, will cover compositions of matter, methods of treatment, and formulations. The issued U.S. patent is expected to expire in March 2031, and the European patents, Japanese patents and patents in the other countries are expected to expire in September 2029. We cannot be certain that the claims in our patent applications covering composition of matter will be considered patentable by the U.S. Patent and Trademark Office, or U.S. PTO, and courts in the United States or by the patent offices and courts in foreign countries. Method-of-use patents protect the use of a product for the specified method. This type of patent does not prevent a competitor from making and marketing a product that is identical to our product for an indication that is outside the scope of the patented method. Moreover, even if competitors do not actively promote their product for our targeted indications, physicians may prescribe these products off-label. Although off-label prescriptions may infringe or contribute to the infringement of method-of-use patents, the practice is common and such infringement is difficult to prevent or prosecute.

Furthermore, even if they are unchallenged, our patents and patent applications may not adequately protect our intellectual property, provide exclusivity for our product candidates or prevent others from designing around our claims. If the patent applications we hold with respect to SD-809 or our other product candidates fail to issue or if the breadth or strength of protection of our patents or patent applications is threatened, it could threaten our ability to commercialize our products. We cannot offer any assurances about which, if any, patents will issue or whether any issued patents will be found not invalid and not unenforceable or will go unthreatened by third parties. Further, if we encounter delays in regulatory approvals, the period of time during which we could market SD-809 or our other product candidates under patent protection could be reduced. Since patent applications in the United States and most other countries are confidential for a period of time after filing, and some remain so until issued, we cannot be certain that we were the first to file any patent application related to SD-809 or any of our other product candidates. Furthermore, if third parties have filed such patent applications, an interference proceeding in the United States can be provoked by a third party or instituted by us to determine who was the first to invent any of the subject matter covered by the patent claims of our applications. For patent applications filed on or after March 16, 2013, under the “first inventor-to-file” system for patent applications of the Leahy-Smith America Invents Act, priority of patent applications in the United States is determined based on filing date. In the United States, the natural expiration of a patent is generally 20 years after it is filed, or 20 years after the filing date of any non-provisional application to which it claims priority. Various extensions may be available; however the life of a patent, and the protection it affords, is limited. Once the patent life has expired for SD-809 or any of our other product candidates, we may be open to competition from generic medications.

In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable or which we elect not to patent, processes for which patents are difficult to enforce and any other elements of our drug discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. Although we expect all of our employees to assign their inventions to us, and all of our employees, consultants, advisors and any third parties who have access to our proprietary know-how, information or technology to enter into confidentiality agreements, we cannot provide any assurances that all such agreements have been duly executed or that our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. If the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating the trade secret. In addition, our competitors 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.

Our ability to obtain patents is highly uncertain because, to date, some legal principles remain unresolved, there has not been a consistent policy regarding the breadth or interpretation of claims allowed in patents in the United States and the specific content of patents and patent applications that are necessary to support and interpret patent claims is highly uncertain due to the complex nature of the relevant legal, scientific and factual issues. Changes in either patent laws or interpretations of patent laws in the United States and other countries may diminish the value of our intellectual property or narrow the scope of our patent protection. For example, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, which was signed into law in September 2011, includes a number of significant changes to U.S. patent law. These include changes in the way patent applications will be prosecuted and may also affect patent litigation. The U.S. PTO has developed new and untested regulations and procedures to govern the full implementation of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first to file provisions, only became effective in March 2013. The Leahy-Smith Act has also introduced procedures making it easier for third parties to challenge issued patents, as well as to intervene in the prosecution of patent applications. Finally, the Leahy-Smith Act contains new statutory provisions that require the U.S. PTO to issue new regulations for their implementation and it may take the courts years to interpret the provisions of the new statute. Accordingly, it is not clear what, if any, impact the Leahy-Smith Act will have on the cost of prosecuting our patent applications, our ability to obtain patents based on our patent applications and our ability to enforce or defend our issued patents. An inability to obtain, enforce and defend patents covering our proprietary technologies would materially and adversely affect our business prospects and financial condition.

We may not be able to protect our intellectual property rights throughout the world.

Filing, prosecuting and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. Further, the laws of some foreign countries do not protect proprietary rights to the same extent or in the same manner as the laws of the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent

protection to develop their own products and further, may export otherwise infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These products may compete with our products and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing. As a result, we may encounter significant problems in protecting and defending our intellectual property both in the United States and abroad. For example, if the issuance to us, in a given country, of a patent covering an invention is not followed by the issuance, in other countries, of patents covering the same invention, or if any judicial interpretation of the validity, enforceability, or scope of the claims in, or the written description or enablement in, a patent issued in one country is not similar to the interpretation given to the corresponding patent issued in another country, our ability to protect our intellectual property in those countries may be limited. Changes in either patent laws or in interpretations of patent laws in the United States and other countries may materially diminish the value of our intellectual property or narrow the scope of our patent protection. If we are unable to prevent material disclosure of the non-patented intellectual property related to our technologies to third parties, and there is no guarantee that we will have any such enforceable trade secret protection, we may not be able to establish or maintain a competitive advantage in our market, which could materially adversely affect our business, results of operations and financial condition.

Further, many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property protection, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

We may be involved in lawsuits to protect or enforce our patents, which could be expensive, time consuming and unsuccessful.

Competitors may infringe our patents. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that a patent of ours is not valid or is unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated or interpreted narrowly and could put our patent applications at risk of not issuing.

Interference proceedings provoked by third parties or brought by us may be necessary to determine the priority of inventions with respect to our patents or patent applications. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our defense of litigation or interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. We may not be able to prevent misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the United States.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of our common stock.

Proceedings to enforce our patent rights in the United States could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights in the United States may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees on any issued patent are due to be paid to the U.S. PTO and foreign patent agencies in several stages over the lifetime of the patent. The U.S. PTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. If we fail to maintain the patents and patent applications covering SD-809 or other future product candidates, our competitors might be able to enter the market, which would have a material adverse effect on our business.

Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

Because we rely on third parties to manufacture SD-809 and intend to rely on third parties for the manufacture of our other product candidates, we must, at times, share trade secrets with them. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, consulting agreements or other similar agreements with our advisors, employees, third-party contractors and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, including our trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and may have a material adverse effect on our business.

We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of third parties.

We employ individuals who were previously employed at other biotechnology or pharmaceutical companies. We may be subject to claims that we or our employees, consultants or independent contractors have inadvertently

or otherwise used or disclosed confidential information of our employees' former employers or other third parties. Litigation may be necessary to defend against these claims. There is no guarantee of success in defending these claims, and if we are successful, litigation could result in substantial cost and be a distraction to our management and other employees.

We may be subject to claims challenging the inventorship or ownership of our patents and other intellectual property.

We may also be subject to claims that former employees or other third parties have an ownership interest in our patents or other intellectual property. We may be subject to ownership disputes in the future arising, for example, from conflicting obligations of consultants or others who are involved in developing SD-809 or our other product candidates. Litigation may be necessary to defend against these and other claims challenging inventorship or ownership. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

Intellectual property rights do not necessarily address all potential threats to our competitive advantage.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and may not adequately protect our business, or permit us to maintain our competitive advantage. The following examples are illustrative:

- Others may be able to make compounds that are similar to our product candidates but that are not covered by the claims of the patents that we own or have exclusively licensed.
- We might not have been the first to make the inventions covered by the issued patent or pending patent application that we own or have exclusively licensed.
- We might not have been the first to file patent applications covering certain of our inventions.
- Others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights.
- It is possible that our pending patent applications will not lead to issued patents.
- Issued patents that we own may be held invalid or unenforceable, as a result of legal challenges by our competitors.
- Our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets.
- We may not develop additional proprietary technologies that are patentable.
- The patents of others may have an adverse effect on our business.

Should any of these events occur, they could significantly harm our business, results of operations and prospects.

Risks related to this offering and ownership of our common stock

The price of our stock may be volatile, and you could lose all or part of your investment.

Prior to our recently completed initial public offering, there was no public market for our common stock. The trading price of our common stock is likely to be highly volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control, including limited trading volume. In addition to the factors discussed in this "Risk Factors" section and elsewhere in this prospectus, these factors include:

- the commencement, enrollment or results of our ongoing and planned clinical trials of SD-809 or any other future clinical trials we may conduct, or changes in the development status of SD-809 or any future product candidate;
- any delay in filing our NDA for SD-809 and any adverse development or perceived adverse development with respect to the FDA's review of the NDA, including without limitation the FDA's issuance of a "refusal to file" letter or a request for additional information;
- adverse results or delays in our clinical trials;
- our decision to initiate a clinical trial, not to initiate a clinical trial or to terminate an existing clinical trial;
- adverse regulatory decisions, including failure to receive regulatory approval for SD-809 or any of our other product candidates;
- changes in laws or regulations applicable to our product candidates, including but not limited to clinical trial requirements for approvals;
- adverse developments concerning our manufacturers;
- our inability to obtain adequate product supply for any approved drug product or inability to do so at acceptable prices;
- our ability to build a commercial organization or enter into a marketing collaboration with a third party;
- our failure to commercialize SD-809, develop additional product candidates and commercialize additional drugs;
- additions or departures of our key scientific or management personnel;
- unanticipated serious safety concerns related to the use of SD-809 or any future product candidates;
- introduction of new products or services offered by us or our competitors;
- announcements of significant acquisitions, strategic partnerships, joint ventures or capital commitments by us or our competitors;
- our ability to effectively manage our growth;
- the size and growth, if any, of the hyperkinetic movement disorder market;
- our ability to successfully enter new markets or develop additional product candidates;
- actual or anticipated variations in quarterly operating results;
- our cash position;
- our failure to meet the estimates and projections of the investment community or that we may otherwise provide to the public;

- publication of research reports about us or our industry or positive or negative recommendations or withdrawal of research coverage by securities analysts;
- changes in the market valuations of similar companies;
- overall performance of the equity markets;
- issuances of our debt or equity securities;
- sales of our common stock by us or our stockholders in the future;
- trading volume of our common stock;
- changes in accounting practices;
- ineffectiveness of our internal controls;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;
- significant lawsuits, including patent or stockholder litigation;
- general political and economic conditions; and
- other events or factors, many of which are beyond our control.

In addition, the stock market in general, and the NASDAQ Global Market and biotechnology companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance. If the market price of our common stock after this offering does not exceed the offering price per share offered in this offering, you may not realize any return on your investment in us and may lose some or all of your investment. In the past, securities class action litigation has often been instituted against companies following periods of volatility in the market price of a company's securities. This type of litigation, if instituted, could result in substantial costs and a diversion of management's attention and resources, which would harm our business, operating results or financial condition.

Our principal stockholders and management own a significant percentage of our shares and will be able to exert significant control over matters subject to stockholder approval.

As of September 30, 2014, our executive officers, directors and 5% stockholders and their affiliates beneficially owned an aggregate of approximately 68.3% of our outstanding voting shares and, upon completion of this offering, will beneficially own approximately 58.5% of our outstanding voting shares (assuming no exercise of the underwriters' option to purchase additional shares). Therefore, even after this offering, these stockholders may have the ability to influence us through this ownership position. These stockholders may be able to determine all matters requiring stockholder approval. For example, they may be able to control elections of directors, amendments of our organizational documents, or approval of any merger, sale of assets, or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may feel are in your best interest as one of our stockholders.

Sales of a substantial number of shares of our common stock in the public market by our existing stockholders could cause our stock price to decline.

Sales of a substantial number of shares of our common stock in the public market or the perception that these sales might occur, could depress the market price of our common stock and could impair our ability to raise capital through the sale of additional equity securities. We are unable to predict the effect that sales may have on the prevailing market price of our common stock.

We and the selling stockholders, along with our directors, executive management team and the entities affiliated with our directors have agreed that for a period of 60 days after the date of this prospectus, subject to specified exceptions, we or they will not offer, sell, contract to sell, pledge or otherwise dispose of, directly or indirectly, any of our common stock. Subject to certain limitations, approximately 9,414,021 shares of our common stock will become eligible for sale upon expiration of such lock-up period, as calculated and described in more detail in the section entitled "Shares Eligible for Future Sale." Shares issued or issuable upon exercise of options and warrants vested as of the expiration of the lock-up period will also be eligible for sale at that time. Sales of shares by these stockholders upon expiration of the lock-up period could have a material adverse effect on the trading price of our common stock. Certain holders of our common stock are entitled to rights with respect to the registration of their shares under the Securities Act of 1933, as amended, or the Securities Act, subject to the 60-day lock-up arrangement described above. Registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act, except for shares held by our affiliates as defined in Rule 144 under the Securities Act. Any sales of securities by these stockholders could have a material adverse effect on the trading price of our common stock.

Future sales and issuances of our common stock or rights to purchase common stock by us, including pursuant to our equity incentive plans which provide for an automatic increase in the number of shares of common stock issuable thereunder each calendar year through 2024, could result in additional dilution of the percentage ownership of our stockholders and could cause our stock price to decline.

We expect that significant additional capital will be needed in the future to continue our planned operations, including conducting clinical trials, commercialization efforts, expanded research and development activities and costs associated with operating as a public company. To the extent we raise additional capital by issuing equity or convertible 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 the rights of our existing stockholders, including stockholders who purchase shares in this offering.

Pursuant to the 2014 Plan, our management is authorized to grant stock options to our employees, directors and consultants. The number of shares available for future grant under our 2014 Plan will automatically increase on January 1st each year through January 1, 2024, by an amount equal to four percent of all shares of our capital stock outstanding as of December 31st of the preceding calendar year, subject to the ability of our board of directors to take action to reduce the size of such increase in any given year. On January 1, 2015, the initial automatic increase pursuant to the 2014 Plan occurred, resulting in 1,109,847 additional shares available for future grant under the 2014 Plan. In addition, our board of directors may grant or provide for the grant of rights to purchase shares of our common stock pursuant to the terms of the ESPP. The number of shares of our common stock reserved for issuance under our ESPP will automatically increase on January 1st each year through January 1, 2024, by an amount equal to the lesser of 530,000 shares or one percent of all shares of our capital stock outstanding as of December 31st of the preceding calendar year, subject to the ability of our board of directors to take action to reduce the size of such increase in any given year. On January 1, 2015, the initial automatic increase pursuant to the ESPP occurred, resulting in 277,461 additional shares available for future grant under the ESPP. Unless our board of directors elects not to increase the number of shares underlying our 2014 Plan and ESPP each year, our stockholders may experience additional dilution, which could cause our stock price to decline.

If you purchase shares of our common stock sold in this offering, you will incur immediate and substantial dilution.

The public offering price of shares offered in this offering will be substantially higher than the pro forma net tangible book value per share of our outstanding common stock immediately following this offering based on the total value of our tangible assets less our total liabilities. Therefore, if you purchase our common stock in this offering, you will incur immediate and substantial dilution in the amount of $46.34 per share, the difference between the public offering price per share of $56.50 and the pro forma net tangible book value per share of our outstanding common stock as of September 30, 2014. This dilution is due in large part to the fact that our earlier investors paid substantially less than the public offering price when they purchased their shares. In addition, you may also experience additional dilution upon future equity and convertible debt issuances or the exercise of stock options to purchase common stock granted to our employees, consultants and directors under our stock option and equity incentive plans. As of September 30, 2014, options to purchase 1,960,460 shares of common stock at a weighted average exercise price of $8.55 per share and warrants exercisable for up to 708,207 shares of common stock at an exercise price of $4.20 per share were outstanding. See "Dilution." As a result of the dilution to investors purchasing shares in this offering, investors may receive significantly less than the purchase price paid in this offering, if anything, in the event of our liquidation.

We will receive only limited proceeds from this offering and will have broad discretion in the use of the net proceeds to us from this offering, and may not use them effectively.

We will receive only limited proceeds from this offering, as a portion of the proceeds will be paid to the selling stockholders. We will receive net proceeds of only $158.7 million from this offering, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us, and assuming no exercise of the underwriters' option to purchase additional shares. Our management has broad discretion in the application of the net proceeds to us from this offering, including for any of the purposes described in the section entitled "Use of Proceeds," and you will not have the opportunity as part of your investment decision to assess whether the net proceeds are being used appropriately. Because of the number and variability of factors that will determine our use of the net proceeds to us from this offering, their ultimate use may vary substantially from their currently intended use. Our management may not apply the net proceeds to us from this offering in ways that ultimately increase the value of your investment. The failure by our management to apply these funds effectively could harm our business. Pending their use, we may invest the net proceeds to us 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 the net proceeds to us from this offering in ways that enhance stockholder value, we may fail to achieve expected financial results, which could cause our stock price to decline.

Our ability to use our net operating tax loss carryforwards and certain other tax attributes may be limited.

Under Section 382 of the Internal Revenue Code of 1986, as amended, if a corporation undergoes an "ownership change," generally defined as a greater than 50% change (by value) in its equity ownership over a three year period, the corporation's ability to use its pre-change net operating loss carryforwards and other pre-change tax attributes, such as research tax credits, to offset its post-change income may be limited. We believe that with our initial public offering and other transactions that have occurred over the past three years, we may have triggered an "ownership change" limitation. In addition, we may experience ownership changes in the future as a result of subsequent shifts in our stock ownership. As a result, if we earn net taxable income, our ability to use our pre-change net operating loss carryforwards and other pre-change tax attributes to offset U.S. federal taxable income may be subject to limitations, which could potentially result in increased future tax liability to us.

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

We have never declared or paid any cash dividend on our common stock. 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. Our credit facility also contains a negative covenant that prohibits us from paying dividends without the prior written consent of Oxford. Any return to stockholders will therefore be limited to appreciation, if any, of their stock.

We are an emerging growth company, and we cannot be certain if the reduced reporting requirements applicable to emerging growth companies will make our common stock less attractive to investors.

We are an emerging growth company, as defined in the JOBS Act. For as long as we continue to be an emerging growth company, we may take advantage of 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 of the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in this prospectus and 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. We could be an emerging growth company for up to five years following the year in which we completed our initial public offering, although circumstances could cause us to lose that status earlier, including if the market value of our common stock held by non-affiliates exceeds $700 million as of any June 30 before that time or if we have total annual gross revenue of $1.0 billion or more during any fiscal year before that time, in which cases we would no longer be an emerging growth company as of the following December 31, or if we issue more than $1.0 billion in non-convertible debt during any three year period before that time, in which case we would no longer be an emerging growth company immediately. Even after we no longer qualify as an emerging growth company, we may still qualify as a "smaller reporting company" which would allow us to take advantage of many of the same exemptions from disclosure requirements including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. 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 share price may be more volatile.

Under the JOBS Act, emerging growth companies can also delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies. As a result, changes in rules of U.S. generally accepted accounting principles or their interpretation, the adoption of new guidance or the application of existing guidance to changes in our business could significantly affect our financial position and results of operations.

We incur significant increased costs as a result of operating as a new public company, and our management is required to devote substantial time to compliance initiatives.

We completed an initial public offering on February 10, 2014. As a new public company, we incur significant legal, accounting and other expenses that we did not incur as a private company. We are now subject to the reporting requirements of the Securities Exchange Act of 1934, as amended, or the Exchange Act, which require, among other things, that we file with the Securities and Exchange Commission, or the SEC, annual, quarterly and current reports with respect to our business and financial condition. In addition, the Sarbanes-Oxley Act, as well as rules subsequently adopted by the SEC and the NASDAQ Global Market to implement

provisions of the Sarbanes-Oxley Act, imposes significant requirements on public companies, including requiring establishment and maintenance of effective disclosure and financial controls and changes in corporate governance practices. Further, in July 2010, the Dodd-Frank Wall Street Reform and Consumer Protection Act, or the Dodd-Frank Act, was enacted. There are significant corporate governance and executive compensation-related provisions in the Dodd-Frank Act that require the SEC to adopt additional rules and regulations in these areas such as "say on pay" and proxy access. Recent legislation permits emerging growth companies to implement many of these requirements over a longer period and up to five years from the pricing of an initial public offering. We intend to take advantage of this new legislation but cannot guarantee that we will not be required to implement these requirements sooner than budgeted or planned and thereby incur unexpected expenses. Stockholder activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new regulations and disclosure obligations, which may lead to additional compliance costs and impact the manner in which we operate our business in ways we cannot currently anticipate.

We expect the rules and regulations applicable to public companies to substantially increase our legal and financial compliance costs and to make some activities more time-consuming and costly. If these requirements divert the attention of our management and personnel from other business concerns, they could have a material adverse effect on our business, financial condition and results of operations. The increased costs will decrease our net income or increase our net loss, and may require us to reduce costs in other areas of our business or increase the prices of our products or services. For example, 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. We cannot predict or estimate the amount or timing of additional costs we may incur to respond to these requirements. The impact of these requirements could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees or as executive officers.

If our disclosure controls and procedures are not effective, our public reporting may be unreliable, which may lead to misinformation being disseminated to the public.

Our management evaluated our disclosure controls and procedures as of September 30, 2014 and concluded that as of that date, our disclosure controls and procedures were effective. However, in May 2014, we failed to timely furnish a Form 8-K to the SEC with respect to our results of operations and financial condition for the quarter ended March 31, 2014. While we believe that we have remediated the cause of the failure to timely furnish this Form 8-K, if we have failed to correct this issue or if our disclosure controls and procedures otherwise prove to be ineffective, our ability to report information required to be disclosed on a timely and accurate basis may be adversely affected. Any such failure in our disclosure controls and procedures could result in misinformation being disseminated to the public. Investors relying upon any such misinformation may make an uninformed investment decision.

If securities or industry analysts do not continue to publish research or reports, or publish inaccurate or unfavorable research or reports about our business, our share price and trading volume could decline.

The trading market for our common stock depends, in part, on the research and reports that securities or industry analysts publish about us or our business. As a newly public company, we have only limited research coverage by research analysts. We do not have any control over these analysts. Research analysts may elect not to initiate or continue to provide research coverage of our common stock, and such lack of research coverage may adversely affect the market price of our common stock. The price of our stock could decline if one or more research analysts downgrade our stock or issue other unfavorable commentary or research. If one or more of these analysts ceases coverage of us or fails to publish reports on us regularly, demand for our common stock could decrease and we could lose visibility in the financial markets, which could cause our share price and trading volume to decline.

Anti-takeover provisions under our charter documents and Delaware law could delay or prevent a change of control which could limit the market price of our common stock and may prevent or frustrate attempts by our stockholders to replace or remove our current management.

Our amended and restated certificate of incorporation and amended and restated bylaws contain provisions that could delay or prevent a change of control of our company or changes in our board of directors that our stockholders might consider favorable. Some of these provisions include:

- a prohibition on stockholder action through written consent, which requires that all stockholder actions be taken at a meeting of our stockholders;
- a requirement that special meetings of stockholders be called only by the chairman of the board of directors, the chief executive officer, the president or by a majority of the total number of authorized directors;
- advance notice requirements for stockholder proposals and nominations for election to our board of directors;
- a requirement that no member of our board of directors may be removed from office by our stockholders except for cause and, in addition to any other vote required by law, upon the approval of at least 66 2/3% of all outstanding shares of our voting stock then entitled to vote in the election of directors;
- a requirement of approval of at least 66 2/3% of all outstanding shares of our voting stock to amend any bylaws by stockholder action or to amend specific provisions of our certificate of incorporation; and
- the authority of the board of directors to issue preferred stock on terms determined by the board of directors without stockholder approval and which preferred stock may include rights superior to the rights of the holders of common stock.

In addition, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which may prohibit certain business combinations with stockholders owning 15% or more of our outstanding voting stock. These anti-takeover provisions and other provisions in our amended and restated certificate of incorporation and amended and restated bylaws could make it more difficult for stockholders or potential acquirers to obtain control of our board of directors or initiate actions that are opposed by the then-current board of directors and could also delay or impede a merger, tender offer or proxy contest involving our company. These provisions could also discourage proxy contests and make it more difficult for you and other stockholders to elect directors of your choosing or cause us to take other corporate actions you desire. Any delay or prevention of a change of control transaction or changes in our board of directors could cause the market price of our common stock to decline.

Special note regarding forward-looking statements

This prospectus and the documents incorporated by reference herein contain forward-looking statements. The forward-looking statements are contained principally in the sections entitled "Prospectus Summary," "Risk Factors," "Management's Discussion and Analysis of Financial Condition and Results of Operations" and "Business" in this prospectus or the documents incorporated herein by reference. These statements relate to future events or to our future financial performance and involve known and unknown risks, uncertainties and other factors which may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements. Forward-looking statements include, but are not limited to, statements about:

- the success, cost, timing and potential indications of our product development activities and clinical trials, including our ongoing and later trials of SD-809;
- our ability to obtain and maintain regulatory approval of our product candidates, including SD-809, in any of the indications for which we plan to develop them, and any related restrictions, limitations, and/or warnings in the label of an approved product candidate;
- the future results in ongoing or later clinical trials, including SD-809, and our ability to obtain orphan drug designation for SD-809 or any of our other product candidates;
- our ability to obtain funding for our operations, including funding necessary to complete the clinical trials of any of our product candidates, including SD-809;
- our plans to research, develop and commercialize our product candidates, including SD-809;
- our ability to attract and retain collaborators with development, regulatory and commercialization expertise;
- the size of the markets for our product candidates, and our ability to serve those markets;
- our ability to successfully commercialize our product candidates, including SD-809;
- the rate and degree of market acceptance of our product candidates, including SD-809;
- our ability to develop and maintain sales and marketing capabilities, whether alone or with potential future collaborators;
- regulatory developments in the United States and foreign countries;
- the performance of our third-party suppliers and manufacturers;
- the success of competing therapies that are or become available;
- our ability to attract and retain key scientific or management personnel;
- our expectations regarding the period during which we qualify as an emerging growth company under the JOBS Act;
- our use of the proceeds from this offering;
- the accuracy of our estimates regarding expenses, future revenues, capital requirements and needs for additional financing; and
- our expectations regarding our ability to obtain and maintain intellectual property protection for our product candidates and our ability to operate our business without infringing on the intellectual property rights of others.

In some cases, you can identify these statements by terms such as “anticipate,” “believe,” “could,” “estimate,” “expects,” “intend,” “may,” “plan,” “potential,” “predict,” “project,” “should,” “will,” “would” or the negative of those terms, and similar expressions that convey uncertainty of future events or outcomes. These forward-looking statements reflect our management’s beliefs and views with respect to future events and are based on estimates and assumptions as of the date of this prospectus and are subject to risks and uncertainties. We discuss many of these risks in greater detail under the heading “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. Given these uncertainties, you should not place undue reliance on these forward-looking statements.

You should carefully read this prospectus, the documents that we incorporate by reference into this prospectus and the documents we reference in this prospectus and have filed as exhibits to the registration statement, of which this prospectus is a part, completely and with the understanding that our actual future results may be materially different from what we expect. We qualify all of the forward-looking statements in this prospectus by these cautionary statements.

Except as required by law, we assume no obligation to update these forward-looking statements publicly, or to update the reasons actual results could differ materially from those anticipated in any forward-looking statements, whether as a result of new information, future events or otherwise.

Use of proceeds

We estimate that we will receive net proceeds of approximately $158.7 million (or approximately $190.5 million if the underwriters' option to purchase additional shares is exercised in full) from the sale of the shares of common stock offered by us in this offering, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us. We will not receive any proceeds from the sale of common stock by the selling stockholders.

The principal purposes of this offering are to increase our public float, increase our financial flexibility, and facilitate an orderly distribution of shares by the selling stockholders. We anticipate that we will use the net proceeds to us of this offering to fund building the commercial organization for, and commercial launch of, SD-809, if approved, for the potential treatment of chorea associated with Huntington's disease; for research and development, including, but not limited to funding future clinical and regulatory expenses related to SD-809 and related to the NDA filing and preparation for the commercial launch of SD-809, if approved, for other indications, such as tardive dyskinesia; to support the development of SD-560 and other programs in our pipeline; and for working capital and other general corporate purposes, including making monthly principal and interest payments on our credit facility.

We may also use a portion of the net proceeds to us from this offering to in-license, acquire, or invest in complementary businesses, technologies, products or assets. However, we have no current plans, commitments or obligations to do so.

We believe that the net proceeds to us from this offering and our existing cash and cash equivalents and marketable securities, together with interest thereon, will be sufficient to fund our operations at least through 2017, including through the completion of our ongoing and planned clinical trials and the filing of an NDA and commercial launch of SD-809, if approved, for the treatment of chorea associated with Huntington's disease.

Our expected use of net proceeds to us from this offering represents our current intentions based upon our present plans and business condition. As of the date of this prospectus, we cannot predict with certainty all of the particular uses for the net proceeds to us to be received upon the completion of this offering, or the amounts that we will actually spend on the uses set forth above. The amounts and timing of our actual use of the net proceeds to us will vary depending on numerous factors, including our ability to obtain additional financing, the progress, cost and results of our preclinical and clinical development programs, and whether we are able to enter into future licensing or collaboration arrangements. We may find it necessary or advisable to use the net proceeds to us for other purposes, and our management will have broad discretion in the application of the net proceeds to us , and investors will be relying on our judgment regarding the application of the net proceeds from this offering.

Pending their use, we plan to invest the net proceeds to us from this offering in short- and intermediate-term, interest-bearing obligations, investment-grade instruments, certificates of deposit or direct or guaranteed obligations of the U.S. government.

Price range of our common stock

Our common stock has been listed on The NASDAQ Global Market since February 4, 2014 under the symbol "ASPX." Prior to that date, there was no public market for our common stock. Shares sold in our initial public offering on February 4, 2014 were priced at $12.00 per share.

On January 22, 2015, the closing price for our common stock as reported on The NASDAQ Global Market was $56.90 per share. The following table sets forth the ranges of high and low sales prices per share of our common stock as reported on The NASDAQ Global Market for the period indicated. Such quotations represent inter-dealer prices without retail markup, markdown or commission and may not necessarily represent actual transactions.

Year ended December 31, 2014	High	Low
First Quarter (from February 4, 2014)	$35.78	$13.25
Second Quarter	33.09	14.75
Third Quarter	26.59	17.01
Fourth Quarter	54.50	22.53

Year ended December 31, 2015	High	Low
First Quarter (through January 22, 2015)	$64.06	$50.25

As of January 20, 2015, there were 45 stockholders of record, which excludes stockholders whose shares were held in nominee or street name by brokers. The actual number of common stockholders is greater than the number of record holders, and includes stockholders who are beneficial owners, but whose shares are held in street name by brokers and other nominees. This number of holders of record also does not include stockholders whose shares may be held in trust by other entities.

Dividend policy

We have never declared or paid any cash dividends on our capital stock. We currently intend to retain all available funds and any future earnings to support our operations and finance the growth and development of our business. We do not intend to pay cash dividends on our common stock for the foreseeable future. Any future determination related to our dividend policy will be made at the discretion of our board of directors and will depend upon, among other factors, our results of operations, financial condition, capital requirements, contractual restrictions, business prospects and other factors our board of directors may deem relevant. In addition, unless waived, the terms of our credit facility limit our ability to pay cash dividends.

Capitalization

The following table sets forth our cash and cash equivalents, marketable securities and our capitalization as of September 30, 2014:

- on an actual basis; and
- on a pro forma basis, giving effect to the sale by us of 3,000,000 shares of our common stock at the public offering price of $56.50 per share, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us.

You should read this table together with our audited financial statements and the related notes, "Management's Discussion and Analysis of Financial Condition and Results of Operations" and other financial information contained in this prospectus or incorporated by reference herein.

	As of September 30, 2014	
	Actual	Pro forma
	(unaudited) (in thousands, except share and per share amounts)	
Cash, cash equivalents and marketable securities	$ 159,482	$ 318,162
Capitalization:		
Note payable, including current portion, net of discount	$ 14,550	$ 14,550
Stockholders' equity:		
Preferred stock; $0.0001 par value: 10,000,000 shares authorized, no shares issued and outstanding, actual and pro forma	—	—
Common stock; $0.0001 par value: 200,000,000 shares authorized, 27,482,166 shares issued and 26,768,046 outstanding, actual; 200,000,000 shares authorized, 30,482,166 shares issued and 29,768,046 outstanding, pro forma	3	3
Additional paid-in capital	244,505	403,185
Accumulated deficit	(105,026)	(105,026)
Accumulated other comprehensive loss	(17)	(17)
Non-controlling interest	4,279	4,279
Total stockholders' equity	143,744	302,424
Total capitalization	$ 158,294	$ 316,974

The number of common shares outstanding shown in the table above on an actual basis is based on the number of shares of our common stock outstanding as of September 30, 2014, and excludes:

- 714,120 shares of common stock subject to repurchase as of September 30, 2014;
- 1,960,460 shares of common stock issuable upon the exercise of stock options outstanding as of September 30, 2014, at a weighted-average exercise price of $8.55 per share;
- 708,207 shares of common stock issuable upon the exercise of warrants outstanding as of September 30, 2014, at a weighted-average exercise price of $4.20 per share;
- 1,167,253 shares of common stock reserved for future issuance under the 2014 Plan, as of September 30, 2014; and
- 292,105 shares of common stock reserved for issuance under the ESPP, as of September 30, 2014.

Dilution

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

Our historical net tangible book value per share is determined by dividing our total tangible assets less total liabilities by the actual number of outstanding shares of our common stock. The historical net tangible book value of our common stock as of September 30, 2014 was $143.7 million, or $5.37 per share.

After giving pro forma effect to our sale of 3,000,000 shares of our common stock in this offering at the public offering price of $56.50 per share and after deducting the underwriting discounts and commissions and estimated offering expenses payable by us, our net tangible book value as of September 30, 2014, would have been approximately $302.4 million, or $10.16 per share of common stock. This represents an immediate increase in net tangible book value of $4.79 per share to our existing stockholders, and an immediate dilution of $46.34 per share to new investors purchasing shares of common stock in this offering at the public offering price.

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

Public offering price per share		$56.50
Historical net tangible book value deficit per share as of September 30, 2014	$5.37	
Pro Forma increase in net tangible book value per share attributable to new investors	4.79	
Pro forma net tangible book value per share after this offering		10.16
Dilution per share to new investors participating in this offering		$46.34

If the underwriters exercise in full their option to purchase up to 600,000 additional shares of common stock at the public offering price of $56.50 per share, the pro forma net tangible book value after this offering would be $334.3 million, or $11.01 per share, representing an increase in pro forma net tangible book value of $5.64 per share to existing stockholders and immediate dilution of $45.49 per share to investors participating in this offering.

The foregoing discussion is based on 26,768,046 shares of common stock outstanding as of September 30, 2014 and excludes:

- 714,120 shares of common stock subject to repurchase as of September 30, 2014;
- 1,960,460 shares of common stock issuable upon the exercise of stock options outstanding as of September 30, 2014, at a weighted-average exercise price of $8.55 per share;
- 708,207 shares of common stock issuable upon the exercise of warrants outstanding as of September 30, 2014, at a weighted-average exercise price of $4.20 per share;
- 1,167,253 shares of common stock reserved for future issuance under the 2014 Plan, as of September 30, 2014; and
- 292,105 shares of common stock reserved for future issuance under our ESPP, as of September 30, 2014.

Furthermore, we may choose to raise additional capital through the sale of equity or convertible debt securities due to market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans. To the extent we issue additional shares of common stock or other equity or convertible debt securities in the future, there will be further dilution to investors participating in this offering.

Selected financial data

The following selected financial data should be read together with our financial statements and related notes and "Management's Discussion and Analysis of Financial Condition and Results of Operations" incorporated by reference in this prospectus. The selected financial data in this section are not intended to replace our financial statements and the related notes. Our historical results are not necessarily indicative of the results that may be expected in the future and results of interim periods are not necessarily indicative of the results for the entire year.

We derived the selected statement of operations data for the years ended December 31, 2011, 2012 and 2013 and the selected balance sheet data as of December 31, 2012 and 2013 from our audited financial statements incorporated by reference into this prospectus from our Annual Report on Form 10-K for the year ended December 31, 2013. The selected statement of operations data for the nine months ended September 30, 2013 and 2014 and the selected balance sheet data as of September 30, 2014 were derived from our unaudited financial statements incorporated by reference into this prospectus from our Quarterly Report on Form 10-Q for the quarter ended September 30, 2014. The unaudited financial statements have been prepared on a basis consistent with our audited financial statements incorporated by reference in this prospectus and include, in our opinion, all adjustments, consisting only of normal recurring adjustments, necessary for the fair presentation of the financial information in those statements.

	Year ended December 31,			Nine months ended September 30,	
	2011	2012	2013	2013	2014
				(unaudited)	
				(in thousands, except share and per share data)	
Statement of Operations Data:					
Operating expenses:					
Research and development	$ 4,080	$ 11,741	$ 10,003	$ 6,524	$ 21,365
General and administrative	1,893	1,688	3,189	1,760	8,689
Total operating expenses	5,973	13,429	13,192	8,284	30,054
Gain on sale of assets	3,086	–	–	–	–
Operating loss	(2,887)	(13,429)	(13,192)	(8,284)	(30,054)
Other income (expense)	73	(1,683)	(2,437)	244	(9,479)
Net loss	(2,814)	(15,112)	(15,629)	(8,040)	(39,533)
Net income attributed to non-controlling interest	–	–	–	–	13
Net loss attributed to Auspex common stockholders	$ (2,814)	$ (15,112)	$(15,629)	$ (8,040)	$ (39,546)
Net loss per common share attributable to Auspex common stockholders, basic and diluted(1)	$(21,318)	$(114,485)	$ (371)	$(60,909)	$ (1.92)
Weighted-average shares outstanding used to calculate net loss per common share attributable to Auspex common stockholders, basic and diluted(1)	132	132	42,112	132	20,586,368

(1) See Note 2 to our audited financial statements incorporated by reference into this prospectus from our Annual Report on Form 10-K for the year ended December 31, 2013 for an explanation of the method used to calculate the basic and diluted net loss per common share and the number of shares used in the computation of the per share amounts.

	As of December 31,		As of September 30,
	2012	2013	2014
			(unaudited)
			(in thousands)
Balance Sheet Data:			
Cash, cash equivalents and marketable securities	$ 4,279	$ 36,650	$159,482
Working capital	3,306	33,400	156,908
Total assets	4,488	38,872	166,304
Long-term debt, including current portion, net of discount	—	14,420	14,550
Accumulated deficit	(49,851)	(65,480)	105,026
Total stockholders' equity (deficit)	(49,618)	(64,938)	143,744

Business

Overview

We are a biopharmaceutical company focused on the development and commercialization of novel medicines for people with movement disorders and other rare diseases, including orphan diseases, which are rare diseases that affect fewer than 200,000 people in the United States. Our pipeline includes product candidates to address unmet medical needs in hyperkinetic movement disorders, such as chorea (abnormal involuntary movements) associated with Huntington's disease, an orphan disease, and tardive dyskinesia and Tourette syndrome, subsets of either of which may be deemed to be orphan diseases, as well as other orphan indications.

In the United States, a majority of the 30,000 Huntington's disease patients manifest chorea, an estimated 500,000 people suffer from tardive dyskinesia and approximately 100,000 children have tics (abnormal involuntary movements or vocalizations) associated with Tourette syndrome. Movement disorders are debilitating medical conditions which can lead to physical injury, social isolation, loss of independence and interference with employment and activities of daily living. These debilitating disorders are poorly addressed and remain largely untreated. We believe that there is a significant need for a safe and efficacious treatment for these serious conditions.

Our lead product candidate, SD-809, is a small molecule inhibitor of vesicular monoamine 2 transporter or VMAT2, that is designed to regulate the levels of a specific neurotransmitter, dopamine, in the brain. A number of hyperkinetic movement disorders are triggered by abnormal dopamine regulation in the brain. We have recently completed a successful Phase 3 clinical trial of SD-809 for the potential treatment of chorea associated with Huntington's disease. In this trial, which we refer to as First-HD, SD-809 met its primary efficacy endpoint of a statistically significant improvement in the total maximal chorea, or TMC, score, on the Unified Huntington's Disease Rating Scale, or UHDRS, over placebo, as well as showed significant improvements in multiple secondary endpoints including patient global impression of change, or PGIC, clinical global impression of change, or CGIC, and the physical functioning scale of the 36-Item Short-Form Health Survey developed by the RAND Corporation, or the SF-36, a measure of quality of life. Importantly, in First-HD, SD-809 showed a favorable safety and tolerability profile with low rates of depression, somnolence, akathisia/restlessness and anxiety.

We have also successfully completed the four week switch portion of an ongoing open-label safety clinical trial, which we refer to as ARC-HD Switch, where 37 patients on stable doses of tetrabenazine (marketed as Xenazine® in the United States), approved by the U.S. Food and Drug Administration, or FDA, solely for the treatment of chorea associated with Huntington's disease, were switched overnight to SD-809 (at approximately half the dose of tetrabenazine). In this trial, SD-809 maintained chorea control, with chorea scores declining by approximately one point from baseline at Weeks 1 and 4. In addition, data from 21 patients were available at Week 8 at the time of the analysis; these data demonstrated approximately a two point decline from baseline chorea scores. Data for the remaining 15 patients will be available at a future date. Since its launch in the fourth quarter of 2008, annual tetrabenazine sales in the United States have grown to approximately $250 million for the year ended December 31, 2013, which represents approximately 4,000 patients on tetrabenazine therapy in the United States at the end of 2013. Sales of tetrabenazine for the 12 months ended September 30, 2014 are expected to be approximately $290 million, which represents an estimated $70,000 to $75,000 annual cost of treatment per patient. Based on the 14% price increase of Xenazine effective January 2, 2015, reported by the Red Book Online, the annual cost of treatment in 2015 is estimated to be $80,000 to $85,000 per patient. We believe this limited usage of tetrabenazine is primarily due to its undesirable tolerability and side effect profile (including depression, somnolence, akathisia and anxiety), its dosing frequency, drug interactions, the requirement for genotyping for drug metabolizing enzymes, a warning and precaution in the label regarding corrected QT, or QTc, prolongation and other factors.

We believe that the favorable efficacy and safety data demonstrated in First-HD and ARC-HD Switch, combined with earlier clinical trial results, suggest that SD-809, if approved, could become the therapy of choice for treating chorea associated with Huntington's disease, while also suggesting the potential to address unmet needs in a variety of other hyperkinetic movement disorders which will need to be confirmed in additional clinical trials. There are no FDA-approved treatments for tardive dyskinesia and there are limited treatment options treatment of tics associated with Tourette syndrome. A 2007 retrospective chart review conducted by physicians at the Baylor College of Medicine reported that over 75% of patients treated with tetrabenazine had moderate to marked improvement for chorea associated with Huntington's disease, tardive dyskinesia and tics associated with Tourette syndrome. With the same mechanism of action as tetrabenazine, SD-809 may potentially be used to treat these indications. We believe that SD-809 has the potential to change the treatment paradigm and expand the treatment of chorea associated with Huntington's disease, tardive dyskinesia and tics associated with Tourette syndrome. We have initiated two safety and efficacy clinical trials of SD-809 for the potential treatment of tardive dyskinesia. Aim to Reduce Movements in tardive dyskinesia, or ARM-TD, is a Phase 2/3 randomized, double-blind, placebo-controlled, dose-titration clinical trial of SD-809 in approximately 90 patients with tardive dyskinesia. Topline results from this trial are expected in mid-2015. Based on feedback we obtained at a meeting with the FDA, the ARM-TD trial may qualify as one of the two pivotal trials needed for a 505(b)(2) New Drug Application, or NDA, filing, subject to FDA review. Addressing Involuntary Movements in tardive dyskinesia, or AIM-TD, is a Phase 3, randomized, double-blind, placebo-controlled, parallel group clinical trial in approximately 200 people with tardive dyskinesia. We expect topline results from this trial in 2016. Contingent upon successful completion of both studies, we plan to file an NDA for SD-809 for the treatment of tardive dyskinesia in 2016.

In addition to the Huntington's disease and tardive dyskinesia programs, we have initiated an open-label preliminary efficacy, pharmacokinetic and safety Phase 1b clinical trial of SD-809 for the treatment of tics associated with Tourette syndrome. We plan to enroll approximately 20 subjects to evaluate preliminary efficacy and safety, in addition to studying the pharmacokinetics of SD-809 in adolescents. We expect topline data from this trial by mid-2015.

Based on the results of our clinical trials, we plan to submit an NDA to the FDA for SD-809 for the treatment of chorea associated with Huntington's disease by mid-2015 and, if approved, expect to launch commercial sales in 2016. This NDA will use a Section 505(b)(2) regulatory path which we expect will allow us to rely, in our NDA filing, on certain prior nonclinical and clinical safety findings made by the FDA in its approval of the tetrabenazine NDA. We expect the FDA to review data from our own clinical trials as well, including data from First-HD and ARC-HD, as part of its review of our NDA submission. We are also targeting an NDA submission to the FDA for SD-809 for the treatment of tardive dyskinesia in 2016 pending results from our two ongoing clinical trials.

We intend to commercialize SD-809 for chorea associated with Huntington's disease, if approved, through a small specialty sales force. There are a limited number of neurologists nationwide who treat movement disorders and we believe that such a sales force could effectively address the U.S. market for chorea associated with Huntington's disease. We discovered and developed SD-809 and we retain unencumbered worldwide rights for its development and commercialization. We currently own issued composition of matter patents for SD-809 that are expected to expire in 2031 in the United States and 2029 in Europe, before any patent term extension or equivalent to which we may be entitled in the United States or other jurisdictions where we have issued patents. We have additional patent applications for SD-809 that, if issued, will cover composition of matter, methods of treatment, manufacturing, formulations and other applications and aspects of SD-809, which could potentially extend the patent exclusivity period for SD-809. SD-809 has also been granted an orphan drug designation by the FDA for the treatment of Huntington's disease and an orphan drug designation for the treatment of Tourette syndrome in the pediatric population (defined as zero through 16 years of age).

In addition to the issued or allowed patents covering SD-809, we have in our portfolio 60 issued or allowed patents and 60 active patent applications in prosecution covering, among other things, the deuterium-containing form of 58 drugs. These patents include composition of matter and other patents for SD-560, a deuterium-containing form of pirfenidone, which we are evaluating in a Phase 1 cross-over clinical trial versus pirfenidone, with data expected by mid-2015, as well as for SD-1077, a deuterium-containing form of levodopa, which we will be evaluating in proof of concept clinical trials with data expected in 2016. Our portfolio also includes other deuterium-containing compounds that are at various stages of development. We may selectively and opportunistically sell or partner rights for any of these compounds, acquire one or more additional programs or develop and commercialize them ourselves based on strategic considerations and availability of resources. We have recently filed trademark applications to cover the mark AUSTEDO, which is a brand name we have submitted to the FDA for approval to use for SD-809 for the treatment of chorea associated with Huntington's disease.

Our strategy

Our goal is to be a leading biopharmaceutical company focused on the development and commercialization of new medicines in orphan indications initially targeting hyperkinetic movement disorders. Key elements of our strategy to achieve this goal are to:

- *Develop and commercialize SD-809 to be a market leader for the treatment of chorea associated with Huntington's disease, based on its differentiated profile.* We expect data from our completed Phase 3 clinical trial of SD-809 in patients with chorea associated with Huntington's disease will serve as the basis for our NDA submission. Because the only FDA-approved treatment for chorea associated with Huntington's disease has limitations, we believe SD-809, if approved, would be an attractive treatment alternative for patients with chorea.

- *Develop SD-809 for the treatment of additional hyperkinetic movement disorders with unmet medical needs, including tardive dyskinesia and Tourette syndrome.* We have initiated two safety and efficacy clinical trials of SD-809 in patients with tardive dyskinesia, where there are no approved therapies. We also have initiated a Phase 1b clinical trial in adolescent patients with tics associated with Tourette syndrome, where we believe that physicians consider existing therapies to be inadequate. We expect the results from these trials will form the basis of both our NDA submission for the treatment of tardive dyskinesia, and the remaining development activities, including pivotal clinical trials, required for regulatory approval of SD-809 in tics associated with Tourette syndrome.

- *Build targeted sales and marketing capabilities in the United States for SD-809, initially focused on movement disorder neurologists*. Subject to obtaining approval for SD-809 for the treatment of chorea associated with Huntington's disease, we anticipate building a commercial infrastructure, initially including a small specialty sales force.

- *Leverage our expertise in deuterium chemistry as well as clinical and regulatory development to derive value from our broad portfolio of proprietary product candidates*. We have over 50 additional deuterium-containing product candidates in our portfolio at various stages of development. These compounds could be advanced or partnered in the future based on strategic considerations and availability of resources.

Overview of hyperkinetic movement disorders

Hyperkinetic movement disorders are characterized by abnormal, involuntary muscle contractions and can arise from a variety of causes, typically either genetic or drug-induced. Many hyperkinetic movement disorders,

including chorea associated with Huntington's disease, tardive dyskinesia, Tourette syndrome and others, benefit from treatment with drugs that reduce dopamine nerve transmission, such as inhibitors of VMAT2. Increased involuntary motor output is often driven by improper dopamine regulation in the brain.

Huntington's disease. Huntington's disease is a hereditary neurodegenerative orphan disease that results in motor, cognitive and psychiatric disability, primarily due to the destruction of neurons in the brain. The National Institutes of Health estimates that 30,000 people in the United States have Huntington's disease. One of the most visually prominent symptoms of this disease is chorea, which occurs in 90% of patients. In addition to the 30,000 individuals with Huntington's disease, more than 200,000 people in the United States carry the gene and are at risk for Huntington's disease. In approximately 70% of patients with Huntington's disease, chorea is moderate to severe and can result in difficulty walking, speaking, swallowing or performing simple everyday tasks. Psychiatric symptoms, such as depression and anxiety, are common among these patients. The only FDA-approved treatment for chorea associated with Huntington's disease is tetrabenazine, an inhibitor of VMAT2, which decreases presynaptic dopamine levels. While the drug is effective at controlling chorea, its package insert shows high rates of adverse events and its short half-life requires three or more times daily dosing in a majority of patients. The most common adverse events listed in the tetrabenazine label include sedation/somnolence, fatigue, insomnia, depression, akathisia, anxiety and nausea (see Table 1 from the Xenazine label below). In addition, the FDA-approved label for tetrabenazine states that patients requiring greater than 50 mg/day, or 50% of the maximal dose, should be genotyped for the drug-metabolizing enzyme, CYP2D6. The distribution of the drug is also highly restricted and is subject to an FDA-mandated Risk Evaluation and Mitigation Strategies, or REMS, program. We believe the foregoing properties of tetrabenazine have contributed to its limited use. Since its launch in the fourth quarter of 2008, annual tetrabenazine sales in the United States have grown to approximately $250 million for the year ended December 31, 2013, which represents approximately 4,000 patients on tetrabenazine therapy in the United States at the end of 2013. Sales of tetrabenazine for the 12 months ended September 30, 2014 are expected to be approximately $290 million, which represents an estimated $70,000 to $75,000 annual cost of treatment per patient. Based on the 14% price increase of Xenazine effective January 2, 2015, reported by the Red Book Online, the annual cost of treatment in 2015 is estimated to be $80,000 to $85,000 per patient.

Tardive dyskinesia. Tardive dyskinesia is a hyperkinetic movement disorder that is induced by dopamine receptor blocking agents, such as neuroleptics, which are used for treating psychiatric conditions, including schizophrenia and bipolar disease, as well as by certain drugs, such as metoclopramide, which are used for treating various gastrointestinal disorders. Neuroleptics are estimated to be used by approximately four million Americans according to federal government data from the Medical Expenditure Panel Survey. From 1996 to 2011, annual neuroleptic prescriptions in the United States increased from 21 million to 57 million, and annual sales increased from $1 billion to $18 billion, according to IMS Health Institute data. Tardive dyskinesia typically manifests as rapid, repetitive, stereotypic movements involving the tongue, lips and jaw that may involve puffing of cheeks, protruding of the tongue, lip smacking, puckering, pursing and chewing. In the United States, an estimated 500,000 patients have tardive dyskinesia. These patients are managed largely by psychiatrists and movement disorder neurologists, and there are no FDA-approved treatments for the condition.

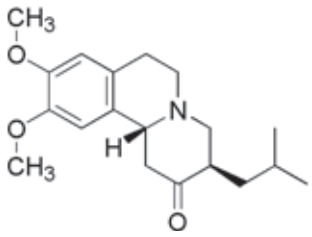
Tourette syndrome. Tourette syndrome is a hyperkinetic movement disorder manifested by motor and phonic tics, which are often accompanied by neurobehavioral disorders such as attention-deficit hyperactivity disorder, or ADHD, and obsessive-compulsive disorder, or OCD. Tics can be simple such as blinking, eye rolling, nose twitching, head nodding and mouth pouting, or more complex such as touching, squatting, jumping or hopping. Tics can result in significant long-term social, legal and developmental consequences for patients, as well as injury and physical disability including pain and secondary neurological deficits. In the United States, an estimated 100,000 children have tics associated with Tourette syndrome. According to the U.S. Centers for Disease Control and Prevention, 37% of these children have moderate to severe forms of Tourette syndrome. The mean age of onset is at four to six years, with peak severity around 12 years of age, with an estimated 13%

to 22% of affected children continuing to take medications for tics as adults. The FDA Office of Orphan Products Development has designated SD-809 as an orphan drug for the treatment of Tourette syndrome in the pediatric population (defined as zero through 16 years of age). There have been no new drugs introduced for treating tics associated with Tourette syndrome in more than 30 years, and we believe that physicians consider the two approved neuroleptics to be inadequate. These treatments carry, among other adverse events, the risk of causing permanent neurologic deficits, such as tardive dyskinesia.

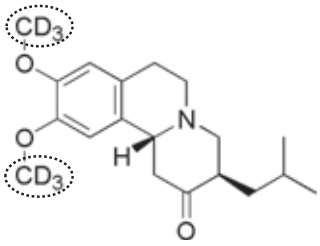
The SD-809 opportunity

Using our know-how in deuterium chemistry, we made chemical modifications at specific positions in the tetrabenazine molecule to create the novel drug candidate SD-809 (deutetrabenazine). We developed SD-809 to have the same shape, size, charge, intrinsic potency and target pharmacology of tetrabenazine and to improve its tolerability and safety profile. Deuterium is a non-toxic, naturally occurring form of hydrogen. SD-809 is an oral small molecule with potential for once-daily or twice-daily dosing. We have successfully completed a Phase 3 clinical trial of SD-809 (AUSTEDO™; the brand name has been submitted to the FDA for approval) for the treatment of chorea associated with Huntington's disease and are also conducting two clinical trials of SD-809 for the treatment of tardive dyskinesia, as well as one Phase 1b clinical trial for the treatment of tics associated with Tourette syndrome.

The structural difference between SD-809 and tetrabenazine is six substitutions of deuterium atoms (D; ^{2}H) for hydrogen atoms (H; ^{1}H), as depicted below.

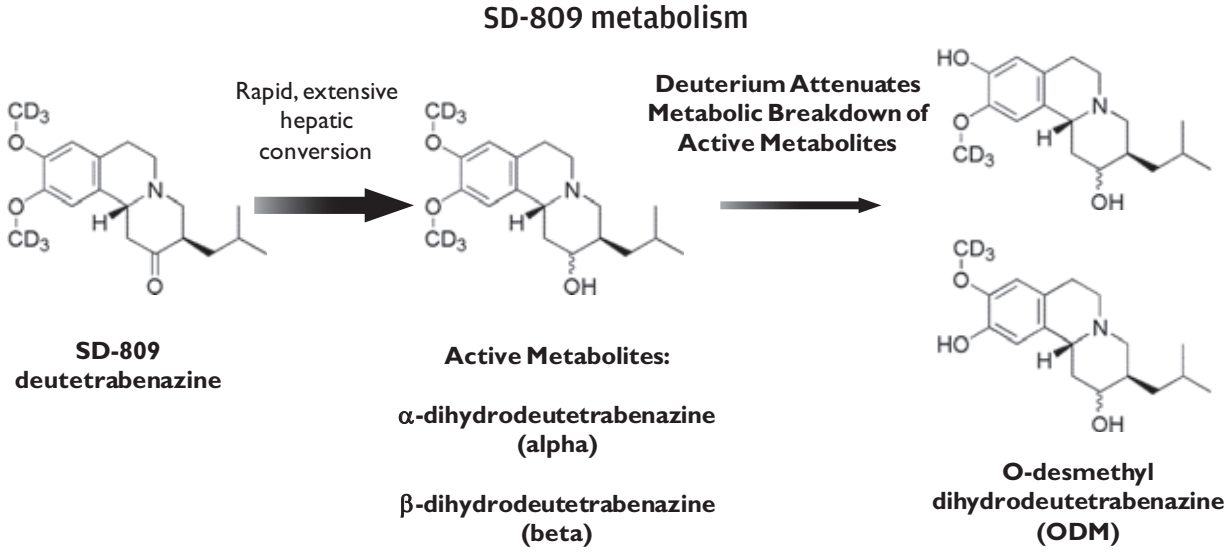


Xenazine®
(tetrabenazine)



SD-809
(deutetrabenazine)

The carbon-deuterium covalent bond requires about eight times more energy to break than the carbon-hydrogen bond. The substitution of deuterium for hydrogen at specific positions attenuates the breakdown of active metabolites (as depicted below), which may result in a differentiated profile such as less frequent dosing, improved tolerability, reduced interpatient variability in drug metabolism, and reduced QT interval prolongation as well as reduced drug interactions and reduced need for CYP2D6 genotyping. We believe that this profile will allow SD-809 to address unmet needs in a variety of hyperkinetic movement disorders.



The FDA-approved label for Xenazine reports high rates of treatment-emergent adverse events including, among others, somnolence/sedation, insomnia, fatigue, depression, akathisia and anxiety. In some cases, this led to an alteration in dosing. The full Table 1 from the FDA-approved label, provided below, shows adverse reactions that occurred in more than 4% of patients and with a greater frequency in Xenazine-treated patients than in placebo-treated patients. Text below the FDA-approved label shown in Table 1 states that “Dose escalation was discontinued or dosage of study drug was reduced because of one or more adverse reactions in 28 of 54 (52%) patients randomized to XENAZINE. These adverse reactions consisted of sedation (15), akathisia (7), parkinsonism (4), depression (3), anxiety (2), fatigue (1) and diarrhea (1). Some patients had more than one adverse reaction and are, therefore counted more than once.”

Table 1. Treatment Emergent Adverse Reactions in Patients Treated with XENAZINE and with a Greater Frequency than Placebo in the 12-Week, Double-Blind, Placebo-Controlled Trial of XENAZINE

Body System	AE Term	XENAZINE n = 54 n (%)	Placebo n = 30 n (%)
PSYCHIATRIC DISORDERS	Sedation/somnolence	17 (31%)	1 (3%)
	Insomnia	12 (22%)	–
	Depression	10 (19%)	–
	Anxiety/anxiety aggravated	8 (15%)	1 (3%)
	Irritability	5 (9%)	1 (3%)
	Appetite decreased	2 (4%)	–
	Obsessive reaction	2 (4%)	–
CENTRAL & PERIPHERAL NERVOUS SYSTEM	Akathisia	10 (19%)	–
	Balance difficulty	5 (9%)	–
	Parkinsonism/bradykinesia	5 (9%)	–
	Dizziness	2 (4%)	–
	Dysarthria	2 (4%)	–
	Gait unsteady	2 (4%)	–
	Headache	2 (4%)	1 (3%)
GASTROINTESTINAL SYSTEM DISORDERS	Nausea	7 (13%)	2 (7%)
	Vomiting	3 (6%)	1 (3%)
BODY AS A WHOLE—GENERAL	Fatigue	12 (22%)	4 (13%)
	Fall	8 (15%)	4 (13%)
	Laceration (head)	3 (6%)	–
	Ecchymosis	3 (6%)	–
RESPIRATORY SYSTEM DISORDERS	Upper respiratory tract infection	6 (11%)	2 (7%)
	Shortness of breath	2 (4%)	–
	Bronchitis	2 (4%)	–
URINARY SYSTEM DISORDERS	Dysuria	2 (4%)	–

Source: Xenazine FDA-approved label (9/2012)

Although our reliance on the FDA's prior findings of safety for tetrabenazine may require any approved labeling for SD-809 to include certain safety information from the tetrabenazine label, we also intend to submit safety data from our First-HD and ARC-HD clinical trials to the FDA. These data should allow for SD-809 labeling to include certain safety information that is different than the tetrabenazine label. However, since our First-HD Phase 3 clinical trial does not include a tetrabenazine arm, we will not be able to make direct comparative claims regarding the safety of SD-809 and tetrabenazine either in labeling or in our promotional materials for SD-809 from this trial.

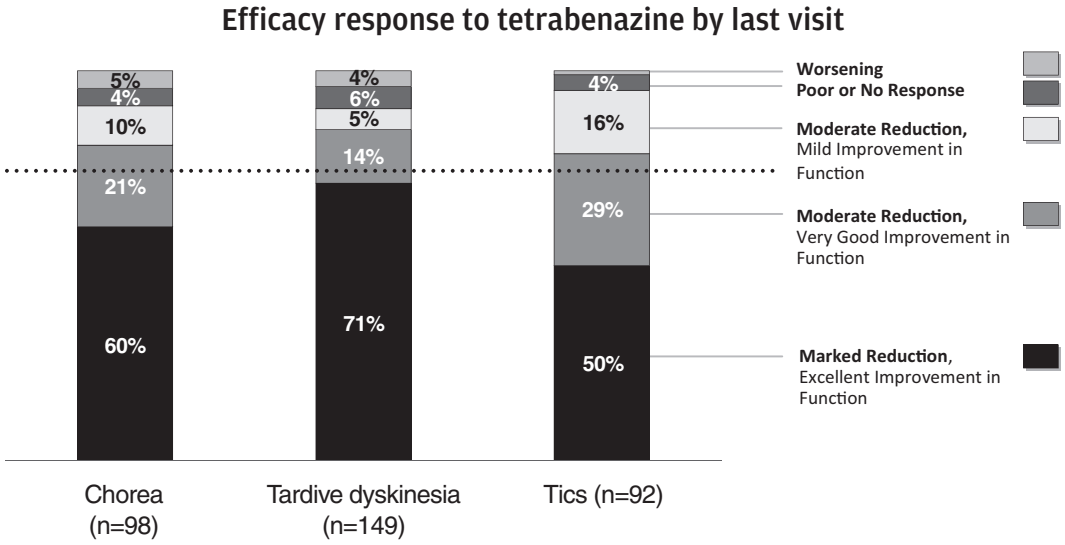
We believe that many of the side effects are driven by tetrabenazine's Cmax (the maximum concentration that a drug achieves in the tested area of the body after administration) and widely-fluctuating levels of its active metabolites. We believe, as a result of the short half-life of the active metabolites of tetrabenazine, the majority of patients treated with tetrabenazine are dosed three or more times daily. The FDA-approved label for tetrabenazine states that patients requiring greater than 50 mg/day should be genotyped for the drug-metabolizing enzyme, CYP2D6. CYP2D6 is a member of the cytochrome p450 super-family of enzymes that are involved in drug metabolism and determine what level of drug exposure patients may experience. Approximately 4,000 patients were on tetrabenazine therapy in the United States at the end of 2013. We believe this limited usage of tetrabenazine is primarily due to its dosing frequency as well as its poor tolerability and side effect profile.

In April 2011, we commissioned a survey of 60 neurologists and 29 psychiatrists who were asked to evaluate two hypothetical product profiles of SD-809, which included reduced interpatient variability, less frequent dosing and a reduction in adverse events of either 15% or 33%, compared to tetrabenazine. Neurologists or psychiatrists who were qualified were asked to complete the survey for up to two of (1) chorea associated with Huntington's disease, (2) tardive dyskinesia and (3) tics associated with Tourette syndrome, with only neurologists having the option to respond for chorea associated with Huntington's disease. The responses to the survey suggest that tetrabenazine is prescribed by the physicians surveyed to treat mild to severe symptoms of Huntington's disease in 17% to 37% of patients. The survey further suggests that if the two hypothetical product profiles of SD-809 described above were available for prescription, tetrabenazine and hypothetical SD-809 would be used for the treatment of mild to severe symptoms of Huntington's disease in 29% to 59% of patients, with 18% to 37% of patients being treated with hypothetical SD-809. For the treatment of mild to severe tardive dyskinesia, the survey suggests that tetrabenazine and hypothetical SD-809 would be used in 13% to 41% of patients, compared to 1% to 18% if tetrabenazine were the only treatment option. Similarly, for the treatment of mild to severe tics associated with Tourette syndrome, the survey suggests that tetrabenazine and hypothetical SD-809 would be used in 19% to 50% of patients, compared to 1% to 13% if tetrabenazine were the only treatment option. In addition, there was not a significant difference in the preference between the two hypothetical profiles of SD-809 surveyed, which may suggest that physicians are looking for a product with even a modest improvement in safety over the current standard of care. The survey suggests that SD-809 could have a target profile that is valued by physicians for the treatment of hyperkinetic movement disorders as most of the physicians surveyed indicated that they would prescribe SD-809, if approved, with either of the two hypothetical profiles described above, for the treatment of chorea associated with Huntington's disease, tardive dyskinesia and tics associated with Tourette syndrome.

We believe that SD-809, based on its profile and the topline results of the clinical trials in patients with chorea associated with Huntington's disease, may provide significant benefits for not only patients with chorea associated with Huntington's disease but also potentially for patients with other hyperkinetic movement disorders, such as tardive dyskinesia and Tourette syndrome, which will need to be confirmed with additional clinical trials in patients with tardive dyskinesia and Tourette syndrome. An FDA-approved label with promotable indication statements for the treatment of tardive dyskinesia and Tourette syndrome may be an important commercial driver.

Rationale for SD-809 development in hyperkinetic movement disorders

Currently the only FDA-approved treatment for chorea associated with Huntington's disease is tetrabenazine. We believe tetrabenazine is also used off-label by physicians to treat patients suffering from tardive dyskinesia and tics associated with Tourette syndrome, and this use may account for up to 50% of tetrabenazine usage in the United States based on our discussions with treating physicians. As depicted below, a 2007 retrospective chart review, conducted by physicians at the Baylor College of Medicine, reported that over 75% of patients treated with tetrabenazine had moderate to marked improvement for chorea associated with Huntington's disease, tardive dyskinesia and tics associated with Tourette syndrome.



Source: *Kenney et al. Long-term tolerability of tetrabenazine in the treatment of hyperkinetic movement disorders. Mov. Disorders 22(2), 193-197(2007)*

We believe that the SD-809 profile has the potential to change the treatment paradigm and expand the treatment of chorea associated with Huntington's disease, tardive dyskinesia and Tourette syndrome. Our research and results from others in the field show that deuterium-containing compounds have target and receptor binding that is indistinguishable from their corresponding non-deuterium-containing compounds. Consistent with these observations, our research comparing binding of the active metabolites of SD-809 and tetrabenazine to VMAT2 also demonstrates indistinguishable binding of these compounds to the target. Since target binding is well established in pharmacology as the fundamental basis of a compound's efficacy, we believe that the historical clinical literature for tetrabenazine's efficacy is also supportive of SD-809's potential efficacy in hyperkinetic movement disorders. The positive topline Phase 3 efficacy results of SD-809 for the treatment of chorea associated with Huntington's disease, we believe, validates our approach for treating this debilitating disorder as well as potentially other movement disorders.

SD-809 for the treatment of chorea associated with Huntington's disease.

We have successfully completed our First-HD Phase 3 clinical trial of SD-809 for the treatment of chorea associated with Huntington's disease. First-HD was a randomized double-blind, placebo-controlled parallel-group, 12-week clinical trial with the same primary endpoint (change in TMC score from baseline to maintenance therapy) and a similar patient population as the registration trial for tetrabenazine. The overall treatment period was 12 weeks in duration with a titration period that lasted eight weeks and a maintenance period that lasted four weeks, similar to the registration trial for tetrabenazine. After the 12-week treatment

period, there was a one-week washout period. First-HD enrolled 90 patients with a 1:1 randomization scheme, as compared to the 84 patients randomized with a 2:1 randomization scheme in the tetrabenazine trial. In First-HD, SD-809 met its primary efficacy endpoint, the change from baseline to maintenance therapy on TMC score of the UHDRS, demonstrating a meaningful 2.5 point improvement ($p<0.0001$) or 21% improvement ($p<0.0001$) over placebo, as well as showing significant improvements in multiple secondary endpoints including PGIC, CGIC and quality of life compared to placebo. Importantly, the trial showed that SD-809 has a favorable safety and tolerability profile with low rates of adverse events, such as depression, somnolence, akathisia and anxiety.

SD-809 for the treatment of tardive dyskinesia and tourette syndrome.

Tetrabenazine has been evaluated by physicians for the treatment of tardive dyskinesia in more than 400 patients across multiple published studies. A review of 11 case reports, retrospective chart reviews and open-label studies from 1961-2007, in study populations of two to 149 patients with tardive dyskinesia, shows that, of the more than 400 patients included in the review, approximately 85% responded to treatment with tetrabenazine. One open-label, single-arm, randomized, investigator-sponsored study showed a 9.7 point reduction from a baseline of 17.9 in the commonly accepted endpoint of Abnormal Involuntary Movements Scale, or AIMS, score. On the basis of this clinical experience, several treatment algorithms published between 2009 and 2012 have reported that tetrabenazine is effective for the treatment of tardive dyskinesia. Tetrabenazine has also been evaluated by physicians for the treatment of tics associated with Tourette syndrome in more than 300 patients across multiple published studies. A review of ten publications from 1974-2008 in study populations of five to 120 patients with tics associated with Tourette syndrome shows that, of the more than 300 patients included in the review, approximately 75% responded to treatment with tetrabenazine. We believe that this historical physician experience with tetrabenazine has established the dose required for efficacy in tardive dyskinesia and Tourette syndrome. This will inform our dose selection for our clinical trials.

In addition, two placebo-controlled clinical trials with another VMAT2 inhibitor (NBI-98854) in patients with moderate to severe tardive dyskinesia suggest that this mechanism of action may have clinical activity. In one study, the mean reduction in AIMS from baseline to Week 6 was 33% in the NBI-98854 group, and 3% in the placebo group. The mean daily dose at the end of study was 64 mg in the active group. In a second study, the same primary endpoint was negative; when re-analyzed using blinded central video review of the AIMS, the difference from placebo was reported as statistically significant, with the re-analyzed reduction in the active arm as approximately 16% with a mean daily dose of 50 mg at the end of the study.

We believe that SD-809's favorable efficacy, safety and tolerability profile, as well as simplified dosing regimen, reduced interpatient variability in drug metabolism, reduced drug interactions, reduced QT prolongation and a reduced need for CYP2D6 genotyping, could potentially expand the treatment of hyperkinetic movement disorders, if the FDA approves SD-809 for these indications.

Clinical development of SD-809

We are developing SD-809 for the treatment of chorea associated with Huntington's disease. Our clinical program has consisted of the following trials:

- First-HD, a randomized, double-blind, placebo-controlled, parallel-group Phase 3 clinical trial in patients not receiving tetrabenazine, which we expect will serve as the basis for our NDA submission and commercialization in this indication; and
- ARC-HD, an open-label, long-term, safety clinical trial which has two components: ARC-HD Switch, in which subjects on tetrabenazine switch overnight to SD-809, and ARC-HD Rollover, in which patients who successfully complete First-HD may rollover. Subjects in ARC-HD Switch or ARC-HD Rollover may receive SD-809 for one year or longer.

We have also completed several clinical trials in support of the development of SD-809. We submitted an investigational new drug application, or IND, for SD-809 to the FDA in 2011 to begin U.S. clinical development. The following key attributes of SD-809, when compared to tetrabenazine, have been demonstrated by our completed Phase 1 clinical trials:

Attribute	Clinical trial
Reduced interpatient variability	Two-period crossover clinical trial
Reduced drug interaction	Drug interaction clinical trial*

* Not a head-to-head comparison trial of SD-809 with tetrabenazine, but produced data that, when compared to available data on tetrabenazine, revealed the indicated attributes of SD-809.

We have also completed a thorough QT study of SD-809 in healthy volunteers. This was an active- and placebo-controlled crossover trial to determine the effect of single doses of SD-809 on the QT interval. The thorough QT clinical trial demonstrated that at two different dose levels, SD-809 had no clinically significant effect on cardiac repolarization as assessed by the QT interval. A tetrabenazine arm was included for comparison and demonstrated an increase in QTc that is consistent with the effect reported in the FDA label for Xenazine. The clinical trial's assay sensitivity was established by the observation of characteristic QTc prolongation following dosing with moxifloxacin.

We also have four ongoing clinical trials to support the further development of SD-809 for the treatment of tardive dyskinesia and Tourette syndrome:

- ARM-TD, an efficacy and safety dose-titration Phase 2/3 clinical trial in patients with drug-induced tardive dyskinesia;
- AIM-TD, an efficacy and safety fixed-dose Phase 3 clinical trial in patients with drug-induced tardive dyskinesia, which if successful, will be combined with ARM-TD to serve as the basis of our NDA submission in tardive dyskinesia;
- RIM-TD, an open-label, long-term, safety clinical trial in which patients who successfully complete ARM-TD and AIM-TD may rollover into this trial, where all the patients may receive SD-809 for at least one year; and
- Phase 1b open-label efficacy, pharmacokinetic and safety clinical trial in patients with Tourette syndrome.

Phase 3 clinical trials of SD-809 in Huntington's disease: First-HD and ARC-HD

First-HD: Phase 3 placebo-controlled trial

We have completed our First-HD Phase 3 clinical trial of SD-809, which was a 1:1 randomized, double-blind, placebo-controlled, parallel-group trial designed to evaluate, and generate label information for, the safety, tolerability and efficacy of SD-809 for treating chorea associated with Huntington's disease. Patients were treated with SD-809 or placebo starting at 6 mg once per day to up to 24 mg twice per day (48 mg total maximum daily dose). Key inclusion criteria for patients included having a TMC score of greater than or equal to eight at screening and baseline, as well as a total functional capacity of greater than or equal to five at screening. Key exclusion criteria included serious, untreated/undertreated psychiatric illness, or recent treatment with tetrabenazine. A total of 90 patients (45 in each group) were enrolled for evaluation over 13 weeks: patients were titrated weekly to an optimal dose up to Week 8, were on maintenance therapy for four weeks, and were taken off study medication in the final week of the trial. A total of 87 patients completed the trial; one patient in the SD-809 group and two in the placebo group discontinued.

This trial was conducted at 34 enrolling centers in the United States and Canada in collaboration with the Huntington Study Group. The primary and secondary efficacy results and safety data from the First-HD and ARC-HD Switch clinical trials were confirmed by the Huntington Study Group's independent analysis. Efficacy analyses included all patients randomized to treatment.

The primary efficacy endpoint for this trial was change in TMC score on the UHDRS from baseline to maintenance therapy, where final score is defined as the average of values from the Week 9 and Week 12 visits. The TMC score is a clinician-based, quantitative assessment of chorea in seven body regions: face, mouth/tongue, trunk, and the four extremities. Each region is rated from zero (no chorea) to four (severe chorea), with an overall score ranging from zero to a maximum of 28. The primary efficacy endpoint is the same endpoint that was accepted by the FDA when it considered and approved the NDA for tetrabenazine in 2008. SD-809 met the pre-specified primary efficacy endpoint. Patients taking SD-809 achieved a meaningful improvement of 2.5 points on the TMC score from baseline to maintenance therapy compared to placebo ($p < 0.0001$). In a pre-specified additional analysis of efficacy, this 2.5 point improvement in TMC represented a reduction of 21 percentage points over placebo ($p<0.0001$). The mean dose at Week 12 among patients treated with SD-809 was approximately 40 mg.

Another pre-specified motor endpoint, the total motor score, or TMS, of the UHDRS, was also analyzed, and is summarized in the table below. The TMS assesses all the motor features of Huntington's disease and includes items other than chorea, such as gait and postural instability. The statistically significant improvement in the TMS score of 4.0 points over placebo suggests a potential benefit of SD-809 on other motor symptoms besides chorea.

Pre-specified motor endpoints	SD-809	Placebo	Treatment effect	Favors	P-value
Change in TMC Score[1] from Baseline to Maintenance Therapy*	4.4 Point Improvement	1.9 Point Improvement	Improvement of 2.5 Points over Placebo	SD-809	$p < 0.0001$
Percent Change in TMC Score from Baseline to Maintenance Therapy	37 Percentage Points Improvement	16 Percentage Points Improvement	Improvement of 21 Percentage Points over Placebo	SD-809	$p < 0.0001$
Change in TMS[1] from Baseline to Maintenance Therapy	7.4 Point Improvement	3.4 Point Improvement	Improvement of 4.0 Points over Placebo	SD-809	$p = 0.002$

[1] TMC and TMS are subscales of the Unified Huntington's Disease Rating Scale

* Primary efficacy endpoint

The clinical relevance of the change in chorea was assessed by four pre-specified secondary endpoints. These four pre-specified secondary endpoints assessed the change from baseline to end-treatment and were tested in a hierarchical testing procedure: (1) treatment success based on PGIC; (2) treatment success based on CGIC; (3) the physical functioning scale of the SF-36; and (4) balance, as assessed by the Berg Balance Test, or BBT. Both PGIC and CGIC assess the change in the patient's overall Huntington's disease symptoms relative to baseline using a seven-point Likert scale, a measurement scale commonly used in clinical research where a patient provides answers to pre-specified questions with a limited number of response options. Responses ranged from "very much worse" to "very much improved." Success was defined as "much improved" or "very much improved" on the Likert scale. Patients and clinicians were asked "With respect to your (or the subject's) overall Huntington's disease symptoms, how would you describe yourself compared to immediately before starting study medication."

The physical functioning scale is a component of the SF-36 and is a validated patient-reported quality of life instrument that has been used in many disease states. The 10-item Physical Functioning Scale was chosen as a trial endpoint because it captures meaningful physical activities, such as lifting or carrying groceries, climbing one or more flights of stairs, bending, kneeling, walking 100 yards or more, moderate to vigorous activities and

self-care such as bathing or dressing. The SF-36 physical functioning scale has been shown to measure impairment experienced by people living with Huntington's disease. The BBT is a 14-item assessment of balance and was used to evaluate if a change in balance was associated with a reduction in chorea since medications currently used to treat chorea may worsen balance. SD-809 did not worsen balance, and in fact the data numerically favored SD-809, although the improvement was not statistically significant. As summarized in the following table, the first three key secondary endpoints, including two patient-rated measures of benefit (PGIC and SF-36 physical functioning scale), showed statistically significant superiority of SD-809 over placebo.

Pre-specified key secondary endpoints[1]	Favors	P-value
1. Patient Global Impression of Change (PGIC)[2]	SD-809	p = 0.002
2. Clinical Global Impression of Change (CGIC)[2]	SD-809	p = 0.002
3. SF-36 Physical Functioning Scale (a Quality of Life measure) from Baseline to Week 12	SD-809	p = 0.03
4. Berg Balance Test	SD-809	p = 0.14

[1] Analyzed using a pre-specified hierarchical testing procedure

[2] Success defined as much improved or very much improved

In general, SD-809 was well tolerated. The overall rates of adverse events were similar between SD-809 and placebo, and there were no differences between SD-809 and placebo in the rate of dose reduction (6.7% in each group) or dose suspension (2.2% in each group) for adverse events. Results from First-HD show a favorable safety and tolerability profile of SD-809 as indicated by the numbers of patients reporting adverse events in each system organ class.

System organ class	SD-809 n = 45	Placebo n = 45
Psychiatric Disorders	8 (17.8%)	8 (17.8%)
Nervous System Disorders	8 (17.8%)	10 (22.2%)
All Other Body Systems		
Cardiac Disorders	0 (0.0%)	3 (6.7%)
Ear & Labyrinth	1 (2.2%)	1 (2.2%)
Eye Disorders	1 (2.2%)	1 (2.2%)
General Disorders	7 (15.6%)	8 (17.8%)
Gastrointestinal Disorders	9 (20%)	9 (20%)
Hepatobiliary Disorders	1 (2.2%)	0 (0.0%)
Infections and Infestations	5 (11.1%)	5 (11.1%)
Injury, Poisoning and Procedural Complications	4 (8.9%)	6 (13.3%)
Investigations[1]	6 (13.3%)	3 (6.7%)
Musculoskeletal and Connective Tissue Disorders	2 (4.4%)	3 (6.7%)
Renal and Urinary Disorders	2 (4.4%)	1 (2.2%)
Reproductive Systems and Breast Disorders	1 (2.2%)	0 (0.0%)
Respiratory, Thoracic and Mediastinal Disorders	1 (2.2%)	3 (6.7%)
Skin and Subcutaneous Tissue Disorders	2 (4.4%)	1 (2.2%)
Vascular Disorders	2 (4.4%)	0 (0.0%)

[1] No differences greater than 2.2% between SD-809 and placebo for any single adverse event term with the system organ class of investigations.

The numbers of patients reporting adverse events in the system organ classes of psychiatric, nervous system, gastrointestinal and other general disorders are listed below. These body systems are noteworthy because they include many of the symptoms of Huntington's disease.

System organ class	Adverse event term	SD-809 n = 45	Placebo n = 45
PSYCHIATRIC DISORDERS	Insomnia	3 (6.7%)	2 (4.4%)
	Depression/Agitated Depression	2 (4.4%)	3 (6.7%)
	Abnormal Dreams	1 (2.2%)	1 (2.2%)
	Agitation	1 (2.2%)	0 (0.0%)
	Anxiety	1 (2.2%)	1 (2.2%)
	Suicidal Ideation	1 (2.2%)	0 (0.0%)
	Compulsions	0 (0.0%)	1 (2.2%)
	Impulsive Behavior	0 (0.0%)	1 (2.2%)
	Sleep Disorder	0 (0.0%)	3 (6.7%)
NERVOUS SYSTEM DISORDERS	Somnolence	5 (11.1%)	2 (4.4%)
	Dizziness	2 (4.4%)	4 (8.9%)
	Akathisia/Restlessness	1 (2.2%)	1 (2.2%)
	Cognitive Disorder	1 (2.2%)	0 (0.0%)
	Drooling	1 (2.2%)	0 (0.0%)
	Dyskinesia	1 (2.2%)	0 (0.0%)
	Migraine	1 (2.2%)	0 (0.0%)
	Headache	0 (0.0%)	3 (6.7%)
	Loss of Consciousness	0 (0.0%)	1 (2.2%)
	Syncope	0 (0.0%)	1 (2.2%)
GENERAL DISORDERS	Irritability	3 (6.7%)	6 (13.3%)
	Fatigue	3 (6.7%)	2 (4.4%)
	Gait disturbance	1 (2.2%)	0 (0.0%)
	Chest pain	1 (2.2%)	0 (0.0%)
	Hangover	1 (2.2%)	0 (0.0%)
GASTRO- INTESTINAL DISORDERS	Diarrhea	4 (8.9%)	0 (0.0%)
	Dry mouth	4 (8.9%)	3 (6.7%)
	Constipation	2 (4.4%)	1 (2.2%)
	Nausea	1 (2.2%)	2 (4.4%)
	Abdominal pain upper	1 (2.2%)	0 (0.0%)
	Dyspepsia	1 (2.2%)	0 (0.0%)
	Frequent bowel movements	1 (2.2%)	0 (0.0%)
	Gastrointestinal pain	1 (2.2%)	0 (0.0%)
	Vomiting	0 (0.0%)	3 (6.7%)
	Dysphagia	0 (0.0%)	1 (2.2%)
	Flatulence	0 (0.0%)	1 (2.2%)
	Salivary hypersecretion	0 (0.0%)	1 (2.2%)

There was one patient with two serious adverse events (cholecystitis and agitated depression) in the SD-809 group, and one patient with one serious adverse event (exacerbation of chronic obstructive pulmonary disease, or COPD) in the placebo group. The same patient experiencing the serious adverse events in the SD-809 group also reported suicidal ideation, which was not considered a serious adverse event.

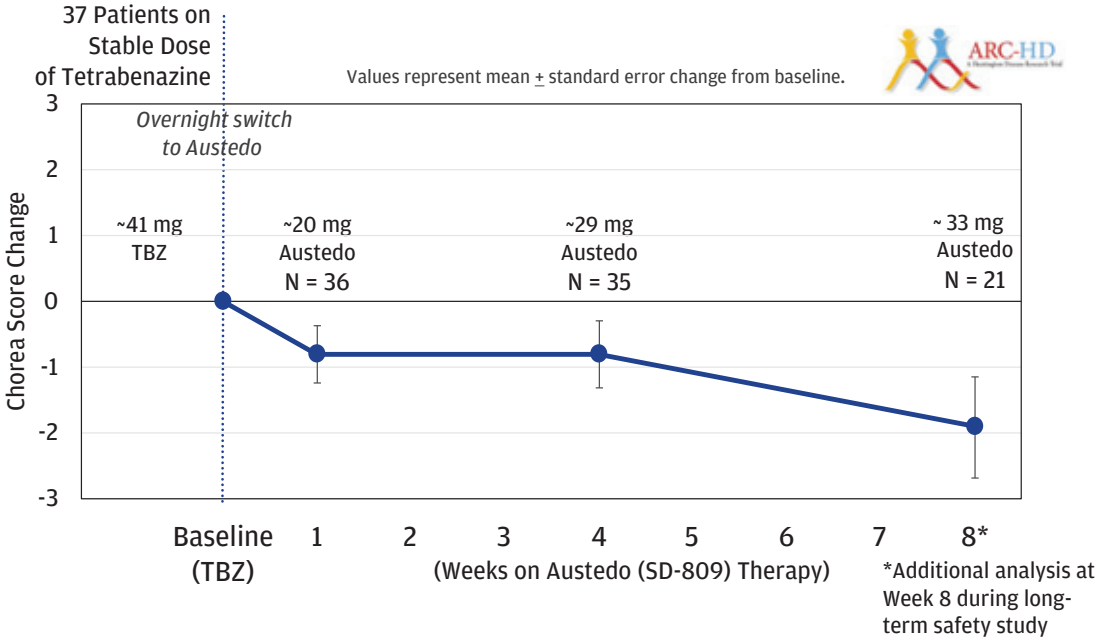
Over 90% of patients who completed the First-HD trial rolled over into ARC-HD, the long-term safety trial of SD-809.

ARC-HD: Open-label switch and long-term safety clinical trial

In parallel with First-HD, we are also conducting our ARC-HD clinical trial of SD-809. One component of ARC-HD, referred to as ARC-HD Switch, was an open-label clinical trial with a four-week "switch" component in 37 patients with chorea associated with Huntington's disease adequately controlled with tetrabenazine. The objectives of ARC-HD Switch were to evaluate the safety of switching subjects on tetrabenazine overnight to SD-809 and to provide guidance to physicians on how to switch such patients to SD-809. We completed the four-week Switch portion of the ARC-HD trial, which has an ongoing long-term safety component. Subjects were required to be on stable doses of tetrabenazine, and to have experienced a therapeutic benefit from this treatment. Although designed primarily as a safety trial, maintenance of chorea control was assessed after switching patients overnight from tetrabenazine to SD-809 (at approximately half the dose of tetrabenazine). The endpoints of ARC-HD Switch were the incidence of adverse events, the observed values and changes in clinical safety parameters and the observed values and changes in TMC values for patients when switched from tetrabenazine (baseline) to SD-809. Total daily dose level at baseline (tetrabenazine) and subsequent visits (SD-809) was summarized using descriptive statistics. Participating patients are eligible to receive open-label SD-809 treatment for one year or longer in a long-term safety trial. ARC-HD Switch was conducted at 13 enrolling centers in the United States, Canada and Australia.

All available data through eight weeks following the switch were included in the analysis. At the time of the analysis in December 2014, data were available from 36 patients at Week 1, 35 patients at Week 4 and 21 patients at Week 8. The mean dose of tetrabenazine at baseline was 41 mg. The mean dose of SD-809 was approximately 20 mg at Week 1, approximately 29 mg at Week 4, and approximately 33 mg at Week 8.

After switching from tetrabenazine to SD-809, chorea was assessed at one and four weeks. Dose adjustments were permitted after Week 1. As indicated in the figure below, the mean TMC score decreased by approximately one point from baseline at Week 1 and Week 4.



- Week 1 change for 36 patients was -0.8 ± 0.4 (mean ± standard error)
- Week 4 change for 35 patients was -0.8 ± 0.5

In addition, data from 21 patients were available at Week 8 at the time of the analysis; these data demonstrated an improvement of 1.9 (± 0.8) points on the TMC score. The improvement from baseline at Week 8 reached statistical significance. We plan to analyze any improvement in TMC score from baseline at Week 8 when the data from all 37 patients are available. It is possible that such data may not be statistically significant. Data for the remaining 16 patients will be available at a future date.

The safety and tolerability experience observed in ARC-HD Switch over the four-week period was consistent with the experience observed in First-HD. The most commonly reported adverse events in ARC-HD Switch patients were somnolence, fall, and nasopharyngitis. In ARC-HD Switch, one patient was hospitalized for pneumonia and another patient was hospitalized overnight for dehydration. Neither of these adverse events was considered related to study medication and both of these events resolved.

Patients from First-HD and ARC-HD Switch rolled over into an open-label, long-term, safety clinical trial, which is the second component of ARC-HD, referred to as ARC-HD Rollover. In ARC-HD Rollover, subjects will return to the clinic at scheduled intervals for evaluation of safety and chorea control. Over 90% of patients who completed the First-HD and ARC-HD Switch trials rolled over into the ARC-HD Rollover long-term safety trial. This trial is not a placebo controlled trial. In ARC-HD Rollover, one patient was briefly hospitalized as a precaution for worsening anxiety, depression and suicidal ideation, events considered possibly related to study medication. Another subject developed depression and suicidal ideation, events considered possibly related to study medication. A third subject was briefly hospitalized for dehydration and confusion, events considered not related to study medication. All of these adverse events resolved.

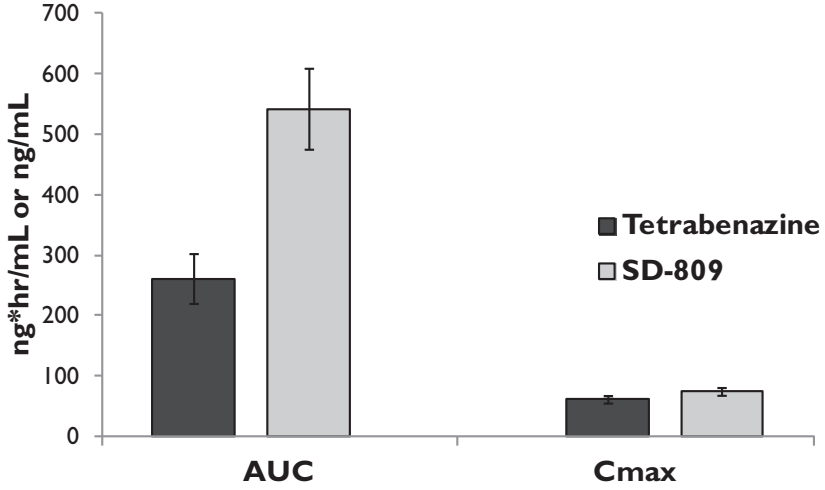
In ARC-HD Rollover, further adjustments of SD-809 dosing may be made, if necessary, but not more than weekly, and in 6 mg daily increments. Four weeks after the last dose of the study drug, patients will be followed up with by phone to evaluate adverse events and concomitant medication usage. The portion of the ARC-HD trial that we believe is needed for submission of the NDA is complete, but the long-term safety trial will remain ongoing.

Summary of data from relevant phase 1 clinical trials of SD-809

Differentiated pharmacokinetic profile

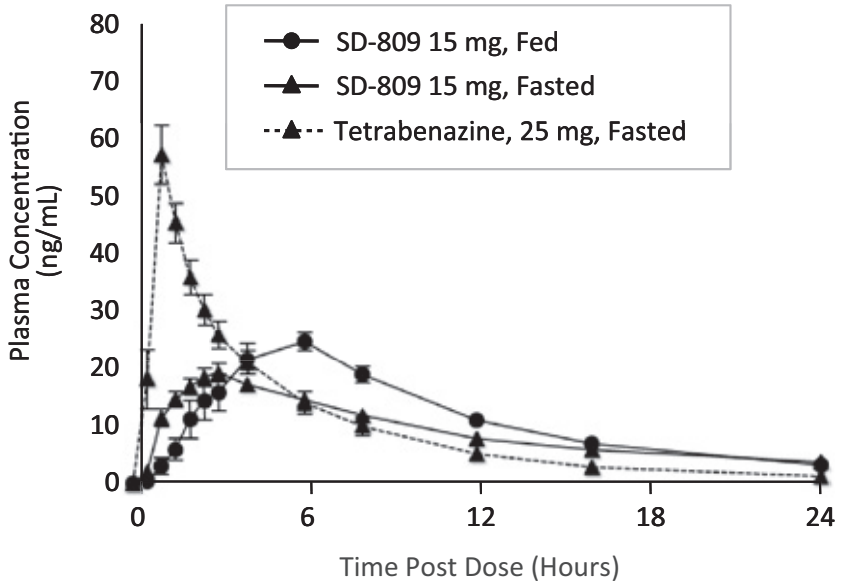
In our Phase 1 single-center, double-blind, randomized, two-period crossover clinical trial, 21 healthy subjects received a single dose of 25 mg of SD-809 or tetrabenazine, and following washout, crossed over to receive the other treatment. The objective of the clinical trial was to compare the pharmacokinetics of SD-809 and tetrabenazine and their respective metabolites and to evaluate the safety and tolerability of a single dose of SD-809. This clinical trial showed that a 25 mg single dose of SD-809 nearly doubled the half-life of the active metabolites (alpha + beta) compared to a 25 mg single dose of tetrabenazine, resulting in more than doubling of the systemic exposure as measured by AUC (area under the drug plasma concentration vs. time curve, a measure of drug exposure). Alpha refers to all stereoisomers and isotopologs of alpha-dihydroxytetrabenazine and beta refers to all stereoisomers and isotopologs of beta-dihydroxytetrabenazine. The Cmax was only slightly higher for SD-809 compared to tetrabenazine, despite the doubling of the systemic exposure.

Pharmacokinetic parameters of total alpha + beta (n=19) comparing 25 mg SD-809 and 25 mg of tetrabenazine



These data suggest that similar exposure can be achieved with SD-809 at approximately half the dose of tetrabenazine and, at the same time, allow Cmax to be reduced by approximately half.

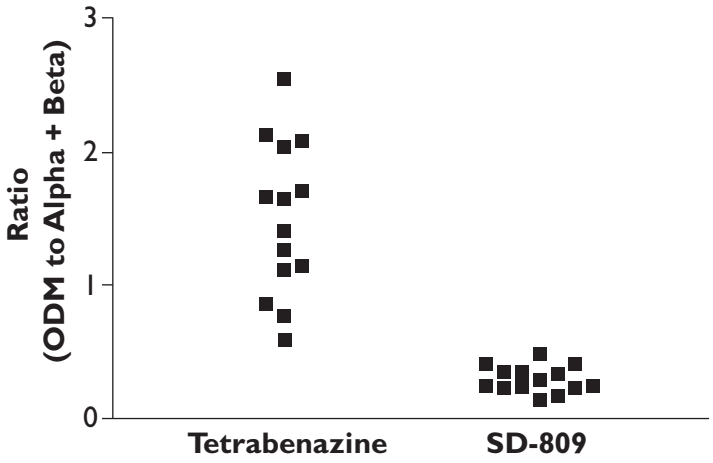
We also conducted a Phase 1 single-center, open-label, five-period, crossover formulation selection clinical trial. The SD-809 active pharmaceutical ingredient was formulated with two different candidate formulations. One of the objectives of the clinical trial was to evaluate and compare the two candidate formulations and to help select a final commercial SD-809 drug product. Each of the 24 healthy subjects received single doses of 25 mg (fasted; per the FDA approval label, it can be administered without regard to meals) of tetrabenazine and 15 mg (fasted or fed) of two clinical formulations of SD-809. Eligible subjects were randomly assigned to a treatment sequence with five periods. The objective of the clinical trial was to evaluate and compare the safety and pharmacokinetics of two candidate formulations of SD-809 relative to tetrabenazine and to evaluate the effect of food (consisting of a high-fat meal) on the bioavailability of candidate formulations of SD-809. In this trial, 15 mg of SD-809 provided similar systemic exposure to active metabolites as 25 mg of tetrabenazine, while substantially reducing Cmax. When SD-809 was given in the fasted state, comparable AUC was achieved as in the fed state (within 15%). The results of this clinical trial, comparing the formulation of SD-809 advanced in the clinic and tetrabenazine (fasted), are shown in the graph below.



Reduced interpatient variability

In the same two-period crossover clinical trial described above, the subjects administered 25 mg of SD-809 demonstrated reduced interpatient variability in drug exposure compared to the same subjects administered 25 mg of tetrabenazine. Specifically, the variability between subjects of the ratio of the ODM to total alpha + beta, a measure of drug metabolism, was reduced significantly for SD-809 compared to tetrabenazine in the same subjects. Each point in the graph below represents an individual subject. All of the subjects in this group were intermediate or extensive metabolizers.

Ratio of ODM to total alpha + beta after 25 mg of SD-809 or tetrabenazine (N=14)



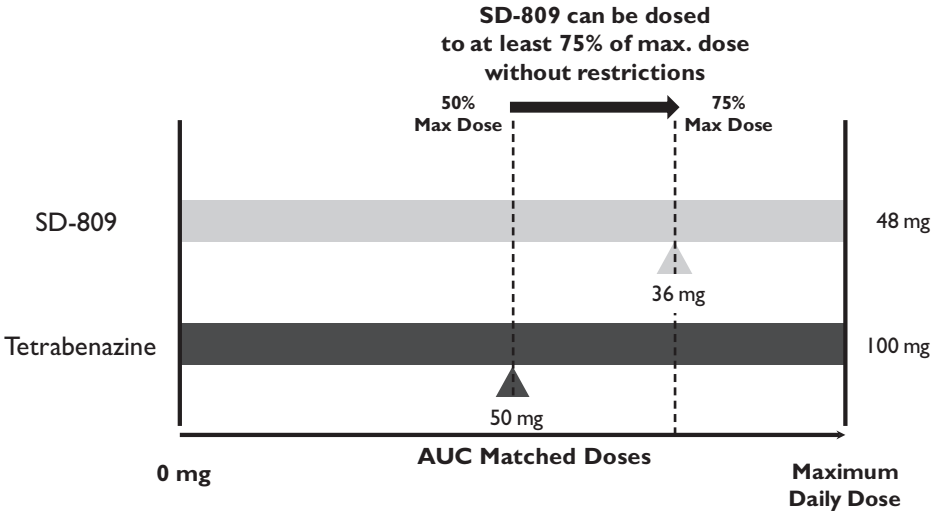
In contrast to the widely distributed values for the ratio of ODM to total alpha + beta in subjects administered tetrabenazine, the same subjects administered SD-809 displayed more uniform drug metabolism with little variability in such ratio. The ratios for SD-809 are less than one, indicating that in each individual, total alpha + beta exceeds the downstream metabolites, whereas with tetrabenazine, total ODM exposures are all generally greater than the total alpha + beta.

Reduced drug interaction

By analyzing exposure and pharmacokinetic data from our clinical trials conducted with SD-809 and tetrabenazine, we were able to establish a dose conversion algorithm to allow us to determine the starting dose of SD-809 appropriate for patients switched from tetrabenazine to SD-809. We determined that the AUC-equivalent dose of SD-809 is approximately half of the daily dose of tetrabenazine (48 mg/day of SD-809 is AUC-equivalent to 100 mg/day of tetrabenazine).

The FDA-approved label for Xenazine states that the daily dose should not exceed 50 mg/day (50% of its maximal dose) in patients taking strong CYP2D6 inhibitors. In contrast, for patients enrolled in our ongoing First-HD clinical trial, the FDA agreed that the total daily dose of SD-809 should not exceed 36 mg/day (75% of its maximal dose) in patients taking strong CYP2D6 inhibitors.

Comparison of maximal dose without restrictions



SD-809 demonstrated the potential for reduced drug interactions and metabolic variability in a Phase 1, single-center, open-label, sequential drug interaction clinical trial. As is typical in drug interaction trials, the objective of the clinical trial was to evaluate the effect of potent CYP2D6 inhibition on the pharmacokinetics of the study drug (in this case a single dose of SD-809). We also evaluated the safety of a single dose of SD-809 in this clinical trial. Twenty-four healthy subjects received a single 22.5 mg dose of SD-809 on Day 1, followed by 20 mg of paroxetine, a potent CYP2D6 inhibitor, on days 4 through 12. On Day 11, 22.5 mg of SD-809 was administered on top of the 20 mg of paroxetine. Pharmacokinetic sampling occurred over 72 hours after each SD-809 treatment.

SD-809 metabolism was less influenced by CYP2D6 inhibition compared to tetrabenazine as described on the Xenazine label. The impact on exposure, measured by AUC of total alpha + beta was three-fold for SD-809 versus five-fold for tetrabenazine, according to the NDA submission materials for tetrabenazine.

SD-809 was well-tolerated with only three subjects reporting one or more treatment-emergent adverse events for either SD-809 alone or SD-809 in combination with paroxetine. Subjects administered SD-809 with paroxetine did not experience any new adverse events that were not observed with SD-809 or paroxetine dosing alone and the rate of adverse events did not increase compared to the SD-809-only or paroxetine-only arms.

Linear dose proportionality

We also conducted a Phase 1 single-center, randomized, open-label, five-way crossover bioavailability clinical trial to evaluate the statistical bioequivalence of various dose levels of SD-809 when administered with a standard meal and evaluate the effect of a high-fat meal on the relative bioavailability of the highest dose strength. Thirty-two subjects received a single dose of 6, 12, 18 or 24 mg of SD-809 after a standard meal, as well as a single dose of 18 mg of SD-809 after a high-fat, high-calorie meal. Pharmacokinetic samples were taken over a 72-hour period following each dose. Statistical bioequivalence for exposure to total alpha + beta over the dose range of 6 to 24 mg of SD-809 was achieved. The pharmacokinetic parameters after a single 18 mg dose of SD-809 after a standard or a high-fat meal were also bioequivalent.

All dosing levels up to 24 mg of SD-809 were generally well-tolerated with similar rates of adverse events (15% at 6 mg to 19% at 24 mg) and there were no dose-dependent increases in adverse events.

Safety observations and metabolic profiling in completed Phase 1 trials

Across all Phase 1 clinical trials, 132 subjects have received single doses of SD-809, ranging from 7.5 to 25 mg, and 24 subjects have received repeated doses of SD-809 for up to five days at 22.5 mg BID. In these trials, no serious adverse events were reported with SD-809 treatment, and all reported adverse events were mild to moderate in intensity. Commonly reported adverse events included headache, somnolence, nausea and dizziness. Multiple dose administration of the drug was associated with a greater incidence of adverse events than with single dose administration. Following exposure to nine days of repeated doses of tetrabenazine and SD-809, adverse events of restlessness, agitation and depressed mood were reported. No significant changes in laboratory parameters, vital signs or ECGs were noted. In addition, triplicate ECG assessments following single doses of SD-809 up to 22.5 mg revealed only small changes in the corrected QT interval.

QT prolongation (the lengthening of time in the heart's electrical cycle) can lead to life-threatening cardiac arrhythmias, with the risk increasing as the degree of prolongation increases. FDA guidance provides that a "negative 'thorough QT/QTc study' is one in which the upper bound of the 95% one-sided confidence interval for the largest time-matched mean effect of the drug on the QTc interval excludes 10 msecs. This definition is chosen to provide reasonable assurance that the mean effect of the study drug on the QT/QTc interval is not greater than around 5 msecs."

QT prolongation is a known effect of tetrabenazine according to its FDA product label. According to the Xenazine FDA label: "QTc Prolongation: The effect of a single 25 or 50 mg dose of tetrabenazine on the QT interval was studied in a randomized, double-blind, placebo controlled crossover study in healthy male and female subjects with moxifloxacin as a positive control. At 50 mg, tetrabenazine caused an approximately 8 msec mean increase in QTc (90% CI: 5.0, 10.4 msec)".

Although not required by the FDA, we recently completed a Phase 1 thorough QT trial in 48 healthy volunteer subjects. This was a single-center, randomized, double-blind, placebo- and positive-controlled six-period crossover study to evaluate the effects of two doses of SD-809 (12mg and 24mg) on cardiac repolarization, based on placebo-corrected, time-matched changes from baseline in the QTcF interval. In addition, the effects of tetrabenazine on cardiac repolarization (also based on placebo-corrected, time-matched changes from baseline in the QTc interval) were evaluated in this study.

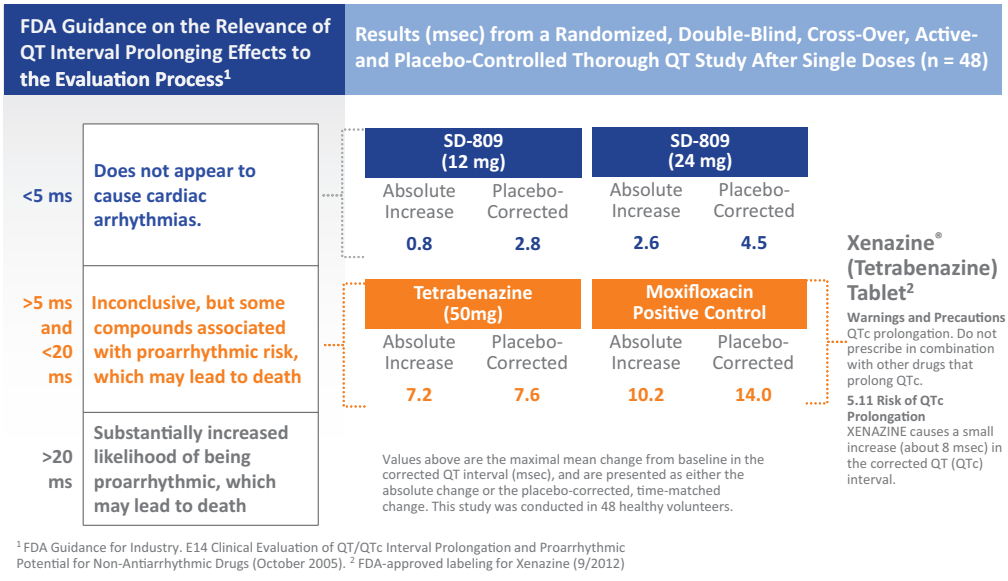
The key outcome measure in this active- and placebo-controlled crossover study was to determine the effect of single doses of SD-809 on the QTc interval. Assay sensitivity was established with a moxifloxacin arm, and a tetrabenazine arm was also included for comparison. A 50 mg dose of tetrabenazine was selected because it was the maximal dose employed in the thorough QT study for Xenazine that led to the warning and precaution

in its product labeling. A 24 mg dose of SD-809 was selected because it provides comparable systemic exposure (AUC) to 50 mg of tetrabenazine, but with a lower Cmax. For SD-809, a 12 mg dose led to a maximal increase in the QTc of approximately 0.8 msec and a 24 mg dose led to maximal increase in the QTc of approximately 2.6 msec. The placebo-corrected time-matched maximal increases in QTc for the 12 and 24 mg doses of SD-809 were 2.8 msec (90% two-sided confidence interval: 0.7 to 4.8) and 4.5 msec (90% two-sided confidence interval: 2.4 to 6.5), respectively. A 50 mg dose of tetrabenazine led to a maximal increase in QTc of approximately 7.2 msec, with a placebo-corrected time-matched maximal increase in QTc of 7.6 msec (90% two-sided confidence interval: 5.6 to 9.5).

The clinical trial demonstrated that at two dose levels, SD-809 had no clinically significant effect on cardiac repolarization as assessed by the QT interval. The tetrabenazine arm included for comparison demonstrated an increase in QTc that is consistent with the effect reported in the FDA label for Xenazine. The clinical trial's assay sensitivity was established by the observation of characteristic QTc prolongation following dosing with moxifloxacin.

According to the International Conference on Harmonisation of Technical Requirements for Registration of Pharmaceuticals for Human Use, or ICH, guidance on the relevance of QT/QTc prolongation, drugs that prolong the mean QT/QTc interval by around 5 msec or less do not appear to cause torsades de pointes, a type of cardiac arrhythmia. Whether this signifies that no increased risk of cardiac arrhythmia exists for these compounds or simply that the increased risk has been too small to detect is not clear. The data on drugs that prolong the mean QT/QTc interval by more than around 5 msec and less than 20 msec are inconclusive, but some of these compounds have been associated with proarrhythmic risk. Drugs that prolong the mean QT/QTc interval by more than 20 msec have a substantially increased likelihood of being proarrhythmic, and might have clinical arrhythmic events captured during drug development. The threshold level of regulatory concern is around 5 msec, as evidenced by an upper bound of the 95% confidence interval around the mean effect on QTc of 10 msec. Note that the upper limit of the 95% one-sided confidence interval is equivalent to the upper limit of the 90% two-sided confidence interval.

This ICH guidance and the results of the thorough QT study are summarized in the figure below:



[1] FDA Guidance for Industry. E14 Clinical Evaluation of QT/QTc Interval Prolongation and Proarrhythmic Potential for Non-Antiarrhythmic Drugs (October 2005). [2] FDA-approved labeling for Xenazine (9/2012)

We plan to include the data from our thorough QT clinical trial in our NDA for SD-809 for the treatment of chorea associated with Huntington's disease.

As one of the Phase 1 clinical trials required by the FDA to enable a Section 505(b)(2) NDA, we completed a single-center, open-label, two-period mass balance and metabolic profiling clinical trial. The objective of the clinical trial was to compare mass balance recoveries and the routes and rates of excretion of the metabolites following administration of single radio-labeled doses of SD-809 and tetrabenazine. The 12 healthy volunteers were divided into two groups, with one group receiving single doses of 25 mg of radio-labeled SD-809 and the other group receiving single doses of 25 mg of radio-labeled tetrabenazine. Pharmacokinetic sampling was taken periodically over 216 hours and urine was also collected through the same time period.

The mass balance recovery of radioactivity after equal doses of SD-809 and tetrabenazine was extensive and similar (92% vs. 91%). The metabolite profile of SD-809 and tetrabenazine reflected the expected effects of deuterium substitution, with subjects treated with SD-809 exhibiting increased exposure to active metabolites (alpha + beta) and no novel major metabolites of SD-809 detected. Each major metabolite of SD-809 was present as a metabolite of tetrabenazine and has been described or referenced in the tetrabenazine NDA.

Ongoing clinical development of SD-809 in additional indications

ARM-TD: Phase 2/3 efficacy dose-titration clinical trial for treatment of tardive dyskinesia

We have initiated our ARM-TD Phase 2/3 clinical trial of SD-809 for the treatment of drug-induced tardive dyskinesia for which we completed a pre-IND meeting with the FDA and submitted an IND in the first quarter of 2014. The IND resulted in a "may proceed" letter, allowing the trial to proceed. ARM-TD will involve approximately 92 subjects, who will be randomized 1:1 to SD-809 or placebo. Patients will titrate to their optimal dosage and be treated for a total of 12 weeks with a titration period that lasts six weeks and a maintenance period that lasts for six weeks. The primary efficacy endpoint will be change in AIMS from baseline to end therapy, which will be assessed by centralized video rating. The safety endpoints include adverse events, vital signs, physical/neurological/laboratory examinations and ECGs. We also intend to offer long-term safety follow-up for the eligible subjects who complete the ARM-TD clinical trial. Based on our recent FDA meeting, we believe that the ARM-TD clinical trial of SD-809 in patients with tardive dyskinesia may qualify as one of the two pivotal trials needed for a 505(b)(2) NDA filing, subject to FDA review. We expect the topline data from this clinical trial to be available in mid-2015. ARM-TD is being conducted at approximately 30 sites in the United States and Europe.

AIM-TD: Phase 3 efficacy fixed-dose clinical trial for treatment of tardive dyskinesia

We have also initiated our second clinical trial of SD-809, AIM-TD, in patients with moderate to severe tardive dyskinesia in 2014, with topline data expected in 2016, potentially allowing us an opportunity to submit an NDA for SD-809 in patients with tardive dyskinesia in 2016 with FDA action expected in 2017. AIM-TD is a Phase 3 randomized, double-blind, placebo-controlled, parallel group trial in patients with moderate to severe drug-induced tardive dyskinesia. We believe it may serve as the second of two pivotal trials to support a labeled indication in tardive dyskinesia. The AIM-TD clinical trial will involve approximately 200 subjects, who will be randomized 1:1:1:1 to three fixed doses of SD-809 or placebo for 12 weeks of treatment. With the exception of fixed dosing in this trial, the design of the AIM-TD clinical trial is similar to that of the ARM-TD trial. As with the ARM-TD trial, the primary efficacy endpoint will be change in AIMS from baseline to Week 12, which will be assessed by blinded centralized video rating. The key secondary efficacy endpoint will be the proportion of subjects who are a treatment success at Week 12, based on the CGIC. The safety endpoints will include adverse events, vital signs, physical/neurological/laboratory examinations and ECGs. We also intend to offer up to one year of open label SD-809 for eligible subjects who successfully complete randomized treatment in the AIM-TD clinical trial. AIM-TD is being conducted at approximately 60 sites in the United States and Europe.

Phase 1b clinical trial in tourette syndrome

We have initiated an open-label preliminary efficacy, pharmacokinetic and safety clinical trial of SD-809 in approximately 20 adolescent patients with tics associated with Tourette syndrome. This clinical trial is being conducted under our current IND. In this clinical trial, subjects will receive treatment for a total of eight weeks. The drug will be titrated to each subject's optimal dosage over the first six weeks, followed by a two-week maintenance period. The efficacy measures in this clinical trial will be a change in the total tic score of Yale Global Tic Severity Scale and the Clinical Global Impression. We also plan to evaluate preliminary efficacy and safety, in addition to studying the pharmacokinetics of SD-809 in adolescents of SD-809. These additional data are expected to further support the dosing assumptions and design elements of a randomized, controlled trial in this patient population and accelerate the development of SD-809 in this indication. We anticipate topline data from this clinical trial to be available by mid-2015. We will decide how to pursue further development and regulatory approval for this indication based on the results of this exploratory clinical trial.

Regulatory strategy for SD-809

We are pursuing a Section 505(b)(2) NDA regulatory strategy, which we expect will allow us to rely in our NDA filing on certain nonclinical and clinical safety findings made by FDA in its approval of the tetrabenazine NDA. Based on our interactions with the FDA, we believe that with the successful completion of First-HD, we will have completed the necessary preclinical studies and clinical trials to submit an NDA under Section 505(b)(2) for SD-809 for the treatment of chorea associated with Huntington's disease. In addition to the results of First-HD, we anticipate that the NDA submission will include the results of ARC-HD Switch, which, if successful, would allow us to provide guidance in our labeling on how to switch patients who are currently on tetrabenazine to SD-809. We also anticipate submitting the available results of ARC-HD Rollover at the time of the NDA filing with the FDA. By pursuing the Section 505(b)(2) regulatory pathway for SD-809, our reliance on the FDA's prior findings of safety from tetrabenazine may require any approved labeling for SD-809 to include, in addition to safety information from our clinical trials, certain safety information that is included in the tetrabenazine label, including warnings and precautions and other safety information. Moreover, since our ongoing ARC-HD long term safety clinical trial is not studying SD-809 in a head-to-head comparison with tetrabenazine, if SD-809 is approved by the FDA, we will not be able to make direct comparative claims regarding the safety and efficacy of SD-809 and tetrabenazine either in labeling or in our promotional materials for SD-809 from this trial. We anticipate submitting our Section 505(b)(2) NDA for SD-809 for the treatment of chorea associated with Huntington's disease and, if approved, expect to launch commercial sales in 2016.

In November 2014, we received orphan drug designation from the FDA for SD-809 for the treatment of Huntington's disease.

Sales and marketing

If we are successful in obtaining regulatory approval for the commercialization of SD-809 for treatment of chorea associated with Huntington's disease, we expect SD-809 would be the only product other than tetrabenazine on the market in the United States approved for this indication. We believe that SD-809 could, over time, capture a significant share of the existing tetrabenazine prescription market and expand the market by treating patients who are currently untreated or under-treated because they cannot tolerate or are at greater risk of side effects from tetrabenazine. We believe that it will be possible for us to commercialize SD-809 for this indication with an initial commercial infrastructure including a small number of sales representatives that call on a focused group of movement disorder neurologists and a select segment of community-based neurologists who manage patients with Huntington's disease. Due to the specialized nature of managing the symptoms of Huntington's disease there are a limited number of treating physicians, which we

believe will enable us to target potential SD-809 prescribers with a small sales force. We expect our commercial organization to launch SD-809, including sales representatives, will initially be approximately 50 employees. We expect approximately an additional 20 employees initially to provide support activities for commercialization of SD-809. We may adjust these numbers in the future as we further refine our commercial plan and based on strategic and business considerations. In the United States, we estimate that approximately 500 movement disorder neurologists are responsible for prescribing about half of all tetrabenazine prescriptions, with the next 500 prescribers accounting for 25% of prescriptions, and the last 1,500-2,000 prescribers accounting for the last 25% of prescriptions, based on extrapolation from IMS Health Institute data. The Huntington Study Group is a network of hundreds of clinical investigators, coordinators and scientists who provide comprehensive care to Huntington's disease patients and families and carry out multi-center clinical research. We have partnered with the Huntington Study Group for our ongoing clinical trials of SD-809 in Huntington's disease. Key opinion leaders from academic movement disorder centers drive the standard of care in this indication; many of these individuals have or will have experience with SD-809 through participation in our clinical trials.

While we plan to focus our initial commercialization efforts on physicians who are responsible for Huntington's disease patients, this sales and marketing infrastructure would serve as the foundation for an expanded focus on physicians who are responsible for tardive dyskinesia and Tourette syndrome patients, subject to marketing approval in these patient populations. Having multiple indications for the same product that can be promoted to an overlapping physician audience would allow us to leverage our commercial infrastructure with these prescribers. In the event SD-809 is approved for the treatment of tardive dyskinesia or Tourette syndrome, we anticipate adding additional sales representatives who will market to psychiatrists and neurologists.

In the United States, tetrabenazine is currently distributed through only three specialty pharmacies. Physicians must fill out a treatment form provided by the manufacturer in order to prescribe the drug, and the drug is shipped directly to patients from one of these three specialty pharmacies. In addition, there are a REMS program and other support services associated with the distribution of tetrabenazine, which are administered through this specialty distribution system. Xenazine is expected to lose market exclusivity related to its orphan drug status for the treatment of chorea associated with Huntington's disease in August 2015. While a generic version of Xenazine may become available in the future as a result of this loss of market exclusivity, we believe that Xenazine's restricted distribution through these three specialty pharmacies, along with the associated support services and the REMS, provide a barrier to the entry of generic drugs. For example, there are over 200 branded drugs that are distributed through specialty pharmacies. Of these, approximately 20 to 30 drugs have a generic alternative to the branded drug. Based on available pricing data on 21 such branded drugs sold exclusively through specialty pharmacies, we found that introduction of generic drugs resulted in an average price reduction of less than 20%. There was a median of three generic drugs available with respect to each of these branded drugs. We believe that pricing for SD-809 will ultimately be determined by a variety of factors, including the nature of the drug label approved by the FDA, safety, tolerability and efficacy profile, distribution system and a host of other factors.

Other programs

We have additional deuterium-containing product candidates in various stages of development that cover a range of disease areas. These programs include SD-560, a deuterium-containing form of pirfenidone, which is currently being evaluated in Phase 1 clinical trials, SD-1077, a deuterium-containing form of levodopa, which we plan to evaluate in Phase 1 proof of concept clinical trials and SD-254, a deuterium-containing form of venlafaxine, which has completed two Phase 1 clinical trials. These and other compounds in our portfolio demonstrate attractive profiles relative to the corresponding non-deuterium-containing compounds, which may enable greater efficacy, reduced dosing frequency and interpatient variability and improved safety and tolerability.

SD-S60 is a deuterium-containing pirfenidone for the treatment of idiopathic pulmonary fibrosis and other fibrotic conditions, which we have tested in vitro and in animal models to date. We are conducting a single-center, double-blind, randomized, two-period crossover clinical trial in healthy volunteers with equal doses of SD-560 or pirfenidone. The objectives of the clinical trial are to compare the pharmacokinetics of SD-560 and pirfenidone and their respective metabolites and to evaluate the safety and tolerability of SD-560 and inform further development activities. We expect the data from this clinical trial to be available by mid-2015. We are pursuing an orphan drug designation for the use of SD-560 in the treatment of idiopathic pulmonary fibrosis in the United States.

SD-1077, a selective deuterium-containing form of levodopa, which is an investigational new drug for the potential treatment of Parkinson's disease, has been shown in preclinical rodent models to improve the half-life of dopamine in the brain resulting in a prolonged treatment effect in these animals, suggesting that SD-1077 may have the potential to prolong the anti-parkinsonian effect in patients. These properties of SD-1077 may enable less frequent dosing, a reduction in the daily dose of levodopa and a reduction in the development of levodopa-induced dyskinesias and other motor complications of Parkinson's disease. SD-1077 has potential to be administered via multiple formulations and/or delivery methods, including orally, inhaled, subcutaneously, and as a pump, among others. We are advancing SD-1077 through preclinical studies in preparation for initiation of clinical development in 2015. These and other compounds in our portfolio could be advanced or partnered in the future based on strategic considerations and availability of resources.

Manufacturing

We do not own or operate manufacturing facilities for the production of SD-809 or any of our other product candidates, nor do we have plans to develop our own manufacturing operations for clinical materials or commercial products in the foreseeable future. We currently depend on third-party contract manufacturing organizations, or CMOs, for all of our required raw materials, drug substance and drug product for our preclinical research and clinical trials.

We currently rely on single suppliers for raw materials including drug substance and single manufacturers for our product candidates and expect to rely on third-party suppliers and manufacturers for the commercial supply of any approved products. We currently employ internal resources and third-party consultants as needed to manage our CMOs. These CMOs offer a comprehensive range of contract manufacturing and packaging services and have successfully handled the scale up of SD-809 in preparation for commercialization.

Competition

Our industry is highly competitive and subject to rapid technological changes. Our potential competitors include large pharmaceutical and biotechnology companies, specialty pharmaceutical and generic drug companies, academic institutions, government agencies and research institutions. Many of our potential competitors have substantially greater financial, technical, commercial and human resources than we do and significantly more experience in the discovery, development and regulatory approval of product candidates and the commercialization of those products. We believe that the key competitive factors that will affect the development and commercial success of SD-809 and the other product candidates that we may develop are their efficacy, safety and tolerability profile, convenience in dosing, product labeling, value and price, in addition to whether there are alternative therapies approved for other indications and prescribed for off-label use and the availability of reimbursement from the government and other third parties. Our commercial opportunity could be reduced if our competitors have products which are better in one or more of these categories.

We expect that, if approved, SD-809 would compete with a number of existing products and other product candidates that target hyperkinetic movement disorders, including certain products that are or may become

generic products. Additionally, the development of new treatment methods for the diseases we are targeting could render our product candidates non-competitive or obsolete.

Huntington's disease. We anticipate that, if approved, SD-809 will compete primarily against Xenazine and, potentially in the future, generic tetrabenazine, for the treatment of chorea associated with Huntington's disease. There are several product candidates in clinical development for the treatment of Huntington's disease. These include Huntexil (prodipidine) and Nerventra (laquinimod), which are being developed by Teva Pharmaceutical Industries; PBT2, which is being developed by Prana Biotechnology Ltd.; SEN0014196 (selisistat), which is being developed by Siena Biotech S.p.A.; Procysbi (cysteamine), which is approved for the treatment of nephropathic cystinosis and is being developed for Huntington's disease by Raptor Pharmaceuticals, Inc.; OMS824, which is being developed by Omeros Corporation; PF-2545920, which is being developed by Pfizer, Inc.; and BN82451, which is being developed by Ipsen. Valeant Pharmaceuticals International owns a modified-release tetrabenazine formulation, but to our knowledge, is not currently developing it and withdrew a planned clinical trial prior to initiating enrollment. We are not aware of any other company that has a modified-release tetrabenazine product candidate in clinical development in the United States.

Tardive dyskinesia. There are currently no approved drugs for the treatment of tardive dyskinesia, but we believe that tetrabenazine is prescribed off-label for this indication. We are aware of several product candidates in clinical development for treatment of tardive dyskinesia including: NBI-98854 (valine ester substituted analog of a single stereoisomer of alpha), which is being developed by Neurocrine Biosciences, Inc.; SNC-102 (acamprosate calcium), which is being developed by Synchroneuron Inc.; and Tardoxal (pyridoxine hydrochloride), which is being developed by Medicure Inc.

Tourette syndrome. There are currently two FDA-approved drugs for the treatment of Tourette syndrome, haloperidol and pimozide, which are generic neuroleptics. These drugs were approved for this indication by the FDA in 1967 and 1983, respectively. We believe that tetrabenazine and guanfacine are prescribed off-label for this indication as well. We are aware of several product candidates in clinical development for the treatment of Tourette syndrome including: ABILIFY (aripiprazole), which is being developed by Otsuka Pharmaceutical Group; NBI-98854 (a valine ester-substituted analog of alpha), which is being developed by Neurocrine Biosciences, Inc.; ecopipam (a synthetic benzazepine derivative), which is being developed by Psyadon Pharmaceuticals Inc.; AZD5213, which is being developed by AstraZeneca plc; CPP-109, which is being developed by Catalyst Pharmaceutical Partners; and EPI-754, which is being developed by Edison Pharmaceuticals, Inc.

Competitors developing deuterium-containing compounds include Concert Pharmaceuticals Inc. However, we are not aware of any company developing a deuterium-substituted tetrabenazine.

Intellectual property and exclusivity

We have been building and continue to expand our intellectual property portfolio relating to our product candidates, including SD-809. We strive to protect and enhance the proprietary technologies that we believe are important to our business and seek patent protection, where appropriate, in the United States and internationally for our product candidates, their methods of use and any other inventions that are important to the development of our business. Our policy is to actively seek to protect our proprietary position by, among other things, filing patent applications in the United States and abroad (including Europe and certain other countries when appropriate) relating to proprietary technologies that are important to the development of our business. However, patent protection may not afford us with complete protection against competitors who seek to circumvent our patents. We also rely on trade secrets, know-how, continuing technological innovation and in-licensing opportunities to develop, strengthen and maintain our proprietary position. We cannot be sure that

patents will be granted with respect to any of our pending patent applications or with respect to any patent applications filed by us in the future, nor can we be sure that any of our existing patents or any patents that may be granted to us in the future will be commercially useful in protecting our technology.

Our success will depend significantly on our ability to obtain and maintain patent and other proprietary protection for the technologies, inventions, know-how and products we consider important to our business, defend our patents, preserve the confidentiality of our trade secrets and operate our business without infringing the patents and proprietary rights of third parties.

Our SD-809 patent portfolio currently includes issued composition of matter patents in the United States (US 8,524,733), Europe (EP 2326643), Japan (JP 5616345), New Zealand (591615), and China (CN 102186848), and an allowed composition of matter application in Australia. The issued U.S. composition of matter patent is expected to expire in March 2031, while the European patent is expected to expire in September 2029, before any patent term extension, or PTE, or equivalent to which we may be entitled under the Hatch-Waxman Act or equivalent laws in other jurisdictions where we have issued patents. We have also received a Notice of Allowance from the United States Patent and Trademark Office for claims covering pharmaceutical compositions of SD-809. In addition we have several pending patent applications in the United States and other countries that, if issued, will cover composition of matter, as well as methods of treatment, manufacture, formulations, indications, dose ranges and other applications and aspects of SD-809, and have the potential to extend the patent coverage beyond 2031. We solely own all the issued patents and the pending patent applications in our SD-809 patent portfolio.

Our SD-254 patent portfolio currently includes issued composition of matter (US 7,456,317) and method of treatment (US 8,138,226) patents in the United States, and issued composition of matter and method of treatment patents in Canada (CA 2,631,581), China (CN 101336226), Hong Kong (1125626B), Japan (JP 5302005), South Korea (KR 1068180) and Mexico (MX 275566). The issued U.S. composition of matter patent is expected to expire in November 2026 and the method of treatment patent is expected to expire in September 2028, before any patent term extension or equivalent to which we may be entitled under the Hatch-Waxman Act.

Our SD-560 patent portfolio currently includes an issued composition of matter patent in the United States (US 8,383,823), an issued method of treatment patent in the United States (US 8,680,123), an allowed food effect application in the United States (U.S. Application Ser. No. 14/003,106), and issued composition of matter and method of treatment patents in Europe (EP 2170828), Japan (JP 5587184) and Australia (AU 2008265595). The issued U.S. patents are expected to expire in June 2028, and the European patent is expected to expire in June 2028, before any patent term extension or equivalent to which we may be entitled under the Hatch-Waxman Act or equivalent laws in other jurisdictions where we have issued patents.

Our SD-1077 is currently in pre-clinical development. We have been developing SD-1077 in collaboration with Imphar AG, a private Germany-based drug development company, and we recently entered into a share purchase agreement to acquire the remaining rights to SD-1077, and related intellectual property, through the acquisition of Imphar AG. Imphar AG had previously granted us exclusive U.S. and select worldwide rights while retaining European and additional worldwide rights, all of which will be transferred to us, along with the related intellectual property, pursuant to the share purchase agreement, subject to the satisfaction of customary closing conditions.

Our SD-1077 patent portfolio currently includes issued composition of matter patents in the United States (US 8,168,820 and US 8,247,603), Europe (EP 1613571) and several other countries. We have an additional 40 issued or allowed patents and 57 patent families in prosecution in our broader patent portfolio covering, among other things, the deuterium-containing form of 56 drugs.

The U.S. patent system permits the filing of provisional and non-provisional patent applications. A non-provisional patent application is examined by the U.S. Patent and Trademark Office, or U.S. PTO, and can mature into a patent once the U.S. PTO determines that the claimed invention meets the standards for patentability. A provisional patent application is not examined for patentability, and automatically expires 12 months after its filing date. As a result, a provisional patent application cannot mature into a patent. The requirements for filing a provisional patent application are not as strict as those for filing a non-provisional patent application. Provisional applications are often used, among other things, to establish an early filing date for a subsequent non-provisional patent application. The term of individual patents depends upon the legal term of the patents in the countries in which they are obtained. In most countries in which we file, the patent term is 20 years from the earliest date of filing a non-provisional patent application. In the United States, a patent's term may be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the U.S. PTO in granting a patent. Alternatively, a patent's term may be shortened if a patent is terminally disclaimed over another patent.

The filing date of a non-provisional patent application is used by the U.S. PTO to determine what information is prior art when it considers the patentability of a claimed invention. If certain requirements are satisfied, a non-provisional patent application can claim the benefit of the filing date of an earlier filed provisional patent application. As a result, the filing date accorded by the provisional patent application may supersede information that otherwise could preclude the patentability of an invention.

The term of a patent that covers an FDA-approved drug may also be eligible for PTE, which permits patent term restoration as compensation for the patent term lost during the FDA regulatory review process. The Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Amendments, permits a PTE of up to five years beyond the expiration of the patent in certain circumstances. The length of the PTE is related to the length of time the drug is under regulatory review. Patent extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval and only one patent applicable to an approved drug may be extended. Similar provisions are available in Europe and certain other foreign jurisdictions to extend the term of a patent that covers an approved drug. In the future, if and when our pharmaceutical product candidates receive FDA or other regulatory approval, we may be able to apply for PTEs on patents covering those products. Depending upon the timing, duration and specifics of FDA approval, if any, of SD-809 or our other product candidates, one or more of our U.S. patents may be eligible for limited PTE.

Government regulation

FDA approval process

In the United States, pharmaceutical products are subject to extensive regulation by the FDA pursuant to the Federal Food, Drug and Cosmetic Act, or FDCA. The FDCA and other federal and state statutes and regulations, govern, among other things, the research, development, testing, manufacture, storage, recordkeeping, approval, labeling, promotion and marketing, distribution, post-approval monitoring and reporting, sampling and import and export of pharmaceutical products. Failure to comply with applicable FDA or other requirements may subject a company to a variety of administrative or judicial sanctions, such as FDA refusal to approve pending applications, clinical holds, warning or untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, withdrawal of product from the market, injunctions, fines, civil penalties and criminal prosecution.

FDA approval is required before any new unapproved drug or dosage form, including a new use of a previously approved drug, can be marketed in the United States. The process required by the FDA before a new drug may be marketed in the United States generally involves:

- completion of pre-clinical laboratory and animal testing and formulation studies in compliance with the FDA's current good laboratory practice, or cGLP, regulations;
- submission to the FDA of an IND for human clinical testing which must become effective before human clinical trials may begin in the United States;
- approval of clinical protocols and informed consent documents by an independent institutional review board, or IRB, at each clinical trial site before each clinical trial may be initiated;
- performance of adequate and well-controlled human clinical trials to establish the safety and efficacy of the proposed drug product for each intended use in accordance with federal regulations and current good clinical practices, or cGCPs, which are international standards meant to protect the rights and health of patients and to define the roles of clinical trial sponsors, administrators and monitors;
- development of a manufacturing process, formulation and packaging process and analytical testing plan which will provide investigational drug product of appropriate quality and quantity for the intended use;
- satisfactory completion of an FDA pre-approval inspection of the facility or facilities at which the product is manufactured to assess compliance with the FDA's current good manufacturing practice, or cGMP, regulations to assure that the facilities, methods and controls are adequate to preserve the drug's identity, strength, quality and purity;
- submission to the FDA of an NDA;
- potential review by an FDA advisory committee, if applicable; and
- FDA review and approval of the NDA.

The preclinical and clinical testing and approval process takes many years and the actual time required to obtain approval, if any, may vary substantially based upon the type, complexity and novelty of the product or disease.

Preclinical tests include laboratory evaluation of product chemistry, formulation and toxicity, as well as animal studies to assess the characteristics and potential safety and efficacy of the product. The conduct of the preclinical tests must comply with federal and other regulations and requirements, including cGLPs. The results of preclinical testing are submitted to the FDA as part of an IND along with other information, including information about product chemistry, manufacturing and controls, analytical data, any available clinical data or literature to support the use of the investigational drug in humans and a proposed clinical trial protocol. The IND may rely in part on information contained in already approved drug applications, where a 505(b)(2) NDA is planned. The IND is a request for authorization from the FDA to administer an investigational drug product to humans. Long-term preclinical tests, such as animal tests of reproductive toxicity and carcinogenicity, may continue after the IND is submitted.

An IND must become effective before clinical trials may begin. An IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA, within the 30-day time period, raises concerns or questions relating to one or more proposed clinical trials, including concerns that insufficient information is provided to determine whether the potential risks to which human research subjects may be exposed are unreasonable. In such a case, the IND may be placed on clinical hold and the IND sponsor and the FDA must resolve any outstanding concerns before clinical trials can begin. Accordingly, submission of an IND may or may not result in the FDA allowing clinical trials to commence.

A separate submission to an existing IND must also be made for each successive clinical trial conducted during product development. Further, an independent IRB, covering each site proposing to conduct the clinical trial must review and approve the plan for any clinical trial and informed consent information for subjects before the trial commences at that site and it must monitor the clinical trial until completed. Clinical trials involve the administration of the investigational new drug to healthy volunteers or patients under the supervision of a qualified investigator in accordance with cGCP requirements, which include the requirement that all research subjects provide their informed consent in writing for their participation in any clinical trial. Clinical trials are conducted under protocols detailing, among other things, the objectives of the clinical trial, the parameters to be used in monitoring safety, and the efficacy criteria to be evaluated. Sponsors of clinical trials generally must register and report, at the NIH-maintained website ClinicalTrials.gov, key parameters of certain clinical trials.

For purposes of an NDA submission and approval, human clinical trials are typically conducted in the following sequential phases, which may overlap or be combined:

- *Phase 1*: In Phase 1, through the initial introduction of the drug into healthy human subjects or patients, the drug is tested to assess metabolism, pharmacokinetics, pharmacological actions, side effects associated with increasing doses, and, if possible, early evidence on effectiveness;
- *Phase 2*: Phase 2 usually involves trials in a limited patient population to determine the effectiveness of the drug for a particular indication, dosage tolerance and optimum dosage and to identify common adverse effects and safety risks;
- *Phase 3*: Phase 3 trials are undertaken to obtain the additional information about clinical efficacy and safety in a larger number of patients, typically at geographically dispersed clinical trial sites, to permit the FDA to evaluate the overall benefit-risk relationship of the drug and to provide adequate information for the labeling of the drug. In most cases, the FDA requires two adequate and well controlled clinical trials to confirm the safety and efficacy of the drug. A single Phase 3 trial with other confirmatory evidence may be sufficient in rare instances where the clinical trial is a large multicenter trial demonstrating internal consistency and a statistically persuasive finding of a clinically meaningful effect on mortality, irreversible morbidity or prevention of a disease with a potentially serious outcome and confirmation of the result in a second trial would be practically or ethically impossible.

The FDA, the IRB, or the 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 risk or for failure to comply with the FDA's or IRB's requirements. 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 clinical trial. The trial sponsor may also suspend or terminate a clinical trial based on evolving business objectives and/or competitive climate.

After completion of the required clinical testing, an NDA is prepared and submitted to the FDA. FDA approval of the NDA is required before marketing of the product may begin in the United States. The NDA must include, among other things, the results of all preclinical, clinical and other testing, including negative or ambiguous results as well as positive findings and a compilation of data relating to the product's pharmacology, chemistry, manufacture and controls, as well as proposed labeling. Under federal law, the submission of most NDAs is subject to a substantial application user fee, currently exceeding $2,335,000, and the manufacturer and/or sponsor under an approved NDA are also subject to annual product and establishment user fees, currently exceeding $110,000 per product and $569,000 per establishment. These fees are typically increased annually. However, NDA user fees may be exempted for the first NDA submission by a sponsor who is defined as a small business.

The FDA has 60 days from its receipt of an NDA to determine whether the application will be accepted for filing based on the agency's threshold determination that it is sufficiently complete to permit substantive review. The FDA may request additional information rather than accept an NDA for filing. In this event, the NDA must be resubmitted with the additional information and is subject to payment of additional user fees. The resubmitted application is also subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review. Under the Prescription Drug User Fee Act, or PDUFA, the FDA has agreed to certain performance goals in the review of NDAs through a two-tiered classification system, Standard Review and Priority Review. Priority Review designation is given to drugs that offer major advances in treatment, or provide a treatment where no adequate therapy exists. The FDA endeavors to review applications subject to Standard Review within ten to twelve months from the date of receipt of the NDA by the FDA and applications subject to Priority Review within six to eight months from the date of receipt of the NDA by the FDA. In both cases, the longer review time applies if a drug is a new molecular entity.

The FDA may refer applications for novel drug products or drug products which present difficult questions of safety or efficacy to a public advisory committee for review, evaluation and recommendation as to whether the application should be approved and under what conditions. Information presented and discussed at an FDA advisory committee meeting is open to the public except for commercial confidential information. The FDA is not bound by the recommendation of an advisory committee, but it typically follows such recommendations.

Before approving an NDA, the FDA may inspect one or more clinical sites to assure compliance with cGCP requirements. Additionally, the FDA will inspect the facility or the facilities at which the drug is manufactured. The FDA will not approve the product unless it determines that the manufacturing process and facilities are in compliance with cGMP requirements and are adequate to assure consistent production of the product within required specifications. To support marketing approval, the data submitted must also be sufficient in quality and quantity to establish the safety and effectiveness of the investigational drug product to the satisfaction of the FDA.

After the FDA evaluates the NDA and the manufacturing facilities, it issues either 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 for an application is complete and that the application is not ready for approval. A complete response letter generally outlines the deficiencies in the submission and may require substantial additional testing or information in order for the FDA to reconsider the application. Even with submission of this additional information, the FDA may ultimately decide that an application does not satisfy the regulatory criteria for approval. If, or when, the deficiencies have been addressed to the FDA's satisfaction in a resubmission of the NDA, the FDA may issue an approval letter.

As a condition of NDA approval, the FDA may require a REMS to ensure that the benefits of the drug outweigh the potential risks. If the FDA determines a REMS is necessary during review of the application, the drug sponsor must agree to the REMS plan at the time of approval. A REMS may be required to include various elements, such as a medication guide or patient package insert, a communication plan to educate healthcare providers of the drug's risks, limitations on who may prescribe or dispense the drug, or other elements to assure safe use, such as special training or certification for prescribing or dispensing, dispensing only under certain circumstances, special monitoring, and the use of patient registries. In addition, the REMS must include a timetable to periodically assess the strategy. The requirement for a REMS can materially affect the potential market and profitability of a drug.

Moreover, product approval may require substantial post-approval testing and surveillance to monitor the drug's safety or efficacy and the FDA has the authority to prevent or limit further marketing of a product based on the results of these post-marketing programs. Once granted, product approvals may be withdrawn if

compliance with regulatory standards is not maintained or problems are identified following initial marketing. Drugs may be marketed only for the approved indications and in accordance with the provisions of the approved label and, even if the FDA approves a product, it may limit the approved indications for use for the product or impose other conditions, including labeling or distribution restrictions or other risk-management mechanisms.

Future changes to some of the conditions established in an approved application, including changes in indications, labeling, or manufacturing processes or facilities, typically require submission and FDA approval of a new NDA or NDA supplement before the change can be implemented, which may require us to develop additional data or conduct additional pre-clinical studies and clinical trials. An NDA supplement for a new indication typically requires clinical data similar to that in the original application and the FDA uses the similar procedures in reviewing NDA supplements as it does in reviewing NDAs.

Post-approval requirements

Once an NDA is approved, a product will be subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to drug listing and registration, recordkeeping, periodic reporting, product sampling and distribution, adverse event reporting and advertising, marketing and promotion, including standards and regulations for direct to consumer advertising, off-label promotion, industry-sponsored scientific and educational activities and promotional activities involving the internet. Drugs may be marketed only for the approved indications and in accordance with the provisions of the approved labeling. While physicians may prescribe for off-label uses, manufacturers are restricted from promoting their approved products for uses outside of the approved indications on 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.

The FDA may require post-approval studies or clinical trials, known as Phase 4 clinical trials, if the FDA finds that scientific data, including information regarding related drugs, suggest that they would be appropriate. The purpose of such studies would be to assess a known serious risk or signals of serious risk related to the drug or to identify an unexpected serious risk when available data indicate the potential for a serious risk. The FDA may also require a labeling change if it becomes aware of new safety information that it believes should be included in the labeling of a drug. The FDA also may require REMS to monitor the effects of an approved product or to ensure that the drug's benefits outweigh its risks, or the FDA may place conditions on an approval that could restrict the distribution or use of the product.

In addition, after approval, quality-control, drug manufacture, packaging and labeling procedures must continue to conform to cGMPs, which impose extensive procedural, substantive and record-keeping requirements. Drug manufacturers and certain of their subcontractors are required to register their establishments with the FDA and certain state agencies. Registration with the FDA subjects entities to periodic unannounced and announced inspections by the FDA, during which the agency inspects manufacturing facilities to assess compliance with cGMPs. Accordingly, manufacturers must continue to expend time, money and effort in the areas of production and quality-control to maintain compliance with cGMPs. Regulatory authorities may withdraw product approvals or request product recalls if a company fails to comply with regulatory standards, if it encounters problems following initial marketing, or if previously unrecognized problems are subsequently discovered. In addition, regulatory authorities may take other enforcement action, including, among other things, untitled or warning letters, the seizure of products, injunctions, consent decrees placing significant restrictions on or suspending manufacturing operations, refusal to approve pending applications or supplements to approved applications, civil penalties and criminal prosecution.

In addition, the distribution of prescription pharmaceuticals is subject to the Prescription Drug Marketing Act, which regulates the distribution of drugs and drug samples at the federal level, and sets minimum standards for the registration and regulation of drug distributors by the states. A growing majority of states also impose certain drug pedigree requirements on the sale and distribution of prescription drugs.

The hatch-waxman amendments

Section 505(b)(2) NDAs

As an alternative path to FDA approval for modifications to formulations or uses of products previously approved by the FDA, an applicant may submit an NDA under Section 505(b)(2) of the FDCA. Section 505(b)(2) was enacted as part of the Hatch-Waxman Amendments to the FDCA and enables the applicant to rely, in part, on the FDA's previous approval of a similar product, or published literature, in support of its application. Section 505(b)(2) permits the filing of an NDA where at least some of the information required for approval comes from studies not conducted by, or for, the applicant and for which the applicant has not obtained a right of reference. If the Section 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 studies or clinical trials of the new product. The FDA may also require companies to perform additional studies or measurements, including clinical trials, to support the change from the approved reference drug. The FDA may then approve the new product candidate for all, or some, of the label indications for which the reference drug has been approved or for any new indication sought by the Section 505(b)(2) applicant.

ANDA approval process

The Hatch-Waxman Amendments also established an abbreviated FDA approval process for drugs that are shown to be bioequivalent to drugs previously approved by the FDA through the NDA process. Approval to market and distribute these drugs is obtained by filing an abbreviated new drug application, or ANDA, with the FDA. An ANDA provides for marketing of a drug product that has the same active ingredients in the same strengths and dosage form as the listed drug and has been shown to be bioequivalent to the listed drug. An ANDA is a comprehensive submission that contains, among other things, data and information pertaining to the active pharmaceutical ingredient, drug product formulation, specifications and stability of the generic drug, as well as analytical methods, manufacturing process validation data and quality control procedures. ANDAs are termed abbreviated because they generally do not include preclinical and clinical data to demonstrate safety and effectiveness. Instead, a generic applicant must demonstrate that its product is bioequivalent to the innovator drug. Drugs approved in this way are commonly referred to as "generic equivalents" to the listed drug and can often be substituted by pharmacists under prescriptions written for the original listed drug.

Orange book listing

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, each of the patents listed in the application for the drug is then published in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations, commonly known as 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 Section 505(b)(2) NDA referencing a drug listed in the Orange Book must certify to the FDA that (1) no patent information on the drug product that is the subject of the application has been submitted to the FDA; (2) such patent has expired; (3) the date on which such patent expires; or (4) 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 Section 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 NDA holder for the reference drug and/or 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 Section 505(b)(2) application also will not be approved until any applicable non-patent exclusivity listed in the Orange Book for the reference drug has expired as described in further detail below.

Non-patent exclusivity

In addition to patent exclusivity, the holder of the NDA for the listed drug may be entitled to a period of non-patent exclusivity, during which the FDA cannot approve an ANDA or Section 505(b)(2) application that relies on the listed drug. For example, a pharmaceutical manufacturer may obtain five years of non-patent exclusivity upon NDA approval of a new chemical entity, or NCE, which is a drug that contains an active moiety that has not been approved by the FDA in any other NDA. An "active moiety" is defined as the molecule or ion responsible for the drug substance's physiological or pharmacologic action. During the five year exclusivity period, the FDA cannot accept for filing any ANDA seeking approval of a generic version of that drug or any Section 505(b)(2) NDA for the same active moiety and that relies on the FDA's findings regarding that drug, except that the FDA may accept an application for filing after four years if the follow-on applicant makes a paragraph IV certification.

A drug, including one approved under Section 505(b)(2), may obtain a three-year period of exclusivity for a particular condition of approval, or change to a marketed product, such as a new formulation for a previously approved product, if one or more new clinical trials (other than bioavailability studies) was essential to the approval of the application and was conducted/sponsored by the applicant. Should this occur, the FDA would be precluded from approving any ANDA or Section 505(b)(2) application for the protected modification until after that three-year exclusivity period has run. However, unlike NCE exclusivity, the FDA can accept an application and begin the review process during the exclusivity period.

Patent term extension

After NDA approval, owners of relevant drug patents may apply for up to a five year patent extension. The allowable PTE is calculated as half of the drug's testing phase—the time between IND application and NDA submission—and all of the review phase—the time between NDA submission and approval up to a maximum of five years. The time can be shortened if the FDA determines that the applicant did not pursue approval with due diligence. The total patent term after the extension may not exceed 14 years.

For patents that might expire during the application phase, the patent owner may request an interim patent extension. An interim patent extension increases the patent term by one year and may be renewed up to four times. For each interim patent extension granted, the post-approval patent extension is reduced by one year. The director of the U.S. PTO must determine that approval of the drug covered by the patent for which a patent extension is being sought is likely. Interim patent extensions are not available for a drug for which an NDA has not been submitted.

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 designation to a drug 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 developing and making a drug 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 a rare disease or condition, the FDA will grant orphan designation for that product for the orphan disease indication, assuming that the same drug has not already been approved for the indication for which the sponsor is seeking orphan designation. If the same drug has already been approved for the indication for which the sponsor is seeking orphan designation, the sponsor must present a plausible hypothesis of clinical superiority in order to obtain orphan designation. Orphan designation must be requested before submitting an NDA. After the FDA grants orphan designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. Orphan designation, however, does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

Orphan designation may provide manufacturers with benefits such as research grants, tax credits, PDUFA application fee waivers, and eligibility for orphan drug exclusivity. If a product that has orphan 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. 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 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 has exclusivity or obtain approval for the same product but for a different indication for which the orphan drug has exclusivity.

In November 2014, we received orphan drug designation of SD-809 for the treatment of Huntington's disease from the FDA.

Expedited review and accelerated approval programs

A sponsor may seek approval of its product candidate under programs designed to accelerate the FDA's review and approval of NDAs. For example, fast track designation may be granted to a drug intended for treatment of a serious or life-threatening disease or condition that has potential to address unmet medical needs for the disease or condition. Under the fast track program, the sponsor of a new drug candidate may request that the FDA designate the drug candidate for a specific indication as a fast track drug concurrent with, or after, the filing of the IND for the drug candidate. The FDA must determine if the drug candidate qualifies for fast track designation within 60 days of receipt of the sponsor's request. The fast track designation may be withdrawn by the FDA if the FDA believes that the designation is no longer supported by data emerging in the clinical trial process. The key benefits of fast track designation are the eligibility for Priority Review, rolling review (submission of portions of an application before the complete marketing application is submitted) and accelerated approval, if relevant criteria are met.

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 or eight months after receipt of the NDA by the FDA, depending on whether the drug is a new molecular entity. Priority Review is granted where there is evidence that the proposed product would be a significant improvement in the safety or effectiveness of the treatment, diagnosis, or prevention of a serious condition. If criteria are not met for Priority Review, the application is subject to the standard FDA review period of ten to twelve months after receipt of the NDA by the FDA, depending on whether the drug is a new molecular entity. Priority Review designation does not change the scientific/medical standard for approval or the quality of evidence necessary to support approval.

Under the accelerated approval regulations, the FDA may approve an NDA on the basis of either a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, 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. In clinical trials, a surrogate endpoint is a measurement of laboratory or clinical signs of a disease or condition that substitutes for a direct measurement of how a patient feels, functions, or survives. Surrogate endpoints can often be measured more easily or more rapidly than clinical endpoints. A drug 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, will allow the FDA to withdraw the drug from the market on an expedited basis. All promotional materials for drug candidates approved under accelerated regulations are subject to prior review by the FDA.

In addition, the FDA Safety and Innovation Act, which was enacted and signed into law in 2012, established a new breakthrough therapy designation. A sponsor may seek FDA designation of its product candidate as a breakthrough therapy if the drug candidate 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. Under the breakthrough therapy program, the sponsor of a new drug candidate may request that the FDA designate the drug candidate for a specific indication as a breakthrough therapy concurrent with, or after, the filing of the IND for the drug candidate. The FDA must determine if the drug candidate qualifies for breakthrough therapy designation within 60 days of receipt of the sponsor's request.

Other healthcare laws and compliance requirements

In the United States, the research, manufacturing, distribution, marketing, sale and promotion of drug products are subject to numerous regulations by various federal, state and local authorities in addition to the FDA including, but not limited to, the U.S. Federal Communications Commission, the U.S. Department of Justice, the U.S. Department of Health and Human Services, and its various enforcement divisions, such as the Centers for Medicare & Medicaid Services, or CMS, the Office of Inspector General, the Office for Human Research Protections, and the Office of Research Integrity, state Attorneys General, state Medicaid Fraud Control Units, and other state and local government agencies. If we obtain FDA approval for any of our product candidates and begin commercializing those products in the United States, our operations may be directly, or indirectly through our customers, subject to various federal and state fraud and abuse laws, including, without limitation, the federal Anti-Kickback Statute, the federal False Claims Act, and physician sunshine laws and regulations. These laws may impact, among other things, our proposed sales, marketing and education programs. In addition, we may be subject to patient privacy regulation by both the federal government and the states in which we conduct our business.

The federal Anti-Kickback Statute prohibits, among other things, any person or entity, including a prescription drug manufacturer, or a party acting on its behalf, from knowingly and willfully soliciting, receiving, offering or paying any remuneration, directly or indirectly, overtly or covertly, in cash or in kind in return for the purchase, recommendation, leasing, ordering or furnishing of an item or service, for which payment may be made in whole or in part under a federal healthcare program such as the Medicare and Medicaid programs. This statute has been interpreted broadly to apply to, among other things, arrangements between pharmaceutical manufacturers, on one hand, and prescribers, purchasers, and formulary managers, on the other. The term "remuneration" expressly includes kickbacks, bribes, or rebates and also has been broadly interpreted to include anything of value, including for example, gifts, discounts, the furnishing of supplies or equipment, credit arrangements, payments of cash, waivers of payments, ownership interests and providing anything at less than its fair market value. There are a number of statutory exceptions and regulatory safe harbors protecting certain business arrangements from prosecution. Failure to meet all of the requirements of a particular applicable statutory exception or safe harbor does not make the conduct per se illegal under the Anti-Kickback Statute. Instead, the legality of the arrangement will be evaluated on a case-by-case basis based on a cumulative review of all of its facts and circumstances. Our practices may not meet all of the criteria for safe harbor protection from federal Anti-Kickback Statute liability in all cases. Further, many states have adopted laws similar to the federal Anti-Kickback Statute and some of these state laws may be broader in scope in that some of these state laws extend to all payors and may not contain safe harbors.

The federal civil False Claims Act prohibits, among other things, any person or entity from knowingly presenting, or causing to be presented, a false or fraudulent claim for payment or approval by a federal healthcare program. The "qui tam" provisions of the False Claims Act allow a private individual to bring civil actions on behalf of the federal government alleging that the defendant has submitted a false claim to the federal government, and potentially to share in any monetary recovery. In recent years, the number of suits brought by private individuals has increased dramatically. In addition, various states have enacted false claims laws analogous to the False Claims Act. Many of these state laws are broader in scope and apply to all payors and, therefore, are not limited to only those claims submitted to the federal government. There are many potential bases for liability under the False Claims Act. Liability arises, primarily, when an entity knowingly submits, or causes another to submit, a false claim for reimbursement to the federal government. The False Claims Act has been used to assert liability on the basis of kickbacks and other improper referrals, improperly reported government pricing metrics such as Best Price or Average Manufacturer Price, and improper promotion of off-label uses not expressly approved by the FDA in a drug's label. Our future activities relating to the reporting of discount and rebate information and other information affecting federal, state and third party reimbursement of our products and the sales and marketing of our products and our service arrangements or data purchases, among other activities, may be subject to scrutiny under these laws. Additionally, the civil monetary penalties statute, among other things, imposes fines against any person who is determined to have presented or caused to be presented claims to a federal healthcare program that the person knows or should know is for an item or service that was not provided as claimed or is false or fraudulent.

Also, the federal Health Insurance Portability and Accountability Act, or HIPAA, created several new federal crimes, including healthcare fraud and false statements relating to healthcare matters. The healthcare fraud statute prohibits knowingly and willfully executing a scheme to defraud any healthcare benefit program, including private third-party payors. 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 healthcare benefits, items or services.

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our future business activities could be subject to challenge under one or more of such laws. In addition, recent health care reform legislation has further strengthened these laws. For

example, the Patient Protection and Affordable Care Act, or PPACA, among other things, amended the intent standard under the federal Anti-Kickback Statute and criminal healthcare fraud statutes to a stricter standard such that a person or entity no longer needs to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. The PPACA also provided 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 federal civil False Claims Act.

In addition, we may be subject to, or our marketing activities may be limited by, data privacy and security regulation by both the federal government and the states in which we conduct our business. HIPAA and its implementing regulations established uniform standards for certain "covered entities," which are healthcare providers, health plans and healthcare clearinghouses, as well as their business associates, governing the conduct of specified electronic healthcare transactions and protecting the security and privacy of protected health information. The American Recovery and Reinvestment Act of 2009, commonly referred to as the economic stimulus package, included an expansion of HIPAA's privacy and security standards called the Health Information Technology for Economic and Clinical Health Act, or HITECH. Among other things, HITECH makes HIPAA's security standards and certain privacy standards directly applicable to business associates. HITECH also created four new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties directly applicable to business associates and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorneys' fees and costs associated with pursuing federal civil actions.

Additionally, the federal Physician Payment Sunshine Act created under Section 6002 of the PPACA and its implementing regulations requires that manufacturers of drugs for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) report annually to the CMS information related to payments or other "transfers of value" made or distributed to physicians (defined to include doctors of medicine, dentists, optometrists, podiatrists and chiropractors), generally, with some exceptions, and teaching hospitals, or to entities or individuals at the request of, or designated on behalf of, the physicians and teaching hospitals. Additionally, applicable manufacturers and applicable group purchasing organizations are required to report annually to the CMS certain ownership and investment interests held by physicians (as defined above) and their immediate family members, and payments or other "transfers of value" to such physician owners and their immediate family members. Manufacturers were required to begin data collection beginning August 1, 2013, and submit reports on payment data to the government for the first reporting period (from August 1, 2013 to December 31, 2013) by June 30, 2014. Thereafter, covered manufacturers must submit reports by the 90th day of each subsequent calendar year. Disclosure of such information was made on a publicly available website beginning in September 2014.

There are also an increasing number of analogous state laws that require manufacturers to file reports with states on pricing and marketing information, such as tracking and reporting of gifts, compensation and other remuneration and items of value provided to health care professionals and health care entities. Many of these laws contain ambiguities as to what is required to comply with the laws. For example, several states have enacted legislation requiring pharmaceutical companies to, among other things, establish and implement commercial compliance programs, file periodic reports with the state, make periodic public disclosures on sales, marketing, pricing, clinical trials and other activities and/or register their sales representatives. Certain state laws also regulate manufacturers' use of prescriber-identifiable data. These laws may affect our future sales, marketing and other promotional activities by imposing administrative and compliance burdens. In addition, given the lack of clarity with respect to these laws and their implementation, our reporting actions could be subject to the penalty provisions of the pertinent state and federal authorities.

If our operations are found to be in violation of any of the health regulatory laws described above or any other laws that apply to us, we may be subject to penalties, including criminal and significant civil monetary penalties,

damages, fines, imprisonment, exclusion from participation in government healthcare programs, injunctions, recall or seizure of products, total or partial suspension of production, denial or withdrawal of pre-marketing product approvals and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations. To the extent that any of our products are sold in a foreign country, we may be subject to similar foreign laws and regulations, which may include, for instance, the U.S. Foreign Corrupt Practices Act, the U.K. Anti-Bribery Act, applicable post-marketing requirements, including safety surveillance, anti-fraud and abuse laws and implementation of corporate compliance programs and reporting of payments or transfers of value to healthcare professionals. We are unable to predict whether we would be subject to actions under these laws or the impact of such actions. However, the cost of defending such claims, as well as any sanctions imposed, could adversely affect our financial performance.

In the United States, most individual states have pharmaceutical distribution laws that require application and registration with State boards of pharmacy. States may also conduct cGMP inspections of pharmaceutical manufacturing facilities operating within their state.

Third-party payor coverage and reimbursement

The commercial success of our products, if and when approved, will depend, in part, upon the availability of coverage and adequate reimbursement from third-party payors at the federal, state and private levels. Patients who are prescribed treatments for their conditions and providers performing the prescribed services generally rely on third-party payors to reimburse all or part of the associated healthcare costs. Sales of our products will therefore depend substantially, both domestically and abroad, on the extent to which the costs of our products will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or are reimbursed by government payors, such as Medicare and Medicaid, private health coverage insurers and other third-party payors. The market for our products will depend significantly on access to third-party payors' formularies, or lists of treatments for which third-party payors provide coverage and reimbursement.

In addition, third-party payors are developing increasingly sophisticated methods of controlling healthcare costs and coverage and reimbursement for therapeutic products can differ significantly from payor to payor. As a result, the coverage determination process will require us to provide scientific and clinical support for the use of our products to each payor separately, with no assurance that adequate coverage and reimbursement will be obtained. The cost of pharmaceuticals and medical devices continues to generate substantial scrutiny from government and other third-party payors. We expect that the pharmaceutical industry will experience continued pricing pressures due to the trend toward managed healthcare, the increasing influence of managed care organizations and additional legislative proposals. Our results of operations and business could be adversely affected by current and future third-party payor policies as well as healthcare legislative reforms.

Some third-party payors also require pre-approval of coverage for new or innovative devices or drug therapies before they will reimburse healthcare providers who use such therapies. While we cannot predict whether any proposed cost-containment measures will be adopted or otherwise implemented in the future, these requirements or any announcement or adoption of such proposals could have a material adverse effect on our ability to obtain adequate prices for our product candidates and to operate profitably.

In international markets, reimbursement and healthcare payment systems vary significantly by country and many countries have instituted price ceilings on specific products and therapies. There can be no assurance that our products will be considered medically reasonable and necessary for a specific indication, that our products will be considered cost-effective by third-party payors, that an adequate level of reimbursement will be available or that the third-party payors' reimbursement policies will not adversely affect our ability to sell our product candidates profitably.

Healthcare reform

In the United States and foreign jurisdictions, the legislative landscape continues to evolve. There have been a number of legislative and regulatory changes to the healthcare system that could affect our future results of operations. In particular, there have been and continue to be a number of initiatives at the United States federal and state levels that seek to reduce healthcare costs. In March 2010, the PPACA was enacted, which includes measures that have the potential to significantly change health care financing by both governmental and private insurers. The provisions of the PPACA of importance to the pharmaceutical and biotechnology industry are, among others, the following:

- an annual, nondeductible fee on any entity that manufactures or imports certain branded prescription drugs and biologic agents, apportioned among these entities according to their market share in certain government healthcare programs;
- an increase in the rebates a manufacturer must pay under the Medicaid Drug Rebate Program to 23.1% and 13% of the average manufacturer price for branded and generic drugs, respectively;
- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts to negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D;
- extension of manufacturers' Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations, unless the drug is subject to discounts under the 340B drug discount program;
- expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to additional individuals and by adding new mandatory eligibility categories for certain individuals with income at or below 133% of the Federal Poverty Level, thereby potentially increasing manufacturers' Medicaid rebate liability;
- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;
- expansion of healthcare fraud and abuse laws, including the False Claims Act and the Anti-Kickback Statute, new government investigative powers and enhanced penalties for noncompliance;
- a licensure framework for follow-on biologic products;
- new requirements under the federal Physician Payment Sunshine Act for drug manufacturers to report information related to payments and other transfers of value made to physicians and teaching hospitals as well as ownership or investment interests held by physicians and their immediate family members; and
- a new requirement to annually report certain drug samples that manufacturers and distributors provide to licensed practitioners, or to pharmacies of hospitals or other healthcare entities.

In addition, other legislative changes have been proposed and adopted since the PPACA was enacted. In August 2011, the President signed into law the Budget Control Act of 2011, which, among other things, created the Joint Select Committee on Deficit Reduction to recommend proposals in spending reductions to Congress. The Joint Select Committee on Deficit Reduction did not achieve its targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, triggering the legislation's automatic reductions to several government programs. These reductions include aggregate reductions to Medicare payments to providers of 2% per fiscal year, which went into effect on April 1, 2013 and will stay in effect through 2024 unless additional Congressional action is

taken. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, further reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on our customers and accordingly, our financial operations. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our products or additional pricing pressures.

Europe / rest of world government regulation

In addition to regulations in the United States, we will be subject to a variety of regulations in other jurisdictions governing, among other things, clinical trials and any commercial sales, marketing and distribution of our products. Whether or not we 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. In addition, we and our collaborators may be subject to foreign laws and regulations and other compliance requirements, including, without limitation, anti-kickback laws, false claims laws, and other fraud and abuse laws, as well as laws and regulations requiring transparency of pricing and marketing information and governing the privacy and security of health information, such as the European Union's Directive 95/46 on the Protection of Individuals with regard to the Processing of Personal Data. 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.

Other regulatory requirements

We are also subject to various laws and regulations regarding laboratory practices, the experimental use of animals and the use and disposal of hazardous or potentially hazardous substances in connection with our research. In each of these areas, as above, the FDA and other government agencies have broad regulatory and enforcement powers, including, among other things, the ability to levy fines and civil penalties, suspend or delay issuance of approvals, seize or recall products, and withdraw approvals, any one or more of which could have a material adverse effect on us.

Material commercial agreements

Our collaboration with concert

In September 2011, we entered into a patent assignment agreement with Concert Pharmaceuticals, Inc., or Concert, pursuant to which Concert assigned to us a U.S. patent application relating to deuterated pirfenidone. Under the terms of the agreement, Concert receives certain royalty payments, or Royalty Payments, equal to a percentage in the low single digits of net sales in the United States invoiced by us or any of our affiliates with respect to certain pharmaceutical products containing deuterated pirfenidone. If we sell to another party all of our U.S. rights to certain deuterated pirfenidone products or if we grant to another party a license to sell certain deuterated pirfenidone products in the United States, Concert will receive an amount, or Sublicense/ Sale Payments, equal to a percentage in the teens of any proceeds we receive therefrom that are attributable to the rights to such deuterated pirfenidone products in the United States. If we are acquired in a change in control transaction at any time that we or any of our affiliates own certain patents or patent applications related to deuterated pirfenidone, Concert will receive 1.44% of any proceeds we receive in such transaction.

Such payment is applied as a credit to any future Royalty Payments and Sublicense/Sale Payments that may be due to Concert under the agreement. The agreement expires upon the earlier to occur of (1) receipt by Concert of the final Sublicense/Sale Payment arising from (a) the sale of our U.S. rights to certain deuterated pirfenidone products or (b) our grant of an exclusive license to sell certain deuterated pirfenidone products in the United States in all indications and fields, or (2) the expiration of the last claim owned by us or any of our affiliates in certain patents or patent applications related to deuterated pirfenidone.

Employees

As of December 31, 2014, we had 35 full-time employees, 11 of whom hold M.D., Ph.D. or Pharm.D. degrees, 23 of whom were engaged in research and development activities and 12 of whom were engaged in business development, marketing, finance, information systems, facilities, human resources or administrative support. None of our employees are represented by any collective bargaining unit. We believe that we maintain good relations with our employees. We expect to have two full-time employees in Germany as part of our acquisition of Imphar AG post-closing.

Facilities

Our principal facilities are located in San Diego, California. We currently lease approximately 18,500 square feet of office space under a lease that expires in March 2020. We believe that our existing facilities are adequate to meet our current needs, and that suitable additional alternative spaces will be available in the future on commercially reasonable terms. We expect to have a small office in Germany post-closing of the Imphar AG transaction.

Legal proceedings

We are currently not a party to any material legal proceedings.

Management

The following table sets forth information about our executive officers and directors as of November 30, 2014.

Name	Age	Position(s)
Pratik Shah, Ph.D.	45	President and Chief Executive Officer, Director
John Schmid	51	Chief Financial Officer
Bharatt Chowrira, Ph.D., J.D.	49	Chief Operating Officer
Samuel Saks, M.D.	60	Chief Development Officer, Director
David Stamler, M.D.	54	Chief Medical Officer
Non-Employee Directors		
Dorsey Bleil(3)	50	Director
Rod Ferguson, Ph.D.(2)(3)	58	Director
R. Scott Greer(1)(2)	55	Director
Gerald Proehl(2)(3)	55	Director
Sepehr Sarshar, Ph.D.(1)	47	Director
Phillip M. Schneider(1)	58	Director
Alex Zisson(1)(2)	45	Director

(1) Member of the audit committee.

(2) Member of the compensation committee.

(3) Member of the nominating and corporate governance committee.

Executive officers

Pratik Shah, Ph.D. has served as our President and Chief Executive Officer since October 2013 and as a member of our board of directors since June 2007. Dr. Shah served as Chairman of our board of directors from September 2008 to October 2013. Dr. Shah has been a partner in the venture capital firm Thomas, McNerney & Partners since 2004. Prior to joining Thomas, McNerney & Partners, he co-founded Kalypsys, Inc., a private clinical-stage pharmaceutical company, where he served as the Chief Business Officer from 2001 to 2004 and was responsible for the overall strategy, business development and operations of the company. Before co-founding Kalypsys, Inc., Dr. Shah was at McKinsey & Company, a consulting firm, from 1999 to 2001 where he focused on biotechnology and venture capital projects. Prior to McKinsey & Company, Dr. Shah co-founded NephRx Corporation, a start-up company focused on the discovery of therapeutic proteins for renal disease, where he served as the Vice President of Operations from 1997 to 1999. In addition to serving on our board of directors, Dr. Shah currently serves on the following private company boards of directors: Cebix Incorporated and SGB, Inc. He holds a B.S. in Biological Sciences from the University of California, Irvine and both a Ph.D. in Biochemistry & Molecular Biology and an M.B.A. in Finance from the University of Chicago. We believe that Dr. Shah's experience in managing, financing and consulting with biotechnology companies and having played a key role in founding and building several life sciences companies qualifies him to serve on our board of directors.

John Schmid joined us as Chief Financial Officer in September 2013. Before joining us, Mr. Schmid co-founded Trius Therapeutics, Inc., a publicly-traded biopharmaceutical company, where he served as the Chief Financial Officer from June 2004 until its merger with Cubist Pharmaceuticals, Inc. in September 2013. Prior to Trius Therapeutics, Inc., Mr. Schmid served as the Chief Financial Officer at GeneFormatics, Inc., a private

biotechnology company, from 1998 to 2003 and Endonetics, Inc., a private medical device company, from 1995 to 1998. Mr. Schmid also currently serves as the chairman of the board of directors of Speak, Inc., a speakers bureau, which he helped found in 1989. Mr. Schmid holds a B.A. in Economics from Wesleyan University and an M.B.A. from the University of San Diego.

Bharatt Chowrira, Ph.D. joined us as Chief Operating Officer in October 2013. Prior to joining us, Dr. Chowrira served as President and Chief Executive Officer of Addex Therapeutics, Inc., a biotechnology company publicly-traded on the SIX Swiss Exchange, from August 2011 to July 2013. Prior to Addex Therapeutics, Inc., he served as Senior Vice President and Chief Operating Officer at Nektar Therapeutics, or Nektar, a publicly-traded clinical-stage biopharmaceutical company, from May 2008 to January 2011, during which time he also served as the chairman of the board of directors of Nektar India PTY Ltd., a wholly owned subsidiary of Nektar. Prior to Nektar, Dr. Chowrira served as the Vice President, Legal and Chief Patent Counsel at Sirna Therapeutics, Inc., a publicly-traded biotechnology company, from June 2003 until its acquisition by Merck & Co., Inc., or Merck, a publicly-traded global health care company, in December 2006, after which he stayed at Merck until May 2008 as Vice President, Sirna and Executive Director, Worldwide Licensing. From 1993 to 2003, Dr. Chowrira held various leadership positions at Sirna Therapeutics, Inc.'s predecessor, Ribozyme Pharmaceuticals, Inc., a publicly-traded biotechnology company. Dr. Chowrira received a J.D. from the University of Denver—Sturm College of Law, a Ph.D. in Molecular Biology from the University of Vermont College of Medicine, an M.S. in Molecular Biology from Illinois State University and a B.S. in Microbiology from the University of Agricultural Sciences, Bangalore, India.

Samuel Saks, M.D. joined us as our Chief Development Officer in November 2013 and has served as a member of our board of directors since September 2009. Dr. Saks was a co-founder of Jazz Pharmaceuticals plc, a publicly-traded biopharmaceutical company, where he served as Chief Executive Officer from 2003 until his retirement in 2009. From 2001 to 2003, Dr. Saks served as Company Group Chairman of ALZA, a then publicly-traded pharmaceutical and medical systems company and served as a member of the Johnson & Johnson Pharmaceutical Group Operating Committee. From 1992 to 2001, Dr. Saks held various positions with ALZA, most recently as its Group Vice President. Prior to ALZA, Dr. Saks held clinical research and development management positions with Schering-Plough Corporation, a publicly-traded pharmaceutical company that merged with Merck in 2009, Xoma Corp., a publicly-traded biotechnology company, and Genentech, Inc., a then publicly-traded biotechnology company. Dr. Saks currently serves on the boards of directors of TONIX Pharmaceuticals Holding Corp. and Depomed, Inc., both of which are publicly-traded pharmaceutical companies, as well as Bullet Biotechnology, Inc., a private biotechnology company, NuMedii, Inc., a private biotechnology company, and Velocity Pharmaceutical Development, LLC, a private drug development firm. Dr. Saks holds a B.S. in Biology and an M.D. from the University of Illinois. He completed his residency in Internal Medicine at Texas Southwestern and his fellowship in Oncology at the University of California, San Francisco. We believe that Dr. Saks' more than 35 years of experience in managing biotechnology companies qualifies him to serve on our board of directors.

David Stamler, M.D. has served as our Chief Medical Officer since January 2011. Prior to joining us, Dr. Stamler served as Senior Vice President and Chief Medical Officer at XenoPort, Inc., a publicly-traded biopharmaceutical company, from 2008 to 2010 and Chief Scientific Officer and Head of Drug Development at Prestwick Pharmaceuticals, Inc., a private pharmaceutical company, from 2005 to 2008. Prior to Prestwick Pharmaceuticals, Inc., Dr. Stamler worked at Fujisawa Pharmaceutical Co. and its subsidiaries from 1997 to 2005, in various leadership roles, including Vice President, Research and Development, Medical Sciences at Fujisawa Healthcare, Inc. from 2003 to 2005 and as Vice President, Clinical Research Center at Fujisawa Research Institute of America from 2000 to 2003. Dr. Stamler began his career at Abbott Laboratories, a publicly-traded global pharmaceuticals and healthcare products company, where he served in various positions from 1993 to 1997, including Director of Clinical Research, Pharmaceutical Products for the International

Division. Dr. Stamler received an M.D. from the University of Chicago—The Pritzker School of Medicine and a B.A. in Biology from the University of Chicago.

Non-employee directors

Lynn Dorsey Bleil has served as a member of our board of directors since May 2014. Ms. Bleil was most recently a Senior Partner (Director) at McKinsey & Company where she worked for over 28 years until her retirement in December 2013. While at McKinsey, Ms. Bleil led the west coast healthcare practice and was a core leader and operating committee member of the global healthcare practice. Ms. Bleil currently serves as a member of the board of directors of DST Systems Inc., a publicly-traded software development firm, and is a Governing Board Director, Park City Medical Center of Intermountain Health Care, Inc., a private, non-profit health services corporation. Ms. Bleil holds a B.S. in chemical engineering from Princeton University and an M.B.A. from the Stanford Graduate School of Business, where she was an Arjay Miller Scholar. We believe that Ms. Bleil's experience advising biotechnology and pharmaceutical companies qualifies her to serve on our board of directors.

Rod Ferguson, Ph.D. has served as a member of our board of directors since January 2013. Dr. Ferguson is currently a Managing Director at Panorama Capital, a technology and life sciences investment firm, which he co-founded in 2006. Prior to co-founding Panorama Capital, Dr. Ferguson served as a Managing Director at JPMorgan Partners, a private equity firm, from 2001 to 2006. From 1999 to 2001, he was a Partner at InterWest Partners, a venture capital firm. Prior to InterWest Partners, he held a variety of positions at Genentech, Inc., a then publicly-traded biotechnology company, from 1988 to 1999, including Senior Director of Business and Corporate Development. Prior to Genentech, Inc., Dr. Ferguson was an Associate at the law firm of McCutchen, Doyle, Brown & Enersen LLP. Dr. Ferguson received a B.S. with honors in Biochemistry from the University of Illinois, a Ph.D. in Biochemistry from the State University of New York at Buffalo and a J.D., cum laude, from Northwestern University. We believe that Dr. Ferguson's experience in investment banking and in financing pharmaceutical companies qualifies him to serve on our board of directors.

R. Scott Greer has served as a member of our board of directors since May 2014. Since June 2003, Mr. Greer has served as Managing Director of Numenor Ventures, LLC, a venture capital firm. In 1996, Mr. Greer co-founded Abgenix, Inc., a company that specialized in the discovery, development and manufacture of human therapeutic antibodies, and from June 1996 through May 2002, he served as its Chief Executive Officer. He also served as a director of Abgenix from 1996 and chairman of the board of directors from 2000 until the acquisition of Abgenix by Amgen, Inc. in April 2006. Prior to Abgenix's formation, Mr. Greer held senior management positions at Cell Genesys, Inc., a biotechnology company, initially as Chief Financial Officer and Vice President of Corporate Development and later as Senior Vice President of Corporate Development, and various positions at Genetics Institute, Inc., a biotechnology research and development company. Mr. Greer currently serves as a member of the boards of directors of StemCells, Inc., a publicly-traded biopharmaceutical company focused on stem cell therapeutics, Nektar Therapeutics, a publicly-traded clinical-stage biopharmaceutical company, Sientra, Inc., a publicly-traded commercial stage medical device company, and Versartis, Inc., a publicly-traded development stage biotechnology company. Mr. Greer holds a B.A. in economics from Whitman College and an M.B.A. from Harvard University. He also was a certified public accountant. We believe that Mr. Greer's experience as an accountant and as an executive of various public and private biotechnology and biopharmaceutical companies qualifies him to serve on our board of directors.

Gerald T. Proehl has served as a member of our board of directors since January 2014 and as the lead independent director of our board of directors since December 2014. Mr. Proehl was the President and Chief Executive Officer of Santarus, Inc., a pharmaceutical company, from January 2002 until its sale to Salix Pharmaceuticals, Inc., a pharmaceutical company, in January 2014. Prior to becoming Santarus' President and

CEO, Mr. Proehl served as Santarus' President and Chief Operating Officer from March 2000 through December 2001 and Vice President, Marketing and Business Development from April 1999 to March 2000. Prior to joining Santarus, Mr. Proehl spent 14 years at Hoechst Marion Roussel, Inc., a global pharmaceutical company, where he served in various capacities in multiple therapeutic areas including gastrointestinal, cardiovascular, wound care and central nervous system. Mr. Proehl currently serves as a member of the boards of directors of Sophiris Bio Inc., a publicly-traded clinical-stage biopharmaceutical company, Tenax Therapeutics, Inc., a publicly-traded pharmaceutical company, Patara Pharma, LLC, a private biotechnology company, and Kinetek Sports, Inc., a private technology company. Mr. Proehl holds a B.S. in education from the State University of New York at Cortland, an M.A. in exercise physiology from Wake Forest University and an M.B.A. from Rockhurst College. We believe that Mr. Proehl's management, operational and marketing experience in the pharmaceutical sector qualifies him to serve on our board of directors.

Sepehr Sarshar, Ph.D. has served as a member of our board of directors since September 2009 and is one of our founders. Dr. Sarshar is a Partner at Sloan Biotech Fund, a venture capital firm, where he has been a Member since 2001. Since January 2011, Dr. Sarshar has been the Manager at Costa Verde Capital, LLC and Costa Verde Capital II, LLC, both venture capital firms. Prior to our founding, Dr. Sarshar worked for Pfizer Inc., a publicly-traded multinational pharmaceutical company, from 1997 to 2000 and for Ontogen Corporation, a private biotechnology company, from 1994 to 1997. Dr. Sarshar received a B.S. in Chemistry and Mathematics from the University of California, Los Angeles and a Ph.D. in Organic Chemistry from Harvard University. We believe that Dr. Sarshar's experience managing pharmaceutical companies and as one of our founders qualifies him to serve on our board of directors.

Phillip M. Schneider has served as a member of our board of directors since January 2014. Mr. Schneider held various positions with IDEC Pharmaceuticals Corporation (now Biogen IDEC Inc.), a publicly-traded global biotechnology company, from 1987 to 2003, including most recently as Senior Vice President and Chief Financial Officer. Prior to IDEC, Mr. Schneider held various management positions from 1984 to 1987 at Syntex Corporation, a publicly-traded global pharmaceuticals company that was acquired by Roche Holdings Ltd., including Manager of Financial Reporting and Senior Analyst. Mr. Schneider worked for KPMG LLP, an audit, tax and advisory services firm, from 1982 to 1984 as a Senior Accountant. Mr. Schneider currently serves as a member of the boards of directors of Arena Pharmaceuticals, Inc., a publicly-traded biopharmaceutical company, which board he joined in 2007, and Pfénex Incorporated, a publicly-traded biopharmaceutical company, which board he joined in 2014. Mr. Schneider served as a member of the board of directors of Gen-Probe Incorporated, a publicly-traded medical diagnostics company that was acquired by Hologic Corporation, from November 2002 to August 2012, and served on the board of directors of CancerVax/Micromet Inc., a publicly-traded biopharmaceutical company that was acquired by Amgen Inc., from September 2003 to March 2008. Mr. Schneider received a B.S. in Biochemistry from the University of California at Davis and an M.B.A. from the University of Southern California. We believe that Mr. Schneider's experience in accounting and finance qualifies him to serve on our board of directors.

Alex Zisson has served as a member of our board of directors since October 2013. Mr. Zisson is currently a Partner in the venture capital firm Thomas, McNerney & Partners and is also currently a board member of Clarus Therapeutics, Inc., a private pharmaceutical company, and Keystone Dental, Inc., a private medical device company. Prior to Thomas, McNerney & Partners, Mr. Zisson spent 11 years in the research department at Hambrecht & Quist, an investment bank (and its successor firms Chase H&Q and JPMorgan H&Q), from 1991 to 2002, including serving as Managing Director from 1997 to 2002 and as the firm's Health Care Strategist following the merger of Chase H&Q and JPMorgan. Mr. Zisson is a Member of the Industry Advisory Board of the Children's Tumor Foundation, a private foundation, and a member of the board of directors of Advanced Medical Research Institute of Canada Development Corporation, a private company focused on economic development in northern Ontario. Mr. Zisson received a B.A. in History from Brown University, where he was

elected to Phi Beta Kappa and graduated magna cum laude. We believe that Mr. Zisson's experience in investment banking and in financing pharmaceutical companies qualifies him to serve on our board of directors.

Board composition

Our business and affairs are organized under the direction of our board of directors, which currently consists of seven members. The primary responsibilities of our board of directors are to provide oversight, strategic guidance, counseling and direction to our management. Our board of directors meets on a regular basis and on an ad hoc basis as required.

Our board of directors has determined that all of our directors other than Drs. Shah and Saks are independent directors, as defined by Rule 5605(a)(2) of the NASDAQ Listing Rules.

In accordance with the terms of our amended and restated certificate of incorporation and amended and restated bylaws, our board of directors is elected annually to a one year term.

The authorized size of our board of directors is currently nine members. The authorized number of directors may be changed only by resolution of our board of directors. Our directors may be removed for cause by the affirmative vote of the holders of at least 66⅔% of our voting stock.

Board leadership structure

The board of directors does not currently have a chairman of the board. We have a separate chair for each committee of our board of directors. The chairs of the committees are expected to report annually to our board of directors on the activities of their committee in fulfilling their responsibilities as detailed in their respective charters or specify any shortcomings should that be the case.

The board of directors appointed Mr. Proehl as the lead independent director in December 2014 to help reinforce the independence of the board of directors as a whole. The position of lead independent director has been structured to serve as an effective balance in the absence of a Chairman of the board. The lead independent director is empowered to, among other duties and responsibilities, approve agendas and meeting schedules for regular meetings of the board of directors, preside over meetings of the board of directors in the absence of a chairman of the board, preside over and establish the agendas for meetings of the independent directors, act as liaison between the Chief Executive Officer and the directors, approve information sent to the board of directors, and act as a liaison to stockholders. In addition, it is the responsibility of the lead independent director to coordinate between the board of directors and management with regard to the determination and implementation of responses to any problematic risk management issues. As a result, we believe that the lead independent director can help ensure the effective independent functioning of the board of directors in its oversight responsibilities. In addition, we believe that the lead independent director is better positioned to build a consensus among directors and to serve as a conduit between the other independent directors and the Chief Executive Officer in his capacity as a member of the board of directors, for example, by facilitating the inclusion on meeting agendas of matters of concern to the independent directors.

Role of the board in risk oversight

The audit committee of our board of directors is primarily responsible for overseeing our risk management processes on behalf of our board of directors. The audit committee receives reports from management at least quarterly regarding our assessment of risks. In addition, the audit committee reports regularly to our board of directors, which also considers our risk profile. The audit committee and our board of directors focus on the

most significant risks we face and our general risk management strategies. While our board of directors oversees our risk management, management is responsible for day-to-day risk management processes. Our board of directors expects management to consider risk and risk management in each business decision, to proactively develop and monitor risk management strategies and processes for day-to-day activities and to effectively implement risk management strategies adopted by the audit committee and our board of directors. We believe this division of responsibilities is the most effective approach for addressing the risks we face and that our board of directors leadership structure, which also emphasizes the independence of our board of directors in its oversight of its business and affairs, supports this approach. Our board of directors has delegated to the board of directors' lead independent director the responsibility of coordinating between the board of directors and management with regard to the determination and implementation of responses to any problematic risk management issues.

Board committees

Our board of directors has established an audit committee, a compensation committee and a nominating and corporate governance committee.

Audit committee

Our audit committee consists of R. Scott Greer, Sepehr Sarshar, Phillip Schneider and Alex Zisson. Our board of directors has determined that each of the members of our audit committee satisfies the NASDAQ Stock Market and SEC independence requirements. Mr. Schneider serves as the chair of our audit committee. The functions of this committee include, among other things:

- evaluating the performance, independence and qualifications of our independent auditors and determining whether to retain our existing independent auditors or engage new independent auditors;
- reviewing and approving the engagement of our independent auditors to perform audit services and any permissible non-audit services;
- monitoring the rotation of partners of our independent auditors on our engagement team as required by law;
- prior to engagement of any independent auditor, and at least annually thereafter, reviewing relationships that may reasonably be thought to bear on their independence, and assessing and otherwise taking the appropriate action to oversee the independence of our independent auditor;
- reviewing our annual and quarterly financial statements and reports, including the disclosures contained under the caption "Management's Discussion and Analysis of Financial Condition and Results of Operations," and discussing the statements and reports with our independent auditors and management;
- reviewing with our independent auditors and management significant issues that arise regarding accounting principles and financial statement presentation and matters concerning the scope, adequacy and effectiveness of our financial controls;
- reviewing with management and our auditors any earnings announcements and other public announcements regarding material developments;
- establishing procedures for the receipt, retention and treatment of complaints received by us regarding financial controls, accounting or auditing matters and other matters;
- preparing the report that the SEC requires in our annual proxy statement;

- reviewing and providing oversight of any related-person transactions in accordance with our related person transaction policy and reviewing and monitoring compliance with legal and regulatory responsibilities, including our code of business conduct and ethics;
- reviewing our major financial risk exposures, including the guidelines and policies to govern the process by which risk assessment and risk management is implemented;
- reviewing on a periodic basis our investment policy; and
- reviewing and evaluating on an annual basis its own performance, including its compliance with its charter.

Our board of directors has determined that Mr. Schneider qualifies as an audit committee financial expert within the meaning of SEC regulations and meets the financial sophistication requirements of the NASDAQ Listing Rules. In making this determination, our board has considered Mr. Schneider's formal education and previous and current experience in financial roles. Both our independent registered public accounting firm and management periodically meet privately with our audit committee.

Compensation committee

Our compensation committee consists of Rod Ferguson, R. Scott Greer, Gerald Proehl and Alex Zisson. Mr. Zisson serves as the chair of our compensation committee. Our board of directors has determined that each of the members of our compensation committee is a non-employee director, as defined in Rule 16b-3 promulgated under the Exchange Act, is an outside director, as defined pursuant to Section 162(m) of the Code, and satisfies the NASDAQ Stock Market independence requirements. The functions of this committee include, among other things:

- reviewing, modifying and approving (or if it deems appropriate, making recommendations to the full board of directors regarding) our overall compensation strategy and policies;
- reviewing and approving the compensation and other terms of employment of our executive officers;
- reviewing and approving performance goals and objectives relevant to the compensation of our executive officers and assessing their performance against these goals and objectives;
- reviewing and approving (or if it deems it appropriate, making recommendations to the full board of directors regarding) the equity incentive plans, compensation plans and similar programs advisable for us, as well as modifying, amending or terminating existing plans and programs;
- evaluating risks associated with our compensation policies and practices and assessing whether risks arising from our compensation policies and practices for our employees are reasonably likely to have a material adverse effect on us;
- reviewing and approving (or if it deems it appropriate, making recommendations to the full board of directors regarding) the type and amount of compensation to be paid or awarded to our non-employee board members;
- establishing policies with respect to votes by our stockholders to approve executive compensation as required by Section 14A of the Exchange Act and determining our recommendations regarding the frequency of advisory votes on executive compensation, to the extent required by law;
- reviewing and assessing the independence of compensation consultants, legal counsel and other advisors as required by Section 10C of the Exchange Act;

- administering our equity incentive plans;
- establishing policies with respect to equity compensation arrangements;
- reviewing the competitiveness of our executive compensation programs and evaluating the effectiveness of our compensation policy and strategy in achieving expected benefits to us;
- reviewing, approving and, if appropriate, making recommendations to the full board of directors regarding the terms of any employment agreements, severance arrangements, change in control protections and any other compensatory arrangements for our executive officers;
- reviewing the adequacy of its charter on a periodic basis;
- reviewing with management and approving our disclosures, if any, under the caption "Compensation Discussion and Analysis" and related tables in our periodic reports or proxy statements to be filed with the SEC;
- preparing the report that the SEC requires in our annual proxy statement; and
- reviewing and assessing on an annual basis its own performance.

Nominating and corporate governance committee

Our nominating and corporate governance committee consists of Lynn Bleil, Gerald Proehl and Rod Ferguson. Our board of directors has determined that each of the members of this committee satisfies the NASDAQ Stock Market independence requirements. Mr. Proehl serves as the chair of our nominating and corporate governance committee. The functions of this committee include, among other things:

- identifying, reviewing and evaluating candidates to serve on our board of directors consistent with criteria approved by our board of directors;
- determining the minimum qualifications for service on our board of directors;
- evaluating director performance on the board and applicable committees of the board and determining whether continued service on our board is appropriate;
- evaluating, nominating and recommending individuals for membership on our board of directors;
- evaluating nominations by stockholders of candidates for election to our board of directors;
- considering and assessing the independence of members of our board of directors;
- developing a set of corporate governance policies and principles, including a code of business conduct and ethics, periodically reviewing and assessing these policies and principles and their application and recommending to our board of directors any changes to such policies and principles;
- considering questions of possible conflicts of interest of directors as such questions arise;
- reviewing the adequacy of its charter on an annual basis; and
- annually evaluating the performance of the nominating and corporate governance committee.

We believe that the composition and functioning of our nominating and corporate governance committee complies with all applicable requirements of the Sarbanes-Oxley Act of 2002, and all applicable SEC and NASDAQ rules and regulations. We intend to comply with future requirements to the extent they become applicable to us.

Compensation committee interlocks and insider participation

None of our current or former executive officers serves as a member of the compensation committee. None of our officers serves, or has served during the last completed fiscal year on the board of directors or compensation committee, or other committee serving an equivalent function, of any other entity that has one or more of its executive officers serving as a member of our board of directors or our compensation committee. Prior to establishing the compensation committee, our full board of directors made decisions relating to compensation of our officers.

Code of business conduct and ethics

We have adopted 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 person performing similar functions. A current copy of the code is available on the Corporate Governance section of our website, www.auspexpharma.com.

Limitation of liability and indemnification

Our amended and restated certificate of incorporation limits the liability of directors to the maximum extent permitted by Delaware law. Delaware law allows a corporation to eliminate the personal liability of directors of a corporation to the corporation and its stockholders for monetary damages for breach of their fiduciary duties as directors, except for liability for any:

- breach of his or her duty of loyalty to the corporation or its stockholders;
- 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
- transaction from which the director derived an improper personal benefit.

Our amended and restated certificate of incorporation does not eliminate a director's duty of care and, in appropriate circumstances, equitable remedies, such as injunctive or other forms of non-monetary relief, will remain available under Delaware law. These limitations also do not affect a director's responsibilities under any other laws, such as the federal securities laws or other state or federal laws. Our amended and restated bylaws provide that we will indemnify our directors and executive officers and may indemnify other officers, employees and other agents, to the fullest extent permitted by law. Our amended and restated bylaws also provide that we are obligated to advance expenses incurred by a director or officer in advance of the final disposition of any action or proceeding and also 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 connection with their services to us, regardless of whether our amended and restated bylaws permit such indemnification. We have obtained a policy of directors' and officers' liability insurance.

We have entered into separate indemnification agreements with our directors and executive officers, in addition to the indemnification provided for in our amended and restated bylaws. These agreements, among other things, require us to indemnify our directors and executive officers for certain expenses, including attorneys' fees, judgments, fines and settlement amounts incurred by a director or executive officer in any action or proceeding arising out of their services as one of our directors or executive officers or any other company or enterprise to which the person provides services at our request. We believe that these bylaw provisions and indemnification agreements are necessary to attract and retain qualified persons as directors and officers.

The limitation of liability and indemnification provisions in our amended and restated certificate of incorporation and amended and restated bylaws may discourage stockholders from bringing a lawsuit against directors for breach of their fiduciary duties. They may also reduce the likelihood of derivative litigation against directors and officers, even though an action, if successful, might benefit us and our stockholders. A stockholder's investment may be harmed to the extent we pay the costs of settlement and damage awards against directors and officers pursuant to these indemnification provisions.

We believe that these provisions in our amended and restated certificate of incorporation and amended and restated bylaws and these indemnification agreements are necessary to attract and retain qualified persons as directors and officers.

There is no pending litigation or proceeding involving any of our directors or executive officers as to which indemnification is required or permitted, and we are not aware of any threatened litigation or proceeding that may result in a claim for indemnification.

Executive and director compensation

Our named executive officers for the year ended December 31, 2014, which consist of our principal executive officer and our two other most highly compensated executive officers, are:

- Pratik Shah, Ph.D., our President and Chief Executive Officer;
- Bharatt Chowrira, Ph.D., J.D., our Chief Operating Officer; and
- John Schmid, our Chief Financial Officer.

Summary compensation table

The following table sets forth information regarding compensation awarded to or earned by our named executive officers during the year ended December 31, 2014 and, for Dr. Shah, December 31, 2013. As an emerging growth company, we comply with the executive compensation disclosure rules applicable to "smaller reporting companies," as such term is defined in the rules promulgated under the Securities Act, which require compensation disclosure for our principal executive officer and the two most highly compensated executive officers other than our principal executive officer.

Name and principal position	Year	Salary ($)	Bonus ($)(2)	Stock awards ($)(3)	Option awards ($)(3)	Non-equity incentive plan compensation(4)	All other compensation ($)(5)	Total ($)
Pratik Shah, Ph.D.	2014	$470,000	$ –	$ –	$1,043,944	$375,000	$1,467	$1,890,411
President and Chief Executive Officer(1)	2013	$102,500	$490,000	$483,130	$ –		$9,606	$1,085,236
John Schmid	2014	$319,233	$ 82,500	$ –	$ 286,400	$163,625	$1,584	$ 853,342
Chief Financial Officer								
Bharatt Chowrira, Ph.D., J.D.	2014	$360,000	$ 60,000	$ –	$ –	$187,688	$1,170	$ 608,858
Chief Operating Officer								

(1) Dr. Shah served as our President and Chief Executive Officer commencing in October 2013.

(2) This column reflects discretionary cash bonuses earned for 2014, as further described below under "—Annual Bonus Opportunities."

(3) In accordance with SEC rules, this column reflects the aggregate fair value of the stock awards and option awards granted during the respective fiscal year computed as of their respective grant dates in accordance with Financial Accounting Standard Board Accounting Standards Codification Topic 718 for stock-based compensation transactions (ASC 718). Assumptions used in the calculation of these amounts are included in the notes to our financial statements incorporated by reference into this prospectus. These amounts do not reflect the actual economic value that will be realized by the named executive officer upon the vesting of the stock awards or option awards, the exercise of the option awards, or the sale of the common stock underlying such stock awards and option awards.

(4) Amounts shown represent annual performance-based bonuses earned for 2014. For more information regarding these bonuses, see below under "—Annual Bonus Opportunities."

(5) This column reflects term life and disability insurance premiums paid by us on behalf of the named executive officers. All of these benefits are provided to the named executive officers on the same terms as provided to all of our regular full-time employees. For more information regarding these benefits, see below under "—Perquisites, Health, Welfare and Retirement Benefits."

Annual base salary

The base salaries of our named executive officers are generally determined and approved at the beginning of each year or, if later, in connection with the commencement of employment of the executive, by our board of directors or the compensation committee. The following represent the 2014 annual base salaries for our named executive officers as approved by our board of directors in May 2014. The 2014 base salary for each of Dr. Shah, Dr. Chowrira and Mr. Schmid became effective on May 1, 2014. The 2013 annual base salaries in effect during 2014 prior to the 2014 base salary increases were $410,000, $300,000 and $275,000 for Dr. Shah, Dr. Chowrira and Mr. Schmid, respectively.

Name	2014 Base salary ($)
Pratik Shah, Ph.D.	$500,000
Bharatt Chowrira, Ph.D., J.D.	$390,000
John Schmid	$340,000

Annual bonus opportunities

Discretionary bonuses

From time to time our board of directors or compensation committee may approve discretionary bonuses for our named executive officers when they determine appropriate, for example, in connection with such officer's commencement of employment with us. On March 27, 2014, our compensation committee approved a discretionary bonus of $60,000 and $82,500 for Dr. Chowrira and Mr. Schmid, respectively.

Annual performance-based bonuses

Our named executive officers also are eligible to receive annual performance-based cash bonuses, which are designed to provide appropriate incentives to our executives to achieve defined annual corporate goals and to reward our executives for individual achievement towards these goals. The annual performance-based bonus each named executive officer is eligible to receive is generally based on the extent to which we achieve our corporate goals and the extent to which the executive achieves his individual goals, if any, that our board of directors establishes each year. At the end of the year, our board of directors or compensation committee reviews our performance against each corporate goal and approves the extent to which we achieved each of our corporate goals.

A target bonus, expressed as a percentage of base salary, is generally provided to each named executive officer prior to or shortly following the beginning of the year. However, there is generally no minimum or maximum bonus percentage or amount established for a named executive officer and, as a result, the bonus amount for each officer may vary from year to year based on corporate and individual performance and such officer's target bonus. However, pursuant to Dr. Shah's offer letter agreement, his 2014 bonus will be a minimum of $90,000. The corporate and individual goals are determined by our board of directors after recommendation by our compensation committee and are generally communicated to the named executive officers each year, prior to or shortly following the beginning of the year to which they relate or if later, in connection with the commencement of employment of the named executive officer. The corporate goals are comprised of a subset of our most important annual corporate goals and various business accomplishments, which vary from time to time depending on our overall strategic objectives. The individual goals are composed of factors that relate to each named executive officer's ability to guide his or her own performance, and the performance of his or her direct employee reports towards reaching our corporate goals.

For 2014, the target bonus for Dr. Shah was 50% of his annual base salary and the target bonus for each of Dr. Chowrira and Mr. Schmid was 35% of his annual base salary. For 2014, Dr. Shah's bonus was entirely

dependent upon our achievement of corporate goals and each of Dr. Chowrira's and Mr. Schmid's bonus was weighted 75% based on our achievement of corporate goals and 25% based on each of Dr. Chowrira's and Mr. Schmid's achievement of their respective individual goals.

Our board of directors established corporate goals for 2014 that were weighted (1) 80% for clinical activities relating to SD-809 and completion of chemistry, manufacturing and controls, or CMC, and activities in support of manufacturing and (2) 20% for commercial activities relating to upcoming clinical studies, manufacturing activities in support of a 505(b)(2) New Drug Application and hiring a new head of regulatory.

In December 2014, our compensation committee reviewed the achievement of the corporate and individual goals for 2014 and, upon recommendation by the compensation committee, our board of directors determined in January 2015 that on an overall basis, the goals had been achieved and in some cases, we had exceeded our goals, resulting in an overall achievement of 150% of our corporate goals. Our compensation committee determined that we had substantially achieved our clinical and other goals, and noted that we had overachieved our clinical/CMC goals due to the positive clinical data for SD-809 in our First-HD and ARC-HD clinical trials as well as the management team's strong contributions during the year. Accordingly, upon recommendation by the compensation committee, our board of directors awarded Dr. Shah a bonus of $375,000, based solely on corporate goal achievement, and awarded Dr. Chowrira and Mr. Schmid a bonus of $187,688 and $163,625, respectively, based on corporate goal achievement and their respective achievement of individual goals.

Equity-based incentive awards

Our equity-based incentive awards are designed to align our interests and the interests of our stockholders with those of our employees and consultants, including our named executive officers. The board of directors is responsible for approving equity grants.

We have historically used stock options as the primary incentive for long-term compensation to our named executive officers because they are able to profit from stock options only if our stock price increases relative to the stock option's exercise price, which exercise price is set at the fair market value of our common stock at the date of grant. We have also granted restricted stock awards from time to time. We may grant equity awards at such times as our board of directors determines appropriate. Our executives generally are awarded an initial grant in the form of a stock option or restricted stock award in connection with their commencement of employment. Additional grants may occur periodically in order to specifically incentivize executives with respect to achieving certain corporate goals or to reward executives for exceptional performance.

Prior to our initial public offering in February 2014, we granted all stock options and restricted stock awards pursuant to our 2010 Equity Incentive Plan, or the 2010 Plan, and prior to the 2010 Plan, our discontinued 2001 Stock Option Plan, or the 2001 Plan. In connection with our initial public offering, we adopted the 2014 Equity Incentive Plan, or the 2014 Plan, as the continuation of and successor to our 2010 Plan. We now grant all stock awards under the 2014 Plan. The terms of our equity plans are described below under "—Equity Benefit Plans."

All stock options are granted with an exercise price per share that is no less than the fair market value of our common stock on the date of grant of each award. Our stock option awards and restricted stock awards generally vest over a four-year period and may be subject to acceleration of vesting and exercisability under certain termination and change of control events.

On January 10, 2014, we granted option awards to purchase 215,153 and 59,026 shares of our common stock to Dr. Shah and Mr. Schmid, respectively. These option awards were granted with an exercise price of $6.57 per share and vest over a four-year period, subject to Dr. Shah's and Mr. Schmid's continued service with us, as applicable. No awards were granted to Dr. Chowrira during 2014.

Agreements with our named executive officers

Below are written descriptions of our offer letter agreements with each of our named executive officers. Each of our named executive officers' employment is "at will" and may be terminated at any time.

Dr. Shah. We entered into an offer letter agreement with Dr. Shah in October 2013 setting forth the terms of his employment as our President and Chief Executive Officer. Pursuant to the agreement, Dr. Shah is entitled to an initial annual base salary of $410,000 and an annual bonus of up to 45% of his annual base salary based on achievement of corporate goals determined by the board of directors and payable in a mix of cash and equity, as determined by the board of directors. As of May 1, 2014, the board of directors increased Dr. Shah's annual base salary to $500,000 and his bonus for 2014 was set at a target of 50% of his annual base salary, as further described above under "—Annual Bonus Opportunities." Dr. Shah's agreement provides for a guaranteed $90,000 annual cash bonus for each of fiscal years 2013 and 2014, provided that he is serving as our Chief Executive Officer at the time of payment of each such bonus. In addition, Dr. Shah was paid a signing bonus of $150,000 in November 2013 and a retention bonus of $250,000 in January 2014 (which was earned in 2013 upon the filing of the registration statement for the first registered offering of our securities under the Securities Act). Dr. Shah was also granted a restricted stock award in October 2013 pursuant to his offer letter covering 894,685 shares of our common stock. Dr. Shah is entitled to certain severance and change of control benefits pursuant to his agreement, the terms of which are described below under "—Potential Payments upon Termination or Change of Control."

Mr. Schmid. We entered into an offer letter agreement with Mr. Schmid in August 2013 setting forth the terms of his employment as our Chief Financial Officer. Pursuant to the agreement, Mr. Schmid is entitled to an initial annual base salary of $275,000, which in May 2014 was increased by the board of directors to $340,000, and an annual bonus of up to 30% of his annual base salary as determined by the board of directors, which in May 2014 was increased by the board of directors to 35% of his annual base salary. Mr. Schmid was also granted a restricted stock award in September 2013 covering 144,444 shares of our common stock. Mr. Schmid is entitled to certain severance and change of control benefits as described below under "—Potential Payments upon Termination or Change of Control."

Dr. Chowrira. We entered into an offer letter agreement with Dr. Chowrira in October 2013 setting forth the terms of his employment as our Chief Operating Officer. Pursuant to the agreement, Dr. Chowrira is entitled to an initial annual base salary of $300,000, which in May 2014 was increased by the board of directors to $390,000, and an annual bonus of up to 30% of his annual base salary based on achievement of individual and corporate goals determined by the board of directors, which in May 2014 was increased by the board of directors to 35% of his annual base salary. Dr. Chowrira was also granted an option to purchase 297,777 shares of our common stock in October 2013. Dr. Chowrira is entitled to certain severance and change of control benefits as described below under "—Potential Payments upon Termination or Change of Control."

Potential payments upon termination or change of control

Regardless of the manner in which a named executive officer's service terminates, the named executive officer is entitled to receive amounts earned during his or her term of service, including salary and unused vacation pay.

Dr. Shah. Pursuant to his offer letter agreement, if we terminate Dr. Shah's employment without cause or if Dr. Shah terminates his employment as a result of a constructive termination, in each case prior to the 30 days prior to a change of control or more than 12 months following a change of control, subject to his execution of an effective release and waiver of claims in favor of us, Dr. Shah will receive (1) continued payment of his base salary for 12 months, (2) a pro-rated portion of any annual bonus that Dr. Shah would have earned based on

achievement of milestones during the 12-month period following his termination, as determined by the board of directors, (3) payment for continued health benefits under COBRA for up to 12 months and (4) accelerated vesting of all of his outstanding stock awards as if he had completed an additional 12 months of employment with us. If we terminate Dr. Shah's employment without cause or if Dr. Shah terminates his employment as a result of a constructive termination, in each case within the 30 days prior to a change of control or the 12 months following a change of control, subject his execution of an effective release and waiver of claims in favor of us, Dr. Shah will receive (1) continued payment of his base salary for 12 months, (2) any committed annual bonus under his employment agreement for the year of such termination, if applicable, (3) any annual bonus that Dr. Shah would have earned based on achievement of milestones during the 12-month period following this termination, as determined by the board of directors, (4) payment for continued health benefits under COBRA for up to 12 months and (5) accelerated vesting of all of his outstanding stock awards in full.

For purposes of Dr. Shah's offer letter agreement:

- "cause" generally means Dr. Shah's (1) willful failure to substantially perform assigned duties or responsibilities that is not cured within 30 days following written notice; (2) engaging in any act of fraud, embezzlement or other illegal conduct detrimental to us; (3) willful violation of any federal or state law or regulation applicable to our business; (4) material breach of any confidentiality agreement or invention assignment agreement with us, if not cured to our satisfaction within 30 days of written notice; or (5) conviction of or entering a plea of *nolo contendere* to a felony involving an act of moral turpitude.
- "change of control" generally means (1) our acquisition by another entity by means of any transaction or series of related transactions (including any reorganization, merger or consolidation or stock transfer, but excluding such transaction effected primarily for the purpose of changing our domicile), unless our stockholders immediately prior to such transaction hold more than 50% of the voting power of the surviving or acquiring entity immediately after such transaction and excluding our sale of securities for the primary purpose of raising additional funds; or (2) a sale, lease or exclusive license or other disposition of all or substantially all of our assets, other than to an entity, more than 50% of the combined voting power of which is owned by our stockholders in substantially the same proportions as their ownership in us immediately prior to such sale, lease, exclusive license or other disposition.
- "constructive termination" generally means Dr. Shah's termination of employment due to any of the following events after giving us written notice and an opportunity to cure (1) a material breach of the offer letter agreement with us; (2) a material reduction in duties, authority or responsibilities; (3) a material reduction in base salary, other than a reduction of less than 15% applicable to all of our senior management; or (4) our relocation of the principal place for performance of Dr. Shah's duties to a location outside of San Diego County, California, that requires a one-way increase in his commuting distance of more than 50 miles.

Mr. Schmid. Mr. Schmid's offer letter agreement provides for severance benefits under certain circumstances, however such severance provisions are superseded by the executive severance benefit plan, the terms of which are described below.

Dr. Chowrira. Dr. Chowrira's offer letter agreement provides for severance and change of control benefits under certain circumstances, however such severance and change of control provisions are superseded by the executive severance benefit plan, the terms of which are described below.

Executive severance plan

In December 2013, our board of directors approved an executive severance benefit plan, or the severance plan, that provides each of our executive officers, including Dr. Chowrira and Mr. Schmid but not including Dr. Shah, potential severance benefits. Under the severance plan, if we terminate the executive's employment without

cause or if the executive terminates employment for good reason, subject to the executive's execution of an effective release and waiver of claims in favor of us, the executive is entitled to continued payment of base salary for six months following termination and accelerated vesting of stock awards subject to time-based vesting as if the executive had completed an additional six months of employment with us. These severance payments are subject to reduction or elimination in the event that the executive receives compensation during such six month period from full time services with another entity. In addition, if the executive's termination without cause or resignation for good reason occurs within 30 days before or 12 months following a change of control, subject to the executive's execution of an effective release and waiver of claims in favor of us, the executive is entitled to continued payment of base salary for 12 months following termination and accelerated vesting of stock awards in full. The severance plan supersedes any severance benefits the executive is entitled to under his offer letter or employment agreement with us.

For purposes of the severance plan, the definition of "cause" and "change of control" have the same general meaning as in Dr. Shah's offer letter agreements described above except that the severance plan also defines "cause" as a participant's material breach of an employment, consulting or other agreement with us, if such breach is not cured within 30 days of written notice, and a participant's indictment for a felony involving an act of moral turpitude. "Good reason" for purposes of the severance plan generally means the following events, conditions or actions taken by us with respect to the executive without cause and without the executive's express written consent: (1) a material reduction in annual base salary other than in connection with a comparable reduction affecting all officers at such executive's level; (2) a material reduction in the executive's authority, duties or responsibilities; (3) a relocation of the executive's principal place of employment to a location outside of San Diego County that increases the executive's one-way commute by more than 50 miles; or (4) a material breach by us of such executive's offer letter, employment agreement or similar agreement with us.

Equity incentive plans

Each of our named executive officers holds stock options or stock awards under our 2010 Plan and/or our 2014 Plan. A description of the termination and change of control provisions in our 2010 Plan and 2014 Plan is provided below under "—Equity Benefit Plans."

Outstanding equity awards at fiscal year-end

The following table sets forth certain information regarding equity awards granted to our named executive officers that remain outstanding as of December 31, 2014.

		Option awards(1)				Stock awards	
Name	Grant date	Number of securities underlying unexercised options (#) exercisable	Number of securities underlying unexercised options (#) unexercisable	Option exercise price ($)(2)	Option expiration date	Number of shares or units of stock that have not vested (#)	Market value of shares or units of stock that have not vested ($)
Pratik Shah, Ph.D.	1/10/2014	215,153	–	$6.57	1/9/2024	–	–
Pratik Shah, Ph.D.	10/1/2013	–	–	–	–	465,983(3)	$24,454,788(4)
John Schmid	1/10/2014	59,026	–	$6.57	1/9/2024	–	–
John Schmid	9/30/2013	–	–	–	–	99,306(5)	$ 5,211,579(4)
Bharatt Chowrira, Ph.D., J.D.	10/10/2013	297,777	–	$0.54	10/9/2023	–	–

(1) All of the outstanding option awards and stock awards were granted under and subject to the terms of the 2010 Plan, described below under "—Equity Benefit Plans." As of December 31, 2014, each option award listed in the table above may be exercised at any time prior to vesting and any unvested shares acquired upon such "early exercise" are subject to our right to repurchase that lapses according to the vesting schedule of the option. In addition, all vesting of option awards and stock awards is subject to the executive's continuous service with us through the vesting dates and the potential vesting acceleration described above under "—Potential Payments upon Termination or Change of Control."

(2) All of the option awards were granted with a per share exercise price equal to the fair market value of one share of our common stock on the date of grant, which, prior to our initial public offering, was determined in good faith by our board of directors.

(3) Represents the unvested portion of a restricted stock award covering 894,685 shares of our common stock, of which 158,433 shares vested on October 1, 2013, 65,237 shares vested on January 15, 2014 and the remaining shares vest such that 1/48th of the original shares granted vest on the 15th day of each month commencing on February 15, 2014 and ending on January 15, 2017.

(4) Represents the market value of the unvested shares subject to the restricted stock award based on the closing price of our common stock on December 31, 2014, which was $52.48 per share.

(5) Represents the unvested portion of a restricted stock award granted to Mr. Schmid pursuant to his offer letter. The shares vest such that 1/48th of the 144,444 shares granted vest on the last day of the month, commencing on October 31, 2013 and ending on September 30, 2017.

Narrative disclosure to outstanding equity awards at fiscal year-end table

During 2014, there were no modifications or cancellations of any stock options or other equity awards held by our named executive officers. Our named executive officers did not exercise any stock option awards during the fiscal year ended December 31, 2014.

Perquisites, Health, Welfare and Retirement Benefits

Our named executive officers are eligible to participate in our employee benefit plans, including our medical, dental, vision, group life, disability and accidental death and dismemberment insurance plans, in each case on the same basis as all of our other employees. In addition, we provide a medical cash subsidy to any employee, including a named executive officer, who chooses not to participate in our benefit plans described above. We provide a 401(k) plan to our employees, including our current named executive officers, as discussed in the section below entitled "—401(k) Plan."

We generally do not provide perquisites or personal benefits to our named executive officers, except in limited circumstances. We do, however, pay the premiums for term life insurance and disability insurance for all of our employees, including our current named executive officers. Our board of directors may elect to adopt qualified or non-qualified benefit plans in the future if it determines that doing so is in our best interests.

401(k) Plan

We maintain a defined contribution employee retirement plan, or 401(k) plan, for our employees. Our named executive officers are eligible to participate in the 401(k) plan on the same basis as our other employees. The 401(k) plan is intended to qualify as a tax-qualified plan under Section 401(k) of the Internal Revenue Code. The 401(k) plan provides that each participant may contribute up to the lesser of 90% of his or her compensation or the statutory limit, which is $17,500 for calendar year 2014. Participants that are 50 years or older can also make "catch-up" contributions, which in calendar year 2014 may be up to an additional $5,500 above the statutory limit. We currently do not make matching contributions into the 401(k) plan on behalf of participants. Participant contributions are held and invested, pursuant to the participant's instructions, by the plan's trustee.

Nonqualified deferred compensation

We do not maintain nonqualified defined contribution plans or other nonqualified deferred compensation plans. Our board of directors may elect to provide our officers and other employees with non-qualified defined contribution or other nonqualified deferred compensation benefits in the future if it determines that doing so is in our best interests.

Equity benefit plans

2014 Equity incentive plan

Our board of directors and stockholders approved and adopted the 2014 Plan in January 2014. The 2014 Plan became effective upon the date of the underwriting agreement for our initial public offering in February 2014. Upon adoption of the 2014 Plan, we may no longer make grants from its 2010 Plan.

Stock awards. The 2014 Plan provides for the grant of incentive stock options, or ISOs, nonstatutory stock options, or NSOs, stock appreciation rights, restricted stock awards, restricted stock unit awards, performance-based stock awards, and other forms of equity compensation, which we refer to collectively as stock awards, all of which may be granted to employees, including officers, non-employee directors and consultants of us and our affiliates. Additionally, the 2014 Plan provides for the grant of performance cash awards. ISOs may be granted only to employees and the maximum number of shares that may be issued upon the exercise of ISOs under our 2014 Plan is 8,318,652 shares. All other awards may be granted to employees, including officers, and to non-employee directors and consultants.

Share reserve. Initially, the aggregate number of shares of our common stock that may be issued pursuant to stock awards under the 2014 Plan after the 2014 Plan became effective is 1,100,000 new shares, plus an amount not to exceed 3,059,326 shares which represents (1) the number of shares reserved for issuance under our 2010 Plan at the time our 2014 Plan became effective, and (2) any shares subject to outstanding stock options or other stock awards that would have otherwise returned to our 2010 Plan (such as upon the expiration or termination of a stock award prior to vesting). Additionally, the number of shares of our common stock reserved for issuance under our 2014 Plan will automatically increase on January 1 of each year, continuing through and including January 1, 2024, by 4% of the total number of shares of our capital stock outstanding on December 31 of the preceding calendar year, or a lesser number of shares determined by our board of directors. On January 1, 2015, the initial automatic increase pursuant to the 2014 Plan occurred, resulting in 1,109,847 additional shares available for future grant under the 2014 Plan.

No person may be granted stock awards covering more than 2,000,000 shares of our common stock under our 2014 Plan during any calendar year pursuant to stock options, stock appreciation rights and other stock awards whose value is determined by reference to an increase over an exercise or strike price of at least 100% of the fair market value on the date the stock award is granted. Additionally, no person may be granted in a calendar year a performance stock award covering more than 2,000,000 shares or a performance cash award having a maximum value in excess of $2,000,000. Such limitations are designed to help assure that any deductions to which we would otherwise be entitled with respect to such awards will not be subject to the $1,000,000 limitation on the income tax deductibility of compensation paid to any covered executive officer imposed by Section 162(m) of the Code. In addition, a maximum of the greater of 1,000,000 shares of our common stock or such number of shares of common stock that has a fair market value on the grant date equal to $1,000,000 may be granted to any non-employee director during any calendar year pursuant to stock awards.

If a stock award granted under the 2014 Plan or a stock award granted under the 2010 Plan that was outstanding at the time our 2014 Plan became effective expires or otherwise terminates without being exercised in full, or is settled in cash, the shares of our common stock not acquired pursuant to the stock award again will become available for subsequent issuance under the 2014 Plan. In addition, the following types of stock awards granted pursuant to the 2014 Plan (or the 2010 Plan if the award was outstanding at the time our 2014 Plan became effective) may become available for the grant of new stock awards under the 2014 Plan: (1) shares that are forfeited to or repurchased by us prior to becoming fully vested; (2) shares withheld to satisfy income or employment withholding taxes; or (3) shares used to pay the exercise or purchase price of a stock award. Shares issued under the 2014 Plan may be previously unissued shares or reacquired shares

bought by us on the open market. As of September 30, 2014, options covering 476,667 shares were outstanding under our 2014 Plan and 1,167,253 shares remained available for the grant of new stock awards under our 2014 Plan.

Administration. Our board of directors, or a duly authorized committee thereof, has the authority to administer the 2014 Plan. Our board of directors may also delegate to one or more of our officers the authority to (1) designate employees (other than other officers) to be recipients of certain stock awards, and (2) determine the number of shares of common stock to be subject to such stock awards. Subject to the terms of the 2014 Plan, our board of directors or the authorized committee, referred to herein as the plan administrator, determines recipients, dates of grant, the numbers and types of stock awards to be granted and the terms and conditions of the stock awards, including the period of their exercisability and vesting schedule applicable to a stock award. Subject to the limitations set forth below, the plan administrator will also determine the exercise price, strike price or purchase price of awards granted and the types of consideration to be paid for the award.

The plan administrator has the authority to modify outstanding awards under our 2014 Plan. Subject to the terms of our 2014 Plan, the plan administrator has the authority to reduce the exercise, purchase or strike price of any outstanding stock award, cancel any outstanding stock award in exchange for new stock awards, cash or other consideration, or take any other action that is treated as a repricing under generally accepted accounting principles, with the consent of any adversely affected participant.

Stock options. ISOs and NSOs are granted pursuant to stock option agreements adopted by the plan administrator. The plan administrator determines the exercise price for a stock option, within the terms and conditions of the 2014 Plan, provided that the exercise price of a stock option generally cannot be less than 100% of the fair market value of our common stock on the date of grant. Options granted under the 2014 Plan vest at the rate specified by the plan administrator.

The plan administrator determines the term of stock options granted under the 2014 Plan, up to a maximum of 10 years. Unless the terms of an option holder's stock option agreement provide otherwise, if an option holder's service relationship with us, or any of our affiliates, ceases for any reason other than disability, death or cause, the option holder may generally exercise any vested options for a period of three months following the cessation of service. The option term may be extended in the event that exercise of the option following such a termination of service is prohibited by applicable securities laws or our insider trading policy. If an optionholder's service relationship with us or any of our affiliates ceases due to disability or death, or an optionholder dies within a certain period following cessation of service, the optionholder or a beneficiary may generally exercise any vested options for a period of 12 months in the event of disability and 18 months in the event of death. In the event of a termination for cause, options generally terminate immediately upon the termination of the individual for cause. In no event may an option be exercised beyond the expiration of its term.

Acceptable consideration for the purchase of common stock issued upon the exercise of a stock option will be determined by the plan administrator and may include (1) cash, check, bank draft or money order, (2) a broker-assisted cashless exercise, (3) the tender of shares of our common stock previously owned by the optionholder, (4) a net exercise of the option if it is an NSO, and (5) other legal consideration approved by the plan administrator.

Unless the plan administrator provides otherwise, options generally are not transferable except by will, the laws of descent and distribution, or pursuant to a domestic relations order. An optionholder may designate a beneficiary, however, who may exercise the option following the optionholder's death.

Tax limitations on incentive stock options. The aggregate fair market value, determined at the time of grant, of our common stock with respect to ISOs that are exercisable for the first time by an optionholder during any

calendar year under all of our stock plans and the stock plans of any of our affiliates may not exceed $100,000. Options or portions thereof that exceed such limit will generally be treated as NSOs. No ISO may be granted to any person who, at the time of the grant, owns or is deemed to own stock possessing more than 10% of our total combined voting power or that of any of our affiliates unless (1) the option exercise price is at least 110% of the fair market value of the stock subject to the option on the date of grant, and (2) the term of the ISO does not exceed five years from the date of grant.

Restricted stock awards. Restricted stock awards are granted pursuant to restricted stock award agreements adopted by the plan administrator. Restricted stock awards may be granted in consideration for (1) cash, check, bank draft or money order, (2) services rendered to us or our affiliates, or (3) any other form of legal consideration. Common stock acquired under a restricted stock award may, but need not, be subject to a share repurchase option in our favor in accordance with a vesting schedule to be determined by the plan administrator. A restricted stock award may be transferred only upon such terms and conditions as set by the plan administrator. Except as otherwise provided in the applicable award agreement, restricted stock that has not vested will be forfeited or repurchased by us upon the participant's cessation of continuous service for any reason.

Restricted stock unit awards. Restricted stock unit awards are granted pursuant to restricted stock unit award agreements adopted by the plan administrator. Restricted stock unit awards may be granted in consideration for any form of legal consideration. A restricted stock unit award may be settled by cash, delivery of stock, a combination of cash and stock as deemed appropriate by the plan administrator, or in any other form of consideration set forth in the restricted stock unit award agreement. Additionally, dividend equivalents may be credited in respect of shares covered by a restricted stock unit award. Except as otherwise provided in the applicable award agreement, restricted stock units that have not vested will be forfeited upon the participant's cessation of continuous service for any reason.

Stock appreciation rights. Stock appreciation rights are granted pursuant to stock appreciation grant agreements adopted by the plan administrator. The plan administrator determines the strike price for a stock appreciation right, which generally cannot be less than 100% of the fair market value of our common stock on the date of grant. Upon the exercise of a stock appreciation right, we will pay the participant an amount equal to the product of (1) the excess of the per share fair market value of our common stock on the date of exercise over the strike price, multiplied by (2) the number of shares of common stock with respect to which the stock appreciation right is exercised. A stock appreciation right granted under the 2014 Plan vests at the rate specified in the stock appreciation right agreement as determined by the plan administrator.

The plan administrator determines the term of stock appreciation rights granted under the 2014 Plan, up to a maximum of ten years. Unless the terms of a participant's stock appreciation right agreement provides otherwise, if a participant's service relationship with us or any of our affiliates ceases for any reason other than cause, disability or death, the participant may generally exercise any vested stock appreciation right for a period of three months following the cessation of service. The stock appreciation right term may be further extended in the event that exercise of the stock appreciation right following such a termination of service is prohibited by applicable securities laws. If a participant's service relationship with us, or any of our affiliates, ceases due to disability or death, or a participant dies within a certain period following cessation of service, the participant or a beneficiary may generally exercise any vested stock appreciation right for a period of 12 months in the event of disability and 18 months in the event of death. In the event of a termination for cause, stock appreciation rights generally terminate immediately upon the occurrence of the event giving rise to the termination of the individual for cause. In no event may a stock appreciation right be exercised beyond the expiration of its term.

Performance awards. The 2014 Plan permits the grant of performance-based stock and cash awards that may qualify as performance-based compensation that is not subject to the $1,000,000 limitation on the income tax deductibility of compensation paid to a covered executive officer imposed by Section 162(m) of the Code. To help assure that the compensation attributable to performance-based awards will so qualify, our compensation committee can structure such awards so that stock or cash will be issued or paid pursuant to such award only after the achievement of certain pre-established performance goals during a designated performance period.

The performance goals that may be selected include one or more of the following: (1) earnings (including earnings per share and net earnings); (2) earnings before interest, taxes and depreciation; (3) earnings before interest, taxes, depreciation and amortization; (4) earnings before interest, taxes, depreciation, amortization and legal settlements; (5) earnings before interest, taxes, depreciation, amortization, legal settlements and other income (expense); (6) earnings before interest, taxes, depreciation, amortization, legal settlements, other income (expense) and stock-based compensation; (7) earnings before interest, taxes, depreciation, amortization, legal settlements, other income (expense), stock-based compensation and changes in deferred revenue; (8) total stockholder return; (9) return on equity or average stockholders' equity; (10) return on assets, investment, or capital employed; (11) stock price; (12) margin (including gross margin); (13) income (before or after taxes); (14) operating income; (15) operating income after taxes; (16) pre-tax profit; (17) operating cash flow; (18) sales or revenue targets; (19) increases in revenue or product revenue; (20) expenses and cost reduction goals; (21) improvement in or attainment of working capital levels; (22) economic value added (or an equivalent metric); (23) market share; (24) cash flow; (25) cash flow per share; (26) share price performance; (27) debt reduction; (28) stockholders' equity; (29) capital expenditures; (30) debt levels; (31) operating profit or net operating profit; (32) workforce diversity; (33) growth of net income or operating income; (34) billings; (35) bookings; (36) employee retention; (37) initiation of phases of clinical trials and/or studies by specific dates; (38) patient enrollment rates; (39) budget management; (40) submission to, or approval by, a regulatory body (including, but not limited to the FDA) of an applicable filing or a product candidate; (41) implementation or completion of projects or processes (including, without limitation, clinical trial initiation, clinical trial enrollment, clinical trial results, new and supplemental indications for existing products, regulatory filing submissions, regulatory filing acceptances, regulatory or advisory committee interactions, regulatory approvals, and product supply); (42) regulatory milestones; (43) progress of internal research or clinical programs; (44) progress of partnered programs; (45) partner satisfaction; (46) timely completion of clinical trials; (47) submission of INDs and NDAs and other regulatory achievements; (48) research progress, including the development of programs; (49) strategic partnerships or transactions (including in-licensing and out-licensing of intellectual property; and (50) to the extent that an award is not intended to comply with Section 162(m) of the Code, other measures of performance selected by our board of directors.

The performance goals may be based on a company-wide basis, with respect to one or more business units, divisions, affiliates, or business segments, and in either absolute terms or relative to the performance of one or more comparable companies or the performance of one or more relevant indices. Unless specified otherwise (1) in the award agreement at the time the award is granted or (2) in such other document setting forth the performance goals at the time the goals are established, we will appropriately make adjustments in the method of calculating the attainment of performance goals as follows: (a) to exclude restructuring and/or other nonrecurring charges; (b) to exclude exchange rate effects; (c) to exclude the effects of changes to generally accepted accounting principles; (d) to exclude the effects of any statutory adjustments to corporate tax rates; (e) to exclude the effects of any "extraordinary items" as determined under generally accepted accounting principles; (f) to exclude the dilutive effects of acquisitions or joint ventures; (g) to assume that any business divested by us achieved performance objectives at targeted levels during the balance of a performance period following such divestiture; (h) to exclude the effect of any change in the outstanding shares of our common stock by reason of any stock dividend or split, stock repurchase, reorganization, recapitalization, merger,

consolidation, spin-off, combination or exchange of shares or other similar corporate change, or any distributions to common stockholders other than regular cash dividends; (i) to exclude the effects of stock based compensation and the award of bonuses under our bonus plans; (j) to exclude costs incurred in connection with potential acquisitions or divestitures that are required to be expensed under generally accepted accounting principles; (k) to exclude the goodwill and intangible asset impairment charges that are required to be recorded under generally accepted accounting principles; (l) to exclude the effect of any other unusual, non-recurring gain or loss or other extraordinary item; and (m) to exclude the effects of the timing of acceptance for review and/or approval of submissions to the FDA or any other regulatory body. In addition, we retain the discretion to reduce or eliminate the compensation or economic benefit due upon attainment of the goals. The performance goals may differ from participant to participant and from award to award.

Other stock awards. The plan administrator may grant other awards based in whole or in part by reference to our common stock. The plan administrator will set the number of shares under the stock award and all other terms and conditions of such awards.

Changes to capital structure. In the event that there is a specified type of change in our capital structure, such as a stock split or recapitalization, appropriate adjustments will be made to (1) the class and maximum number of shares reserved for issuance under the 2014 Plan, (2) the class and maximum number of shares by which the share reserve may increase automatically each year, (3) the class and maximum number of shares that may be issued upon the exercise of ISOs, (4) the class and maximum number of shares subject to stock awards that can be granted in a calendar year (as established under the 2014 Plan pursuant to Section 162(m) of the Code), (5) the class and maximum number of shares that may be awarded to any non-employees director and (6) the class and number of shares and exercise price, strike price, or purchase price, if applicable, of all outstanding stock awards.

Corporate transactions. In the event of certain specified significant corporate transactions, the plan administrator has the discretion to take any of the following actions with respect to stock awards:

- arrange for the assumption, continuation or substitution of a stock award by a surviving or acquiring entity or parent company;
- arrange for the assignment of any reacquisition or repurchase rights held by us to the surviving or acquiring entity or parent company;
- accelerate the vesting of the stock award and provide for its termination prior to the effective time of the corporate transaction;
- arrange for the lapse of any reacquisition or repurchase right held by us;
- cancel or arrange for the cancellation of the stock award in exchange for such cash consideration, if any, as our board of directors may deem appropriate; or
- make a payment equal to the excess of (a) the value of the property the participant would have received upon exercise of the stock award over (b) the exercise price otherwise payable in connection with the stock award.

Our plan administrator is not obligated to treat all stock awards, even those that are of the same type, in the same manner.

Under the 2014 Plan, a corporate transaction is generally the consummation of (1) a sale or other disposition of all or substantially all of our assets, (2) a sale or other disposition of at least 90% of our outstanding securities, (3) a merger, consolidation or similar transaction following which we are not the surviving corporation, or (4) a

merger, consolidation or similar transaction following which we are the surviving corporation but the shares of our common stock outstanding immediately prior to such transaction are converted or exchanged into other property by virtue of the transaction.

Change of control. The plan administrator may provide, in an individual award agreement or in any other written agreement between a participant and us that the stock award will be subject to additional acceleration of vesting and exercisability in the event of a change of control. Under the 2014 Plan, a change of control is generally (1) the acquisition by a person or entity of more than 50% of our combined voting power other than by merger, consolidation or similar transaction; (2) a consummated merger, consolidation or similar transaction immediately after which our stockholders cease to own more than 50% of the combined voting power of the surviving entity; (3) a consummated sale, lease or exclusive license or other disposition of all or substantially of our assets; (4) a complete dissolution or liquidation of the Company, except for a liquidation into a parent corporation, or (5) when a majority of our board of directors becomes comprised of individuals who were not serving on our board of directors on the date of adoption of the 2014 Plan, or the incumbent board, or whose nomination, appointment, or election was not approved by a majority of the incumbent board still in office.

Amendment and termination. Our board of directors has the authority to amend, suspend, or terminate our 2014 Plan, provided that such action does not materially impair the existing rights of any participant without such participant's written consent. No ISOs may be granted after the tenth anniversary of the date our board of directors adopted our 2014 Plan.

2010 Equity incentive plan

Our board of directors and our stockholders approved our 2010 Plan and it became effective in June 2010 and was subsequently amended by our board of directors and stockholders most recently in December 2013. Our 2010 Plan was a continuation of and successor to our 2001 Plan and after our 2010 Plan became effective, no further stock awards were granted under our 2001 Plan. In connection with our initial public offering we adopted the 2014 Plan, which was the successor to and continuation of our 2010 Plan. Therefore, no further stock awards may be granted under our 2010 Plan. As of September 30, 2014, there were a total of 1,483,793 stock options and 691,898 shares from unvested restricted stock awards outstanding under our 2010 Plan.

Stock awards. The 2010 Plan provided for the grant of ISO, NSOs, stock appreciation rights, restricted stock awards and restricted stock unit awards, which we refer to collectively as stock awards, all of which may be granted to employees, including officers, non-employee directors and consultants of us and our affiliates. ISOs may be granted only to employees. All other awards may be granted to employees, including officers, and to non-employee directors and consultants. We have only granted stock options and restricted stock awards under the 2010 Plan.

Share reserve. As of our initial public offering in February 2014, there were no shares of our common stock available for issuance under the 2010 Plan. Outstanding awards under our 2010 Plan as of the effective date of our 2014 Plan that are repurchased, forfeited, expire or are cancelled will become available for grant under our 2014 Plan in accordance with its terms.

Administration. Our board of directors, or a duly authorized committee thereof, has the authority to administer the 2010 Plan. Subject to the terms of the 2010 Plan, our board of directors or the authorized committee, referred to herein as the plan administrator, determined recipients, dates of grant, the numbers and types of stock awards to be granted and the terms and conditions of the stock awards, including the period of their exercisability and vesting schedule applicable to a stock award. Subject to the limitations set forth below, the plan administrator also determined the exercise price, strike price or purchase price of awards granted and the types of consideration to be paid for the award.

The plan administrator has the authority to modify outstanding awards under our 2010 Plan. Subject to the terms of our 2010 Plan, the plan administrator has the authority to reduce the exercise or strike price of any outstanding stock award, cancel any outstanding stock award in exchange for new stock awards, cash or other consideration, or take any other action that is treated as a repricing under generally accepted accounting principles, with the consent of any adversely affected participant.

Stock options. Incentive and nonstatutory stock options are granted pursuant to stock option agreements adopted by the plan administrator. The plan administrator determined the exercise price for a stock option, within the terms and conditions of the 2010 Plan, provided that the exercise price of a stock option generally cannot be less than 100% of the fair market value of our common stock on the date of grant. Options granted under the 2010 Plan vest at the rate specified by the plan administrator.

The plan administrator determined the term of stock options granted under the 2010 Plan, up to a maximum of 10 years. Unless the terms of an option holder's stock option agreement provide otherwise, if an option holder's service relationship with us, or any of our affiliates, ceases for any reason other than disability, death or cause, the option holder may generally exercise any vested options for a period of three months following the cessation of service, with respect to employee option holders, or twelve months following the cessation of service, with respect to consultants or Board of Director member option holders. The option term may be extended in the event that exercise of the option following such a termination of service is prohibited by applicable securities laws or our insider trading policy. If an optionholder's service relationship with us or any of our affiliates ceases due to disability or death, or an optionholder dies within a certain period following cessation of service, the optionholder or a beneficiary may generally exercise any vested options for a period of six months following the cessation of service or death, as applicable. In the event of a termination for cause, options generally terminate 30 days following the employee's termination. In no event may an option be exercised beyond the expiration of its term.

Acceptable consideration for the purchase of common stock issued upon the exercise of a stock option are determined by the plan administrator and may include (1) cash, check, bank draft or money order, (2) a broker-assisted cashless exercise, (3) the tender of shares of our common stock previously owned by the optionholder, (4) a net exercise of the option if it is an NSO, and (5) other legal consideration approved by the plan administrator.

Unless the plan administrator provides otherwise, options generally are not transferable except by will, the laws of descent and distribution, or pursuant to a domestic relations order. An optionholder may designate a beneficiary, however, who may exercise the option following the optionholder's death.

Tax limitations on incentive stock options. The aggregate fair market value, determined at the time of grant, of our common stock with respect to ISOs that are exercisable for the first time by an optionholder during any calendar year under all of our stock plans may not exceed $100,000. Options or portions thereof that exceed such limit will generally be treated as NSOs. No ISO may be granted to any person who, at the time of the grant, owns or is deemed to own stock possessing more than 10% of our total combined voting power or that of any of our affiliates unless (1) the option exercise price is at least 110% of the fair market value of the stock subject to the option on the date of grant, and (2) the option is not exercisable after the expiration of five years from the date of grant.

Restricted stock awards. Restricted stock awards are granted pursuant to restricted stock award agreements adopted by the plan administrator. Restricted stock awards may be granted in consideration for (1) cash or cash equivalents, (2) past services rendered to us or our affiliates, or (3) any other form of legal consideration acceptable to the plan administrator and permissible under applicable law. Common stock acquired under a restricted stock award may, but need not, be subject to a forfeiture condition in our favor in accordance with a

vesting schedule to be determined by the plan administrator. Restricted stock awards may be transferred only upon such terms and conditions as set by the plan administrator. Upon the participant's cessation of continuous service for any reason, we generally may receive any restricted stock that has not vested as of such cessation of service through a forfeiture condition or a repurchase right. Any right we have to repurchase unvested shares will generally be at a price equal to the lower of the fair market value of the shares on the date of repurchase or the original purchase price for the shares.

Changes to capital structure. In the event that there is a specified type of change in our capital structure, such as a stock split or recapitalization, appropriate adjustments will be made to the class and number of shares and price per share of stock subject to all outstanding stock awards.

Corporate transactions. In the event of certain specified significant corporate transactions, unless otherwise provided in a stock award or other written agreement between us and the holder of a stock award, the plan administrator has the discretion to take any of the following actions with respect to stock awards:

- arrange for the assumption, continuation or substitution of a stock award by a surviving or acquiring entity or its parent company;
- arrange for the assignment of any reacquisition or repurchase rights held by us with respect to the shares covered by the stock award to the surviving or acquiring entity or its parent company;
- accelerate the vesting and exercisability, if applicable, of the stock award and provide for its termination at or prior to the effective time of the corporate transaction;
- arrange for the lapse of any reacquisition or repurchase right held by us with respect to the shares covered by the stock award;
- cancel or arrange for the cancellation of the stock award, to the extent not vested or not exercised prior to the effective time of the corporate transaction, in exchange for such cash consideration, if any, as our board of directors may deem appropriate; or
- make a payment in such form determined by our board of directors equal to the excess of (a) the value of the property the holder of the stock award would have received upon exercise of the stock award over (b) any exercise price otherwise payable by such holder in connection with the stock award.

Our plan administrator is not obligated to treat all stock awards or portions thereof or all holders of stock awards, even those that are of the same type, in the same manner.

Under the 2010 Plan, a corporate transaction is generally the consummation of (1) a sale or other disposition of all or substantially all of our assets, (2) a sale or other disposition of at least 90% of our outstanding securities, (3) a merger, consolidation or similar transaction following which we are not the surviving corporation, or (4) a merger, consolidation or similar transaction following which we are the surviving corporation but the shares of our common stock outstanding immediately prior to such transaction are converted or exchanged into other property by virtue of the transaction.

Change of control. The plan administrator may provide, in an individual award agreement or in any other written agreement between a participant and us that the stock award will be subject to additional acceleration of vesting and exercisability in the event of a change of control. Under the 2010 Plan, a change of control is generally (1) the acquisition by a person or entity of more than 50% of our combined voting power other than by merger, consolidation or similar transaction; (2) a consummated merger, consolidation or similar transaction involving us immediately after which our stockholders cease to own more than 50% of the combined voting power of the surviving entity or of its parent entity; (3) our complete dissolution or liquidation or approval by

our stockholders or our board of directors of a plan of complete dissolution or liquidation of us; (4) a consummated sale, lease or exclusive license or other disposition of all or substantially of our assets; or (5) when a majority of our board of directors becomes comprised of individuals who were not serving on our board of directors on the date of adoption of the 2010 Plan, or the incumbent board, or whose nomination, appointment, or election was not approved by a majority of the incumbent board then still in office.

Amendment and termination. The 2010 Plan was originally scheduled to terminate on June 29, 2020. However, the 2010 Plan was succeeded by our 2014 Plan in connection with our initial public offering in February 2014.

2014 Employee stock purchase plan

Our board of directors and stockholders approved and adopted the ESPP in January 2014. The ESPP became effective upon the date of the underwriting agreement for our initial public offering in February 2014. The purpose of the ESPP is to retain the services of new employees and secure the services of new and existing employees while providing incentives for such individuals to exert maximum efforts toward our success and that of our affiliates.

Share reserve. The ESPP authorizes the issuance of 300,000 shares of our common stock pursuant to purchase rights granted to our employees or to employees of any of our designated affiliates. The number of shares of our common stock reserved for issuance will automatically increase on January 1 of each calendar year, through January 1, 2024 by the least of (a) 1% of the total number of shares of our common stock outstanding on December 31 of the preceding calendar year, (b) 530,000 shares, or (c) a number determined by our board of directors that is less than (a) and (b). The ESPP is intended to qualify as an "employee stock purchase plan" within the meaning of Section 423 of the Code. On January 1, 2015, the initial automatic increase pursuant to the ESPP occurred, resulting in 277,461 additional shares available for future grant under the ESPP.

Administration. Our board of directors has delegated its authority to administer the ESPP to our compensation committee. The ESPP is implemented through a series of offerings of purchase rights to eligible employees. Under the ESPP, we may specify offerings with durations of not more than 27 months, and may specify shorter purchase periods within each offering. Each offering will have one or more purchase dates on which shares of our common stock will be purchased for employees participating in the offering. An offering may be terminated under certain circumstances.

Payroll deductions. Generally, all regular employees, including executive officers, employed by us or by any of our designated affiliates, may participate in the ESPP and may contribute, normally through payroll deductions, up to 15% of their earnings for the purchase of our common stock under the ESPP. Unless otherwise determined by our board of directors, common stock will be purchased for accounts of employees participating in the ESPP at a price per share equal to the lower of (a) 85% of the fair market value of a share of our common stock on the first date of an offering or (b) 85% of the fair market value of a share of our common stock on the date of purchase.

Limitations. Employees may have to satisfy one or more of the following service requirements before participating in the ESPP, as determined by our board of directors: (a) customarily employed for more than 20 hours per week, (b) customarily employed for more than five months per calendar year or (c) continuous employment with us or one of our affiliates for a period of time (not to exceed two years). No employee may purchase shares under the ESPP at a rate in excess of $25,000 worth of our common stock based on the fair market value per share of our common stock at the beginning of an offering for each year such a purchase right is outstanding. Finally, no employee will be eligible for the grant of any purchase rights under the ESPP if immediately after such rights are granted, such employee has voting power over 5% or more of our outstanding capital stock measured by vote or value pursuant to Section 424(d) of the Code.

Changes to capital structure. In the event that there occurs a change in our capital structure through such actions as a stock split, merger, consolidation, reorganization, recapitalization, reincorporation, stock dividend, dividend in property other than cash, large nonrecurring cash dividend, liquidating dividend, combination of shares, exchange of shares, change in corporate structure or similar transaction, the board of directors will make appropriate adjustments to (a) the class(es) and number of shares reserved under the ESPP, (b) the class(es) and maximum number of shares by which the share reserve may increase automatically each year, (c) the class(es) and number of shares and purchase price of all outstanding purchase rights and (d) the class(es) and number of shares that are the subject of the purchase limits under each ongoing offering.

Corporate transactions. In the event of certain significant corporate transactions, including: (1) a sale of all our assets, (2) the sale or disposition of 90% of our outstanding securities, (3) the consummation of a merger or consolidation where we do not survive the transaction, and (4) the consummation of a merger or consolidation where we do survive the transaction but the shares of our common stock outstanding immediately prior to such transaction are converted or exchanged into other property by virtue of the transaction, any then-outstanding rights to purchase our stock under the ESPP may be assumed, continued or substituted for by any surviving or acquiring entity (or its parent company). If the surviving or acquiring entity (or its parent company) elects not to assume, continue or substitute for such purchase rights, then the participants' accumulated payroll contributions will be used to purchase shares of our common stock within 10 business days prior to such corporate transaction, and such purchase rights will terminate immediately.

Plan amendments, termination. Our board of directors has the authority to amend or terminate our ESPP, provided that except in certain circumstances any such amendment or termination may not materially impair any outstanding purchase rights without the holder's consent. We will obtain stockholder approval of any amendment to our ESPP as required by applicable law or listing requirements.

Director compensation

Prior to February 2014, we had not paid cash compensation to directors for their service on our board of directors nor had we paid equity compensation to our non-employee directors who were nominated by our principal stockholders for service on our board of directors. Alternatively, we provided equity compensation to our non-employee directors who were not nominated by our principal stockholders from time to time when we determined appropriate.

In January 2014, in connection with Mr. Proehl's appointment to the board of directors, Mr. Proehl was granted an option to purchase 13,333 shares of our common stock with an exercise price of $6.57 per share that vests in full on the one year anniversary of the date of grant, subject to Mr. Proehl's continued service with us. In connection with Mr. Schneider's appointment to the board of directors in January 2014, Mr. Schneider was granted an option to purchase 13,333 shares of our common stock effective and contingent upon the pricing of our initial public offering, at an exercise price per share equal to the price per share at which our common stock was first sold to the public in our initial public offering, which was $12.00 per share. Mr. Schneider's option grant will vest in full on the one year anniversary of the date of grant, subject to Mr. Schneider's continued service with us.

In February 2014, our board of directors adopted a new compensation policy that is applicable to all of our non-employee directors. Our board of directors approved amendments to this compensation policy in May 2014 and January 2015. This compensation policy provides that each such non-employee director will receive the following compensation for service on our board of directors:

- an annual cash retainer of $35,000;
- an additional cash retainer of $25,000 to the chairman of the board of directors;

- an additional cash retainer of $20,000 to the lead independent director of the board of directors;
- an additional annual cash retainer of $7,500, $5,000 and $3,500 for service as a member of the audit committee, compensation committee and the nominating and corporate governance committee, respectively;
- an additional annual cash retainer of $7,500, $5,000 and $3,500 for service as chairman of the audit committee, compensation committee and the nominating and corporate governance committee, respectively; and
- an initial option grant to purchase of 20,000 shares of our common stock when first elected or appointed to our board of directors and an annual option grant to purchase 12,000 shares of our common stock for each non-employee director serving on the board of directors on the date of our annual stockholder meeting, vesting one year following the grant date.

Pursuant to the adoption of the compensation policy, we began paying cash compensation to non-employee directors effective February 2014. Further, upon the appointment of Ms. Bleil and Mr. Greer to our board of directors in May 2014, each were granted initial option grants to purchase 20,000 shares of our common stock. Also in May 2014, our board of directors determined it was in the best interests of the Company to grant Mr. Proehl and Mr. Schneider each an option to purchase 6,667 shares of our common stock, which made their previous award grants consistent with the new compensation policy.

Each of the option grants described above as well as the January 2014 option grants to Mr. Proehl and Mr. Schneider will vest and become exercisable subject to the director's continuous service to us, provided that each option will vest in full upon a change of control (as defined under our 2014 Plan). The term of each option will be 10 years, subject to earlier termination as provided in the 2014 Plan, except that the post-termination exercise period will be 12 months from the date of termination, if such termination is other than for cause or due to death or disability. The options will be granted under our 2014 Plan, the terms of which are described in more detail above under "—Equity Benefit Plans—2014 Equity Incentive Plan."

We have reimbursed and will continue to reimburse all of our non-employee directors for their travel, lodging and other reasonable expenses incurred in attending meetings of our board of directors and committees of our board of directors.

The following table sets forth in summary form information concerning the compensation that we paid or awarded during the year ended December 31, 2014 to each of our non-employee directors:

Name(1)	Fees earned or paid in cash ($)	Option awards ($)(2)	Total ($)
Lynn Bleil	$25,132	$246,048	$271,180
David Collier, M.D.(3)	$ —	$ —	$ —
Rod Ferguson, Ph.D.	$39,392	$ —	$ 39,392
Scott Greer	$31,007	$246,048	$277,055
Gerald Proehl	$45,931	$142,533	$188,464
Sepehr Sarshar, Ph.D.	$38,486	$ —	$ 38,486
Samuel Saks(4)	$ —	$ —	$ —
Phillip Schneider	$45,278	$189,574	$234,852
Alex Zisson	$47,542	$ —	$ 47,542

(1) Dr. Shah was also a director during 2014; as a named executive officer, his compensation is fully reflected in the "—Summary Compensation Table" above. Dr. Shah did not receive any compensation in 2014 for services he provided as a member of our board of directors.

(2) In accordance with SEC rules, this column reflects the aggregate fair value of the stock awards and option awards granted in 2014 computed as of their respective grant dates in accordance with ASC 718. Assumptions used in the calculation of these amounts are included in the notes to our financial statements incorporated by reference into this prospectus. These amounts do not reflect the actual economic value that will be realized by the non-employee director upon the vesting of the stock awards or option awards, the exercise of the option awards or the sale of the common stock underlying such stock awards or option awards. The aggregate number of shares subject to each director's outstanding and unexercised option awards and outstanding stock awards as of December 31, 2014 was as follows:

Name	Aggregate number of outstanding unvested restricted stock awards at December 31, 2014 (#)	Aggregate number of option awards outstanding at December 31, 2014 (#)
Lynn Bleil	–	20,000
David Collier, M.D.(3)	–	–
Rod Ferguson, Ph.D.	–	–
Scott Greer	–	20,000
Gerald Proehl	–	20,000
Sepehr Sarshar, Ph.D.	–	–
Samuel Saks, M.D.	46,248	82,789
Phillip Schneider	–	20,000
Alex Zisson	–	–

(3) Dr. Collier resigned from the board of directors effective January 7, 2014.

(4) Dr. Saks became an executive officer in November 2013 and he received no compensation as a director in 2014. As an executive officer, Dr. Saks earned a base salary of $173,125 and a discretionary bonus of $282,500 for 2014. We also paid Dr. Saks' life and disability premiums of $1,317 in 2014. No equity awards were granted to Dr. Saks in 2014.

Certain relationships and related party transactions

The following includes a summary of transactions since January 1, 2012 to which we have been a party, in which the amount involved in the transaction exceeded $120,000, and in which any of our directors, executive officers or, to our knowledge, beneficial owners of more than 5% of our capital stock or any member of the immediate family of any of the foregoing persons had or will have a direct or indirect material interest, other than equity and other compensation, termination, change in control and other arrangements, which are described under "Executive And Director Compensation."

Loan arrangements

Since January 1, 2012, we have entered into various loan arrangements with beneficial owners of more than 5% of our capital stock or entities affiliated with certain of our directors, pursuant to which we have issued convertible promissory notes and warrants. The participants in these loan arrangements included the following holders of more than 5% of our capital stock, or entities affiliated with them, or entities affiliated with certain of our directors. The following table presents the aggregate principal amount of convertible promissory notes issued to these related parties in these loan arrangements:

Participants(1)	Aggregate principal amount of notes
Thomas, McNerney & Partners(2)	$3,395,000
CMEA Capital(3)	$2,425,000
Sloan Biotech Fund(4)	$ 180,000

(1) Additional detail regarding these stockholders and their equity holdings is provided in "Principal Stockholders."

(2) Consisted of $3,347,470 aggregate principal amount of notes issued to TMP, $34,969 aggregate principal amount of notes issued to TMP Nominee II, LLC and $12,562 aggregate principal amount of notes issued to TMP Associates II, L.P.

(3) Consisted of $2,364,376 aggregate principal amount of notes issued to CMEA and $60,625 aggregate principal amount of notes issued to CMEA Ventures VII (Parallel), L.P.

(4) Consisted of $90,000 aggregate principal amount of notes issued to Sloan Biotech Fund and $90,000 aggregate principal amount of notes issued to Sloan Biotech Fund II.

In October 2012, the holders of our outstanding convertible promissory notes agreed to the cancellation of their notes in exchange for an aggregate of 11,012,456 shares of our Series D Preferred Stock.

In addition to convertible promissory notes, each lender received warrants to purchase shares of our preferred stock in each debt financing. Upon the completion of our initial public offering in February 2014, the outstanding warrants became exercisable for shares of our common stock. The following table presents the aggregate number of shares of common stock issuable upon exercise of the warrants issued to each lender in connection with the loan arrangements described above:

Participants(1)	Warrants to purchase shares of common stock(2)
Thomas, McNerney & Partners(3)	90,228
CMEA Capital(4)	64,449

(1) Additional detail regarding these stockholders and their equity holdings is provided in "Principal Stockholders."

(2) The warrants were originally exercisable for Series C Preferred Stock but became exercisable for Series D Preferred Stock upon the closing of the Series D Preferred Stock financing and then became exercisable for common stock upon the completion of our initial public offering.

(3) Consists of (a) 88,966 shares of common stock issuable upon exercise of warrants issued to TMP, (b) 929 shares of common stock issuable upon exercise of warrants issued to TMP Nominee II, LLC and (c) 333 shares of common stock issuable upon exercise of warrants issued to TMP Associates II, L.P.

(4) Consists of (a) 62,838 shares of common stock issuable upon exercise of warrants issued to CMEA and (b) 1,611 shares of common stock issuable upon exercise of warrants issued to CMEA Ventures VII (Parallel), L.P.

Series D Preferred Stock Financing

In October 2012, we entered into a Series D Preferred Stock Purchase Agreement, or the Series D Purchase Agreement, and held the closing of the first tranche of the Series D Preferred Stock financing, pursuant to which we issued and sold an aggregate of 20,293,201 shares of our Series D Preferred Stock, which includes the 11,012,456 shares of Series D Preferred Stock issued to noteholders in exchange for the cancellation of then-outstanding convertible promissory notes, at a purchase price of $0.86 per share, for an aggregate purchase price of $17,492,739. The following table sets forth the number of shares of Series D Preferred Stock purchased by holders of more than 5% of our common stock, or entities affiliated with them, or entities affiliated with certain of our directors at the initial closing of the preferred stock financing, including shares of Series D Preferred Stock issued to certain holders of more than 5% of our common stock or entities affiliated with certain of our directors in exchange for the cancellation of then-outstanding convertible promissory notes:

Name(1)	Shares of Series D Preferred Stock	Purchase price
Thomas, McNerney & Partners(2)	9,039,190	$7,791,782
CMEA Capital(3)	6,456,566	$5,565,560
Panorama Capital, L.P.	4,350,349	$3,750,001
Sloan Biotech Fund(4)	447,096	$ 385,397

(1) Additional detail regarding these stockholders and their equity holdings is provided in "Principal Stockholders."

(2) Consists of (a) 8,912,642 shares of Series D Preferred Stock issued to TMP for an aggregate purchase price of $7,682,697, (b) 93,104 shares of Series D Preferred Stock issued to TMP Nominee II, LLC for an aggregate purchase price of $80,256 and (c) 33,444 shares of Series D Preferred Stock issued to TMP Associates II, L.P. for an aggregate purchase price of $28,829.

(3) Consists of (a) 6,295,153 shares of Series D Preferred Stock issued to CMEA for an aggregate purchase price of $5,426,422 and (b) 161,413 shares of Series D Preferred Stock issued to CMEA Ventures VII (Parallel), L.P. for an aggregate purchase price of $139,138.

(4) Consists of (a) 223,548 shares of Series D Preferred Stock issued to Sloan Biotech Fund for an aggregate purchase price of $192,699 and (c) 223,548 shares of Series D Preferred Stock issued to Sloan Biotech Fund II for an aggregate purchase price of $192,698.

In May 2013, we entered into Amendment No. 1 to the Series D Purchase Agreement and held an additional closing of the Series D Preferred Stock financing, pursuant to which we issued and sold an aggregate of 2,320,188 shares of our Series D Preferred Stock at a purchase price of $0.86 per share, for an aggregate purchase price of $2,000,002. The following table sets forth the number of shares of Series D Preferred Stock purchased by holders of more than 5% of our common stock or entities affiliated with them at such closing:

Name(1)	Shares of Series D Preferred Stock	Purchase price
Thomas, McNerney & Partners(2)	719,017	$619,793
CMEA Capital(3)	513,584	$442,709
Panorama Capital, L.P.	1,087,587	$937,500

(1) Additional detail regarding these stockholders and their equity holdings is provided in "Principal Stockholders."

(2) Consists of (a) 708,950 shares of Series D Preferred Stock issued to TMP for an aggregate purchase price of $611,115, (b) 7,406 shares of Series D Preferred Stock issued to TMP Nominee II, LLC for an aggregate purchase price of $6,384 and (c) 2,661 shares of Series D Preferred Stock issued to TMP Associates II, L.P. for an aggregate purchase price of $2,294.

(3) Consists of (a) 500,744 shares of Series D Preferred Stock issued to CMEA for an aggregate purchase price of $431,641 and (b) 12,840 shares of Series D Preferred Stock issued to CMEA Ventures VII (Parallel), L.P. for an aggregate purchase price of $11,068.

In July 2013, we entered into Amendment No. 2 to the Series D Purchase Agreement and held the final closing of the Series D Preferred Stock financing pursuant to which we issued and sold an aggregate of 9,860,790 shares of our Series D Preferred Stock at a purchase price of $0.86 per share, for an aggregate purchase price of $8,500,001. The following table sets forth the number of shares of Series D Preferred Stock purchased by holders of more than 5% of our common stock or entities affiliated with them at such second mandatory closing:

Name(1)	Shares of Series D Preferred Stock	Purchase price
Thomas, McNerney & Partners(2)	2,157,047	$1,859,375
CMEA Capital(3)	1,540,748	$1,328,125
Panorama Capital, L.P.	3,262,762	$2,812,501

(1) Additional detail regarding these stockholders and their equity holdings is provided in "Principal Stockholders."

(2) Consists of (a) 2,126,849 shares of Series D Preferred Stock issued to TMP for an aggregate purchase price of $1,833,344, (b) 22,218 shares of Series D Preferred Stock issued to TMP Nominee II, LLC for an aggregate purchase price of $19,152 and (c) 7,980 shares of Series D Preferred Stock issued to TMP Associates II, L.P. for an aggregate purchase price of $6,879.

(3) Consists of (a) 1,502,230 shares of Series D Preferred Stock issued to CMEA for an aggregate purchase price of $1,294,922 and (b) 38,518 shares of Series D Preferred Stock issued to CMEA Ventures VII (Parallel), L.P. for an aggregate purchase price of $33,203.

Immediately prior to the completion of our initial public offering in February 2014, all of the outstanding shares of Series D Preferred Stock were converted to shares of common stock at a conversion ratio of one-for-4.5.

Series E Preferred Stock Financing

In December 2013, we entered into a Series E Preferred Stock Purchase Agreement, or the Series E Purchase Agreement, pursuant to which we issued and sold an aggregate of 11,336,481 shares of our Series E Preferred Stock, at a purchase price of $1.724 per share, for an aggregate purchase price of $19,544,093. The following table sets forth the number of shares of Series E Preferred Stock purchased by holders of more than 5% of our common stock or entities affiliated with them at the initial closing of the preferred stock financing:

Name(1)	Shares of Series E Preferred Stock	Purchase price
Panorama Capital, L.P.	2,266,952	$3,908,225
CMEA Capital(2)	1,397,873	$2,409,933
Thomas, McNerney & Partners(3)	1,118,298	$1,927,946

(1) Additional detail regarding these stockholders and their equity holdings is provided in "Principal Stockholders."

(2) Consists of (a) 1,362,926 shares of Series E Preferred Stock issued to CMEA Ventures VII, L.P. for an aggregate purchase price of $2,349,684 and (b) 34,947 shares of Series D Preferred Stock issued to CMEA Ventures VII (Parallel), L.P. for an aggregate purchase price of $60,249.

(3) Consists of (a) 1,102,642 shares of Series E Preferred Stock issued to Thomas, McNerney & Partners II, L.P. for an aggregate purchase price of $1,900,955, (b) 11,518 shares of Series E Preferred Stock issued to TMP Nominee II, LLC for an aggregate purchase price of $19,857 and (c) 4,138 shares of Series E Preferred Stock issued to TMP Associates II, L.P. for an aggregate purchase price of $7,134.

Immediately prior to the completion of our initial public offering in February 2014, all of the outstanding shares of Series D Preferred Stock were converted to shares of common stock at a conversion ratio of one-for-4.5.

Certain of our directors have affiliations with the investors that participated in the loan arrangements and preferred stock financings described above, as indicated in the table below:

Director	Investor
Rod Ferguson, Ph.D.	Panorama Capital, L.P.
Sepehr Sarshar, Ph.D.	Sloan Biotech Fund
Pratik Shah, Ph.D.	Thomas, McNerney & Partners
Alex Zisson	Thomas, McNerney & Partners

Investor agreements

In connection with our preferred stock financings, we entered into amended and restated investors' rights, voting and right of first refusal and co-sale agreements containing voting rights, information rights, rights of first refusal and registration rights, among other things, with certain holders of our preferred stock and certain holders of our common stock, including all of the holders of more than 5% of our capital stock. These stockholder agreements terminated upon the completion of our initial public offering in February 2014, except for the amended and restated investor rights agreement, which terminates at such time that Rule 144 or another similar exemption under the Securities Act is available for the sale of all of a holder's shares without registration in any three-month period, and contains certain registration rights as more fully described below in "Description of Capital Stock—Registration Rights."

Employment agreements

We have entered into employment arrangements with our executive officers, as more fully described in "Executive and Director Compensation—Agreements with our Named Executive Officers" and "—Potential Payments upon Termination or Change of Control."

Stock options granted to executive officers and directors

We have granted stock options to our executive officers and directors, as more fully described in the section titled "Executive and Director Compensation."

Indemnification agreements

We have entered, and intend to continue to enter, into separate indemnification agreements with each of our directors and executive officers, as described in our Annual Report on Form 10-K for the year ended December 31, 2013 incorporated by reference herein.

Policies and procedures for transactions with related persons

We have adopted a written related-person transactions policy that sets forth our policies and procedures regarding the identification, review, consideration and oversight of "related-person transactions." 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 participants involving an amount that exceeds $120,000.

Transactions involving compensation for services provided to us as an employee, consultant or director are not considered related-person transactions under this policy. A related person is any executive officer, director or a holder of more than five percent of our common stock, including any of their immediate family members and any entity owned or controlled by such persons.

Under the policy, where a transaction has been identified as a related-person transaction, management must present information regarding the proposed related-person transaction to our audit committee (or, where review by our audit committee would be inappropriate, to another independent body of our board of directors) for review. The presentation must include a description of, among other things, the material facts, the direct and indirect interests of the related persons, the benefits of the transaction to us and whether any alternative transactions are available. To identify related-person transactions in advance, we rely on information supplied by our executive officers, directors and certain significant stockholders. In considering related-person transactions, our audit committee or other independent body of our board of directors takes into account the relevant available facts and circumstances including, but not limited to:

- the risks, costs and benefits to us;

- the impact on a director's independence in the event the related person is a director, immediate family member of a director or an entity with which a director is affiliated;
- the terms of the transaction;
- 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 our employees generally.

In the event a director has an interest in the proposed transaction, the director must recuse himself or herself from the deliberations and approval.

Principal and selling stockholders

The following table sets forth information regarding beneficial ownership of our capital stock by:

- each person, or group of affiliated persons, known by us to beneficially own more than 5% of our common stock;
- each of our directors;
- each of our named executive officers;
- all of our current executive officers and directors as a group; and
- each of the selling stockholders.

The percentage ownership information under the column entitled “Before offering” is based on 27,664,035 shares of common stock outstanding as of November 30, 2014. The percentage ownership information under the column entitled “After Offering” is based on the sale by us of 3,000,000 shares of common stock in this offering.

Information with respect to beneficial ownership has been furnished by each director, officer or beneficial owner of more than 5% of our common stock. We have determined beneficial ownership in accordance with the rules of the SEC. These rules generally attribute beneficial ownership of securities to persons who possess sole or shared voting power or investment power with respect to those securities. In addition, the rules include shares of common stock issuable pursuant to the exercise of stock options or warrants that are either immediately exercisable or exercisable on or before January 29, 2015, which is 60 days after November 30, 2014. These shares are deemed to be outstanding and beneficially owned by the person holding those options or warrants for the purpose of computing the percentage ownership of that person, but they are not treated as outstanding for the purpose of computing the percentage ownership of any other person. Unless otherwise indicated, the persons or entities identified in this table have sole voting and investment power with respect to all shares shown as beneficially owned by them, subject to applicable community property laws. Unless otherwise indicated, based on the information supplied to us by or on behalf of the selling stockholders, no selling stockholder is a broker-dealer or an affiliate of a broker-dealer.

The information in the table below with respect to each selling stockholder has been obtained from that selling stockholder. When we refer to the “selling stockholders” in this prospectus, we mean those persons listed in the table below as offering shares, as well as the pledgees, donees, assignees, transferees, successors and others who may hold any of the selling stockholders’ interest.

Except as otherwise noted below, the address for each person or entity listed in the table is c/o Auspex Pharmaceuticals, Inc., 3333 N. Torrey Pines Court, Suite 400, San Diego, California 92037.

Name and address of beneficial owner	Shares beneficially owned prior to the offering		Shares being offered	Shares beneficially owned after the offering	
	Number	Percent		Number	Percent
5% or Greater and Selling Stockholders					
Thomas, McNerney & Partners II, L.P. and affiliates(1) 3366 N. Torrey Pines Court, Suite 220 San Diego, California 92037	5,589,739	20.0%	730,000	4,859,739	15.7%
CMEA Ventures VII, L.P. and affiliates(2) One Letterman Drive Building C, Suite CM500 San Francisco, California 94129	4,030,568	14.5%	–	4,030,568	13.0%
Panorama Capital, L.P.(3) 1999 South Bascom Avenue, Suite 700 Campbell, California 95008	2,770,589	10.0%	270,000	2,500,589	8.2%
FMR LLC(4) 245 Summer Street Boston, MA 02210	2,459,771	8.9%	–	2,459,771	8.0%
Deerfield Management Company, L.P.(5) 780 Third Avenue, 37th Floor New York, NY 10017	1,988,990	7.2%	–	1,988,990	6.5%
Directors and Named Executive Officers					
Pratik Shah, Ph.D.(6)	1,118,171	4.0%	–	1,118,171	3.6%
Bharatt Chowrira, Ph.D., J.D.(7)	305,008	1.1%	–	305,008	*
John Schmid(8)	203,470	*	–	203,470	*
Lynn Dorsey Bleil	–	*	–	–	*
Rod Ferguson, Ph.D.(9)	2,770,589	10.0%	270,000	2,500,589	8.2%
R. Scott Greer(10)	7,190	*	–	7,190	*
Gerald Proehl(11)	14,799	*	–	14,799	*
Sepehr Sarshar, Ph.D.(12)	242,574	*	–	242,574	*
Samuel Saks, M.D.(13)	109,501	*	–	109,501	*
Phillip M. Schneider(14)	13,333	*	–	13,333	*
Alex Zisson(15)	5,589,739	20.0%	730,000	4,859,739	15.7%
All current executive officers and directors as a group (12 persons)(16)	10,481,309	36.5%	1,000,000	9,481,309	29.9%

* Represents beneficial ownership of less than 1%.

(1) Includes (a) 5,196,820 shares of common stock and 314,670 shares of common stock issuable upon the exercise of outstanding warrants held by TMP, (b) 54,286 shares of common stock and 3,286 shares of common stock issuable upon the exercise of outstanding warrants held by TMP Nominee II, LLC, or TMPN II, and (c) 19,498 shares of common stock and 1,179 shares of common stock issuable upon the exercise of outstanding warrants held by TMP Associates II, L.P., or TMPA II. Thomas, McNerney & Partners II, LLC, or TMP II LLC, the general partner of TMP and TMPA II, has voting and dispositive power over the shares held by TMP and TMPA II. In addition, TMPN II has entered into an agreement with TMP II LLC that directs TMPN II to vote and dispose of securities held by TMPN II in the same manner as directed by TMP II LLC with respect to TMP and TMPA II.

(2) Includes (a) 3,707,499 shares of common stock and 222,256 shares of common stock issuable upon the exercise of warrants held by CMEA, and (b) 95,115 shares of common stock and 5,698 shares of common stock issuable upon the exercise of outstanding warrants held by CMEA Ventures VII (Parallel), L.P., or CMEA (Parallel).

(3) Includes 2,770,589 shares of common stock held by Panorama. Dr. Ferguson, Chris Albinson and Shahan Soghikian, as Managing Directors of Panorama Capital, LLC, share voting and investment authority over the shares held by Panorama.

(4) Based solely upon a schedule 13G filed with the SEC on May 12, 2014 by FMR LLC, or Fidelity, reporting beneficial ownership as of May 9, 2014. According to the schedule 13G, (a) 1,478,075 shares of common stock are held by Fidelity Management & Research Company, or FMRC, (b) 959,896 shares of common stock are held by Fidelity SelectCo, LLC, or Fidelity SelectCo, and (c) 21,800 shares of common stock are held by Fidelity Management Trust Company, or FMTC. FMRC, Fidelity SelectCo and FMTC are wholly-owned subsidiaries of Fidelity, of which Edward C. Johnson III is Chairman. Fidelity and Mr. Johnson each have sole dispositive power, but not voting power, over the shares held by FMRC and Fidelity SelectCo. Fidelity and Mr. Johnson each have sole dispositive power and sole power to vote or direct the voting of the shares held by FMTC. Each of Fidelity and Mr. Johnson may be deemed to beneficially own the shares held by FMRC, Fidelity SelectCo and FMTC.

(5) Based solely upon a schedule 13G filed with the SEC on February 12, 2014 by James Flynn on behalf of himself, Deerfield Management Company, L.P. and its affiliated entities, reporting beneficial ownership as of February 10, 2014. Includes (a) 273,514 shares of common stock held by Deerfield Special Situations Fund, L.P., (b) 223,733 shares of common stock held by Deerfield Special Situations International Master Fund, L.P., (c) 695,152 shares of common stock held by Deerfield Private Design Fund II, L.P. and (d) 796,591 shares of common stock held by Deerfield Private Design International II, L.P. Deerfield Management Company, L.P. is the investment manager of each of Deerfield Special Situations

Fund, L.P., Deerfield Special Situations International Master Fund, L.P., Deerfield Private Design Fund II, L.P. and Deerfield Private Design International II, L.P. (collectively, the "Deerfield Funds"). Deerfield Mgmt, L.P. is the general partner of each of the Deerfield Funds. Mr. James E. Flynn is the sole member of the general partner of each of Deerfield Mgmt, L.P. and Deerfield Management Company, L.P. Each of Deerfield Mgmt, L.P., Deerfield Management Company, L.P. and Mr. Flynn may be deemed to beneficially own the shares held by the Deerfield Funds.

(6) Includes (a) 894,685 shares of restricted common stock owned by Dr. Shah, 447,343 shares of which are subject to a right of repurchase by us as of January 29, 2015, and (b) 215,153 shares of common stock that Dr. Shah has the right to acquire from us within 60 days of November 30, 2014 pursuant to the exercise of stock options, 161,365 of which will be unvested but exercisable as of January 29, 2015.

(7) Includes (a) 7,231 shares of common stock and (b) 297,777 shares of common stock that Dr. Chowrira has the right to acquire from us within 60 days of November 30, 2014 pursuant to the exercise of stock options, 204,722 of which will be unvested but exercisable as of January 29, 2015.

(8) Includes (a) 144,444 shares of restricted common stock owned by Mr. Schmid, 99,306 shares of which are subject to a right of repurchase by us as of January 29, 2015, and (b) 59,026 shares of common stock that Mr. Schmid has the right to acquire from us within 60 days of November 30, 2014 pursuant to the exercise of stock options, 44,270 of which will be unvested but exercisable as of January 29, 2015.

(9) Includes the shares of capital stock referred to in footnote (3) above. Dr. Ferguson is a managing director at Panorama and shares the voting and investment control over the shares owned by Panorama; however, Dr. Ferguson disclaims beneficial ownership of such shares, except to the extent of his pecuniary interests therein.

(10) Includes 7,190 shares of common stock held by Mr. Greer.

(11) Includes (a) 1,466 shares of common stock held by Mr. Proehl and (b) 13,333 shares of common stock that Mr. Proehl has the right to acquire from us within 60 days of November 30, 2014 pursuant to the exercise of stock options.

(12) Includes 15,277 shares of common stock held by Dr. Sarshar that are subject to a right of repurchase by us as of January 29, 2015.

(13) Includes (a) 4,258 shares of common stock held by Dr. Saks, (b) 61,664 shares of restricted common stock owned by Dr. Saks, 42,394 of which are subject to a right of repurchase from us as of January 29, 2015, and (c) 43,579 shares of common stock that Dr. Saks has the right to acquire from us within 60 days of November 30, 2014 pursuant to the exercise of stock options.

(14) Includes 13,333 shares of common stock that Mr. Schneider has the right to acquire from us within 60 days of November 30, 2014 pursuant to the exercise of stock options.

(15) Includes the shares of capital stock referred to in footnote (1) above. TMP II LLC is the general partner of TMP and TMPA II and has entered into an agreement with TMPN II that directs TMPN II to vote and dispose of securities held by TMPN II in the same manner as directed by TMP II LLC with respect to TMP and TMPA II. Mr. Zisson is a manager of TMP II LLC and thus shares voting and investment control, directly or indirectly, over the shares owned by TMP, TMPA II and TMPN II; however, Mr. Zisson disclaims beneficial ownership of such shares, except to the extent of his pecuniary interests therein.

(16) Includes (a) 9,414,021 shares of common stock beneficially owned by all current executive officers and directors as a group (including 983 shares of common stock owned by Dr. David Stamler, our Chief Medical Officer), (b) 1,100,793 shares of restricted common stock beneficially owned by all current executive officers and directors as a group, of which 589,043 shares are subject to a right of repurchase by us as of January 29, 2015, and (c) 748,153 shares that all current executive officers and directors as a group have the right to acquire from us within 60 days of November 30, 2014 pursuant to the exercise of stock options (including 105,952 shares of common stock that Dr. Stamler has a right to acquire from us within 60 days of November 30, 2014 pursuant to the exercise of stock options, 18,027 shares of which will be unvested but exercisable as of January 29, 2015), 428,384 of which will be unvested but exercisable as of January 29, 2015 (including the 18,027 shares that Dr. Stamler has a right to acquire pursuant to the exercise of stock options).

Description of capital stock

Our amended and restated certificate of incorporation authorizes us to issue up to 200,000,000 shares of common stock, par value $0.0001 per share, and 10,000,000 shares of preferred stock, par value $0.0001 per share.

As of September 30, 2014, there were:

- 27,482,166 shares of common stock outstanding;
- 1,960,460 shares of common stock subject to outstanding options; and
- 708,207 shares of common stock subject to outstanding warrants.

As of November 30, 2014, we had 45 holders of record of our common stock. The actual number of stockholders is greater than this number of record holders, and includes stockholders who are beneficial owners, but whose shares are held in street name by brokers and other nominees. This number of holders of record also does not include stockholders whose shares may be held in trust by other entities.

The following description of our capital stock is not complete and is subject to and qualified in its entirety by our amended and restated certificate of incorporation and amended and restated bylaws, which are filed as exhibits to the registration statement of which this prospectus is a part, and by the relevant provisions of the Delaware General Corporation Law.

Common stock

Voting

Our common stock is entitled to one vote for each share held of record on all matters submitted to a vote of the stockholders, including the election of directors, and does not have cumulative voting rights. Accordingly, the holders of a majority of the shares of our common stock entitled to vote in any election of directors can elect all of the directors standing for election.

Dividends

Subject to preferences that may be applicable to any then-outstanding preferred stock, the holders of common stock are entitled to receive dividends, if any, as may be declared from time to time by our board of directors out of legally available funds.

Liquidation

In the event of our liquidation, dissolution or winding-up, holders of our common stock will be entitled to share ratably in the net assets legally available for distribution to stockholders after the payment of all of our debts and other liabilities, subject to the satisfaction of any liquidation preference granted to the holders of any outstanding shares of preferred stock.

Rights and preferences

Holders of our common stock have no preemptive, conversion or subscription rights, and there are no redemption or sinking fund provisions applicable to our common stock. The rights, preferences and privileges of the holders of our common stock are subject to, and may be adversely affected by, the rights of the holders of shares of any series of our preferred stock that we may designate and issue in the future.

Fully paid and nonassessable

All of our outstanding shares of common stock are, and the shares of common stock to be issued in this offering will be, fully paid and nonassessable.

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, to establish from time to time the number of shares to be included in each such series, to fix the rights, preferences and privileges of the shares of each wholly unissued series and any qualifications, limitations or restrictions thereon and to increase or decrease the number of shares of any such series, but not below the number of shares of such series then outstanding.

Our board of directors may authorize the issuance of preferred stock with voting or conversion rights that could adversely affect the voting power or other rights of the holders of the common stock. The issuance of preferred stock, while providing flexibility in connection with possible acquisitions and other corporate purposes, could, among other things, have the effect of delaying, deferring or preventing a change in our control that may otherwise benefit holders of our common stock and may adversely affect the market price of the common stock and the voting and other rights of the holders of common stock. As of September 30, 2014, there are no shares of preferred stock outstanding and we have no current plans to issue any shares of preferred stock.

Warrants

As of September 30, 2014, there were outstanding warrants to purchase 708,207 shares of common stock at a weighted-average exercise price of $4.20 per share.

Warrants to purchase up to 317,601 shares of common stock were issued in connection with an equity financing agreement with certain investors for the sale of Series B Preferred Stock and, when issued, were exercisable for shares of Series B Preferred Stock. These warrants automatically became exercisable for shares of Series C Preferred Stock upon the closing of the Series C Preferred Stock financing in January 2010. The warrants to purchase Series C Preferred Stock were subsequently converted to warrants to purchase common stock upon the completion of our initial public offering in February 2014. The warrants are exercisable until the earlier of five years after the issuance date of each respective warrant or upon certain reorganizations or changes in control as set forth in the warrant. These warrants provide for cashless exercise at the option of the holder, and also contain provisions for the adjustment of the exercise price and the number of shares issuable upon the exercise of the warrants in the event of stock dividends, stock splits, reorganizations and reclassifications and consolidations.

Warrants to purchase up to 445,472 shares of common stock were issued in connection with the Series C Preferred Stock financing and, when issued, were exercisable for shares of Series C Preferred Stock. These warrants automatically became exercisable for shares of Series D Preferred Stock upon the closing of the Series D Preferred Stock financing. The warrants to purchase Series D Preferred Stock were subsequently converted to warrants to purchase common stock upon the completion of our initial public offering in February 2014. Such warrants are exercisable until the earlier of five years after the issuance date of each respective warrant or upon certain reorganizations or changes in control as set forth in the warrant. These warrants provide for cashless exercise at the option of the holder, and also contain provisions for the adjustment of the exercise price and the number of shares issuable upon the exercise of the warrants in the event of stock dividends, stock splits, reorganizations and reclassifications and consolidations.

Warrants to purchase an aggregate of 58,001 shares of common stock, or the Oxford Warrants, were issued to affiliates of Oxford Finance LLC as warrants to purchase Series E Preferred Stock in connection with the

execution of the credit facility with Oxford in December 2013. The Oxford Warrants were subsequently converted to warrants to purchase common stock upon the completion of our initial public offering in February 2014. The Oxford Warrants are exercisable for 10 years from the issuance date. The Oxford Warrants provide for cashless exercise at the option of the warrantholder, and also contains provisions for the adjustment of the exercise price and the number of shares issuable upon the exercise of the Oxford Warrants in the event of stock dividends, stock splits, reorganizations and reclassifications and consolidations.

For more information, see "Certain Relationships and Related Party Transactions—Loan Arrangements."

Registration rights

Under our amended and restated investor rights agreement entered into in connection with the Series E Financing, the holders of common stock that was issued upon conversion of all of our preferred stock in connection with the completion of our initial public offering in February 2014, or their transferees, have the right to require us to register their shares with the SEC so that those shares may be publicly resold, or to include their shares in any registration statement we file. Such shares of common stock are hereinafter referred to as the "Registrable Securities."

Demand registration rights. At any time, the holders of a majority of the Registrable Securities having registration rights have the right to demand that we file a registration statement under the Securities Act to register the Registrable Securities requested to be registered by the holders of Registrable Securities. These registration rights are subject to specified conditions and limitations, including a limitation on the number of such registration statements that can be demanded by the holders of Registrable Securities, restrictions on the exercise of such demand registration rights during periods of time that may be detrimental to the Company and its stockholders, and the right of the underwriters to limit the number of shares of Registrable Securities included in any such registration under certain circumstances.

Form S-3 registration rights. If we are eligible to file a registration statement on Form S-3, each holder of shares of Registrable Securities having registration rights has the right to demand that we file no more than one registration statement for the holders on Form S-3 in any 12-month period so long as the aggregate offering price of securities to be sold under the registration statement on Form S-3 is at least $1,000,000, subject to specified exceptions, conditions and limitations.

"Piggyback" registration rights. If we register any securities for public sale, stockholders with registration rights will have the right to include their Registrable Securities in the registration statement, provided that the underwriters of any such underwritten offering will have the right to limit the number of Registrable Securities rights to be included in the registration statement.

Expenses of registration. We will pay all expenses, including for the reasonable fees and costs of one counsel to the holders of Registrable Securities, relating to all demand registrations, Form S-3 registrations and piggyback registrations.

Expiration of registration rights. The registration rights described above will terminate, as to a given holder of Registrable Securities, at any time when such holder can sell all of such holder's Registrable Securities pursuant to Rule 144 promulgated under the Securities Act during any three-month period.

Anti-takeover effects of provisions of our amended and restated certificate of incorporation, our bylaws and Delaware law

Delaware anti-takeover law

We are subject to Section 203 of the Delaware General Corporation Law, or Section 203. Section 203 generally prohibits a public Delaware corporation from engaging in a "business combination" with an "interested stockholder" for a period of three years following the time that such stockholder became an interested stockholder, unless:

- prior to such time the board of directors of the corporation approved either the business combination or the transaction which resulted in the stockholder becoming an interested stockholder;
- upon consummation of the transaction which resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction commenced, excluding for purposes of determining the voting stock outstanding (but not the outstanding voting stock owned by the interested stockholder) those shares owned (1) by persons who are directors and also officers and (2) employee stock plans in which employee participants do not have the right to determine confidentially whether shares held subject to the plan will be tendered in a tender or exchange offer; or
- at or subsequent to such time, the business combination is approved by the board of directors and authorized at an annual or special meeting of stockholders, and not by written consent, by the affirmative vote of at least 66⅔% of the outstanding voting stock which is not owned by the interested stockholder;

Section 203 defines a business combination to include:

- any merger or consolidation involving the corporation and the interested stockholder;
- any sale, transfer, pledge or other disposition involving the interested stockholder of 10% or more of the assets of the corporation;
- subject to exceptions, any transaction that results in the issuance or transfer by the corporation of any stock of the corporation to the interested stockholder;
- subject to exceptions, any transaction involving the corporation that has the effect of increasing the proportionate share of the stock of any class or series of the corporation beneficially owned by the interested stockholder; and
- the receipt by the interested stockholder of the benefit of any loans, advances, guarantees, pledges or other financial benefits provided by or through the corporation.

In general, Section 203 defines an interested stockholder as any entity or person beneficially owning 15% or more of the outstanding voting stock of the corporation and any entity or person affiliated with or controlling or controlled by the entity or person.

Amended and restated certificate of incorporation and amended and restated bylaws

Provisions of our amended and restated certificate of incorporation and amended and restated bylaws may delay or discourage transactions involving an actual or potential change in our control or change in our management, including transactions in which stockholders might otherwise receive a premium for their shares or transactions that our stockholders might otherwise deem to be in their best interests. Therefore, these provisions could adversely affect the price of our common stock. Among other things, our amended and restated certificate of incorporation and amended and restated bylaws:

- permit our board of directors to issue up to 10,000,000 shares of preferred stock, with any rights, preferences and privileges as they may designate (including the right to approve an acquisition or other change in our control);
- provide that the authorized number of directors may be changed only by resolution of the board of directors;
- provide that the board of directors or any individual director may only be removed with cause and the affirmative vote of the holders of at least 66 ⅔% of the voting power of all of our then outstanding common stock;
- provide that all vacancies, including newly created directorships, may, except as otherwise required by law, be filled by the affirmative vote of a majority of directors then in office, even if less than a quorum;
- require that any action to be taken by our stockholders must be effected at a duly called annual or special meeting of stockholders and not be taken by written consent;
- provide that stockholders seeking to present proposals before a meeting of stockholders or to nominate candidates for election as directors at a meeting of stockholders must provide notice in writing in a timely manner and also specify requirements as to the form and content of a stockholder's notice;
- do not provide for cumulative voting rights (therefore allowing the holders of a majority of the shares of common stock entitled to vote in any election of directors to elect all of the directors standing for election, if they should so choose); and
- provide that special meetings of our stockholders may be called only by the chairman of the board, our Chief Executive Officer or by the board of directors pursuant to a resolution adopted by a majority of the total number of authorized directors.

The amendment of any of these provisions, with the exception of the ability of our board of directors to issue shares of preferred stock and designate any rights, preferences and privileges thereto, would require approval by the holders of at least 66⅔% of our then-outstanding common stock.

NASDAQ Global Market Listing

Our common stock is listed on The NASDAQ Global Market under the symbol "ASPX."

Transfer agent and registrar

The transfer agent and registrar for our common stock is American Stock Transfer & Trust Company, LLC. The transfer agent and registrar's address is 6201 15th Avenue, Brooklyn, NY 11219.

Shares eligible for future sale

Prior to our initial public offering in February 2014, there was 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, including shares issued upon exercise of outstanding options and warrants, or the anticipation of these sales, could adversely affect prevailing market prices from time to time and could impair our ability to raise equity capital in the future.

Based on the number of shares of common stock outstanding as of September 30, 2014, upon the completion of this offering we will have 30,482,166 shares of common stock outstanding, assuming (1) no exercise of the underwriters' option to purchase additional shares of common stock and (2) no exercise of outstanding options or warrants. Of those shares, all of the shares sold in this offering and all 11,672,500 shares sold in our initial public offering and July 2014 follow-on offering will be freely tradable, except that any shares held by our "affiliates," as that term is defined in Rule 144 under the Securities Act, or Rule 144, may only be sold in compliance with the limitations described below.

Rule 144

In general, under Rule 144, any person who is not our affiliate and has held their shares for at least six months, including the holding period of any prior owner other than one of our affiliates, may sell shares without restriction, subject to the availability of current public information about us. In addition, under Rule 144, any person who is not an affiliate of ours and has held their shares for at least one year, including the holding period of any prior owner other than one of our affiliates, would be entitled to sell an unlimited number of shares without regard to whether current public information about us is available. A person who is our affiliate or who was our affiliate at any time during the preceding three months, and who has beneficially owned restricted securities for at least six months, including the holding period of any prior owner other than one of our affiliates, is entitled to sell a number of shares within any three-month period that does not exceed the greater of:

- 1% of the number of shares of our common stock then outstanding, which will equal approximately 304,822 shares immediately after this offering; or
- the average weekly trading volume of our common stock on The NASDAQ Global Market during the four calendar weeks preceding the filing of a notice on Form 144 with respect to the sale.

Sales under Rule 144 by our affiliates are also subject to manner of sale provisions and notice requirements, and to the availability of current public information about us.

Rule 701

In general, under Rule 701 of the Securities Act, any of our stockholders who purchased shares from us in connection with a qualified compensatory stock plan or other written agreement before we became subject to the reporting requirements of Section 13 or 15(d) of the Exchange Act is eligible to resell those shares in reliance on Rule 144. An affiliate of the issuer can resell shares in reliance on Rule 144 without having to comply with the holding period requirements of Rule 144, and a non-affiliate of the issuer can resell shares in reliance on Rule 144 without having to comply with the holding period requirements of Rule 144 and without regard to the volume of such sales or the availability of public information about the issuer.

As of September 30, 2014, options to purchase a total of 1,960,460 shares of common stock were outstanding, of which 211,543 were vested. There were also 22,222 shares of common stock outstanding pursuant to stock

options that were early exercised that are subject to repurchase by the Company. In addition, there were 1,100,925 shares of restricted common stock issued since inception, of which 691,898 are subject to repurchase by the Company as of September 30, 2014. Of the total number of shares of our common stock issuable under the options referenced above, substantially all are eligible for sale unless held by an affiliate of ours.

Lock-up agreements

We, along with our directors, executive officers, selling stockholders and stockholders with which our directors are affiliated, have agreed with the underwriters that for a period of 60 days after the date of this prospectus, except with the prior written consent of J.P. Morgan Securities LLC and subject to specified exceptions, we or they will not offer, pledge, sell, 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 shares of common stock, or enter into any swap or other arrangement that transfers to another, in whole or in part, any of the economic consequences of ownership of the common stock. Upon expiration of the "lock-up" period, certain of our stockholders will have the right to require us to register their shares under the Securities Act. See "—Registration Rights" below and "Description of Capital Stock—Registration Rights."

Certain of our employees, including our executive officers and/or directors, may from time to time enter into written trading plans that are intended to comply with Rule 10b5-1 under the Exchange Act. Except in limited circumstances, sales under these trading plans are not permitted until the expiration of the lock-up agreements relating to the offering described above.

Registration rights

The holders of a substantial number of shares of our common stock are entitled to rights with respect to the registration of their shares under the Securities Act, subject to the lock-up agreement described above. Registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act, except for shares purchased by affiliates. Any sales of securities by these stockholders could have a material adverse effect on the trading price of our common stock. See "Description of Capital Stock—Registration Rights."

Equity incentive plans

Shares of our common stock issued under the 2014 Plan, our 2010 equity incentive plan and the ESPP are available for sale in the open market, subject to Rule 144 volume limitations and the lock-up agreements described above, if applicable.

Material U.S. federal income tax consequences to Non-U.S. Holders of our common stock

The following summary describes the material U.S. federal income consequences of the acquisition, ownership and disposition of our common stock acquired in this offering by Non-U.S. Holders (as defined below). This discussion does not address all aspects of U.S. federal income taxes that may be relevant to Non-U.S. Holders in light of their particular circumstances, does not deal with foreign, state and local tax consequences and does not address U.S. federal tax consequences other than income taxes, including the effects of any applicable gift or estate tax or the Medicare contribution tax. Special rules different from those described below may apply to certain Non-U.S. Holders that are subject to special treatment under the Internal Revenue Code of 1986, as amended, or the Code, such as financial institutions, insurance companies, tax-exempt organizations, tax-qualified retirement plans, broker-dealers and traders in securities, commodities or currencies, U.S. expatriates, "controlled foreign corporations," "passive foreign investment companies," corporations that accumulate earnings to avoid U.S. federal income tax, persons that hold our common stock as part of a "straddle," "hedge," "conversion transaction," "synthetic security," integrated investment or other risk reduction strategy, holders deemed to sell our common stock under the constructive sale provisions of the Code, holders who hold or receive our common stock pursuant to the exercise of employee stock options or otherwise as compensation, holders who are subject to the alternative minimum tax or the Medicare contribution tax, partnerships and other pass-through entities, including hybrid entities and partners and investors in such entities or an entity that is treated as a disregarded entity for U.S. federal income tax purposes (regardless of its place of organization or formation). Such Non-U.S. Holders are urged to consult their own tax advisors to determine the U.S. federal, state, local and other tax consequences that may be relevant to them. Furthermore, the discussion below is based upon the provisions of the Code, and Treasury regulations, rulings and judicial decisions thereunder as of the date hereof, and such authorities may be repealed, revoked or modified, perhaps retroactively, so as to result in U.S. federal income tax consequences different from those discussed below. We have not requested a ruling from the U.S. Internal Revenue Service, or the IRS, with respect to the statements made and the conclusions reached in the following summary, and there can be no assurance that the IRS will agree with such statements and conclusions. This discussion assumes that the Non-U.S. Holder holds our common stock as a "capital asset" within the meaning of Section 1221 of the Code (generally, property held for investment).

The following discussion is for general information only and is not tax advice. Persons considering the purchase of our common stock pursuant to this offering should consult their own tax advisors concerning the U.S. federal income tax consequences of acquiring, owning and disposing of our common stock in light of their particular situations as well as any consequences arising under the laws of any other taxing jurisdiction, including any state, local or foreign tax consequences and any U.S. federal non-income tax consequences.

For the purposes of this discussion, a "Non-U.S. Holder" is, for U.S. federal income tax purposes, a beneficial owner of common stock that has not been excluded from this discussion and is not a U.S. Holder. A "U.S. Holder" means a beneficial owner of our common stock that is for U.S. federal income tax purposes (a) an individual who is a citizen or resident of the United States, (b) a corporation or other entity treated as a corporation created or organized in or under the laws of the United States, any state thereof or the District of Columbia, (c) an estate the income of which is subject to U.S. federal income taxation regardless of its source or (d) a trust if it (1) is subject to the primary supervision of a court within the United States and one or more U.S. persons have the authority to control all substantial decisions of the trust or (2) has a valid election in effect under applicable U.S. Treasury regulations to be treated as a U.S. person.

Distributions on our common stock

Subject to the discussion below regarding back-up withholding and foreign accounts, distributions, if any, made on our common stock to a Non-U.S. Holder of our common stock to the extent made out of our current or accumulated earnings and profits (as determined under U.S. federal income tax principles) generally will constitute dividends for U.S. tax purposes and will be subject to withholding tax at a 30% rate or such lower rate as may be specified by an applicable income tax treaty. To obtain a reduced rate of withholding under a treaty, a Non-U.S. Holder generally will be required to provide us or our paying agent with a properly executed IRS Form W-8BEN or Form W-8BEN-E, or other appropriate form, certifying the Non-U.S. Holder's entitlement to benefits under that treaty. If a Non-U.S. Holder holds stock through a financial institution or other agent acting on the holder's behalf, the holder will be required to provide appropriate documentation to such agent. The holder's agent will then be required to provide certification to the applicable withholding agent, either directly or through other intermediaries. These certifications must be provided to us or our paying agent prior to the payment of dividends and must be updated periodically. If you do not timely provide us or our paying agent the required certification but are eligible for a reduced rate of U.S. federal withholding tax under an income tax treaty, you should consult with your own tax advisor to determine if you are able to obtain a refund or credit of any excess amounts withheld by timely filing an appropriate claim for a refund with the IRS.

We generally are not required to withhold tax on dividends paid to a Non-U.S. Holder that are effectively connected with the Non-U.S. Holder's conduct of a trade or business within the United States (and, if required by an applicable income tax treaty, are attributable to a permanent establishment that such holder maintains in the United States) if a properly executed IRS Form W-8ECI, stating that the dividends are so connected, is furnished to us or our paying agent (or, if stock is held through a financial institution or other agent, to such agent). In general, such effectively connected dividends will be subject to U.S. federal income tax, on a net income basis at the regular graduated U.S. federal income tax rates, unless a specific treaty exemption applies. A corporate Non-U.S. Holder receiving effectively connected dividends may also be subject to an additional "branch profits tax," which is imposed, under certain circumstances, at a rate of 30% (or such lower rate as may be specified by an applicable treaty) on the corporate Non-U.S. Holder's effectively connected earnings and profits, subject to certain adjustments.

To the extent distributions on our common stock, if any, exceed our current and accumulated earnings and profits, they will constitute a non-taxable return of capital and will first reduce your basis in our common stock, but not below zero, and then will be treated as capital gain and taxed in the same manner as gain realized from a sale or other disposition of common stock as described in the next section.

Gain on disposition of our common stock

Subject to the discussion below regarding backup withholding and foreign accounts, a Non-U.S. Holder generally will not be subject to U.S. federal income tax with respect to gain realized on a sale or other disposition of our common stock unless (a) the gain is effectively connected with a trade or business of such holder in the United States (and, if required by an applicable income tax treaty, is attributable to a permanent establishment that such holder maintains in the United States), (b) the Non-U.S. Holder is a non-resident alien individual and is present in the United States for 183 or more days in the taxable year of the disposition and certain other conditions are met, or (c) we are or have been a "United States real property holding corporation" within the meaning of Code Section 897(c)(2) at any time within the shorter of the five-year period preceding such disposition or such holder's holding period.

If you are a Non-U.S. Holder described in (a) above, you will be required to pay tax on the net gain derived from the sale at regular graduated U.S. federal income tax rates, unless a specific treaty exemption applies, and corporate Non-U.S. Holders described in (a) above may be subject to the additional 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 (b) above, you will be required to pay a flat 30% tax on the gain derived from the sale, which gain may be offset by U.S. source capital losses (even though you are not considered a resident of the United States). For purposes of (c) above, in general, we would be a United States real property holding corporation if the fair market value of our U.S. real property interests was equal to or exceeded 50% of the sum of the fair market value of our worldwide real property interests plus other assets used or held for use by us in a trade or business. We believe that we are not, and do not anticipate becoming, a United States real property holding corporation. However, because the determination of whether we are a United States real property holding corporation depends on the fair market value of our U.S. real property interests relative to the fair market value of our other business assets, there can be no assurance that we will not become a United States real property holding corporation in the future. Even if we are treated as a United States real property holding corporation, gain realized by a Non-U.S. Holder on a disposition of our common stock will not be subject to U.S. federal income tax so long as (1) the Non-U.S. Holder owned, directly, indirectly and constructively, no more than 5% of our common stock at all times within the shorter of (i) the five-year period preceding the disposition or (ii) the holder's holding period and (2) our common stock is "regularly traded," as defined by applicable Treasury regulations, on an established securities market. We expect our common stock to continue to be "regularly traded" on an established securities market, but there can be no assurance that our common stock will continue to be so traded. If gain on the sale or other taxable disposition of our common stock were subject to taxation under (c) above, the Non-U.S. Holder would be subject to regular U.S. federal income tax with respect to such gain in generally the same manner as a U.S. person.

Information reporting requirements and backup withholding

Generally, we or certain financial middlemen must report information to the IRS with respect to any dividends we pay on our common stock including the amount of any such dividends, the name and address of the recipient, and the amount, if any, of tax withheld. A similar report is sent to the holder to whom any such dividends are paid. Pursuant to tax treaties or certain other agreements, the IRS may make its reports available to tax authorities in the recipient's country of residence.

Dividends paid by us (or our paying agents) to a Non-U.S. Holder may also be subject to U.S. backup withholding. U.S. backup withholding generally will not apply to a Non-U.S. Holder who provides a properly executed IRS Form W-8BEN or Form W-8BEN-E or IRS Form W-8ECI or otherwise establishes an exemption, *provided* we do not have actual knowledge or reason to know such non-U.S. holder is a U.S. person, as defined in the Code. The current backup withholding rate is 28%.

Under current U.S. federal income tax law, U.S. information reporting and backup withholding requirements generally will apply to the proceeds from a disposition of our common stock effected by or through a U.S. office of any broker, U.S. or foreign, except that information reporting and such requirements may be avoided if the holder provides a properly executed IRS Form W-8BEN or Form W-8BEN-E or otherwise meets documentary evidence requirements for establishing Non-U.S. Holder status or otherwise establishes an exemption. Generally, U.S. information reporting and backup withholding requirements will not apply to a payment of disposition proceeds to a Non-U.S. Holder where the transaction is effected outside the United States through a non-U.S. office of a non-U.S. broker. For information reporting purposes, certain brokers with substantial U.S. ownership or operations will generally be treated in a manner similar to U.S. brokers. Information reporting and backup withholding requirements may apply to a payment of disposition proceeds if the broker has actual knowledge, or reason to know, that the holder is a U.S. person, as defined in the Code.

If backup withholding is applied to you, you should consult with your own tax advisor to determine if you are able to obtain a tax benefit or credit with respect to such backup withholding.

Foreign accounts

A U.S. federal withholding tax of 30% may apply to dividends paid after June 30, 2014 and the gross proceeds from a disposition of our common stock paid after December 31, 2016 to a foreign financial institution (as specifically defined for this purpose), including when the foreign financial institution holds our common stock on behalf of a non-U.S. Holder, 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 U.S. account holders of such institution (which may include certain equity and debt holders of such institution, as well as certain account holders that are foreign entities with U.S. owners). Foreign financial institutions located in jurisdictions that have an intergovernmental agreement with the United States governing these withholding and reporting requirements may be subject to different rules. This U.S. federal withholding tax of 30% will also apply to dividends paid after June 30, 2014 and the gross proceeds from a disposition of our common stock paid after December 31, 2016 to a non-financial foreign entity (as specifically defined for this purpose) unless such entity provides the withholding agent with either a certification that it does not have any substantial direct or indirect U.S. owners or provides information regarding direct and indirect U.S. owners of the entity. The withholding tax described above will not apply if the foreign financial institution or non-financial foreign entity otherwise qualifies for an exemption from the rules. Under certain circumstances, a Non-U.S. Holder might be eligible for refunds or credits of such taxes. Holders are encouraged to consult with their own tax advisors regarding the possible implications of the legislation on their investment in our common stock.

THE PRECEDING DISCUSSION OF U.S. FEDERAL INCOME TAX CONSIDERATIONS IS FOR GENERAL INFORMATION ONLY. IT IS NOT TAX ADVICE. EACH PROSPECTIVE INVESTOR SHOULD CONSULT ITS OWN TAX ADVISOR REGARDING THE TAX CONSEQUENCES OF PURCHASING, HOLDING AND DISPOSING OF OUR COMMON STOCK, INCLUDING THE CONSEQUENCES OF ANY PROPOSED CHANGE IN APPLICABLE LAW.

Underwriting

We and the selling stockholders are offering the shares of common stock described in this prospectus through a number of underwriters. J.P. Morgan Securities LLC is acting as book-running manager of the offering and as representative of the underwriters. We and the selling stockholders have entered into an underwriting agreement with the underwriters. Subject to the terms and conditions of the underwriting agreement, we and the selling stockholders have agreed to sell to the underwriters, and each underwriter has severally agreed to purchase, at the public offering price less the underwriting discounts and commissions set forth on the cover page of this prospectus, the number of shares of common stock listed next to its name in the following table:

Name	Number of shares
J.P. Morgan Securities LLC	1,800,000
Stifel, Nicolaus & Company, Incorporated	800,000
BMO Capital Markets Corp.	800,000
Robert W. Baird & Co. Incorporated	300,000
William Blair & Company, L.L.C.	300,000
Total	4,000,000

The underwriters are committed to purchase all the common shares offered by us and the selling stockholders if they purchase any shares. The underwriting agreement also provides that if an underwriter defaults, the purchase commitments of non-defaulting underwriters may also be increased or the offering may be terminated.

The underwriters propose to offer the common shares directly to the public at the public offering price set forth on the cover page of this prospectus and to certain dealers at that price less a concession not in excess of $2.034 per share. After the initial public offering of the shares, the offering price and other selling terms may be changed by the underwriters. Sales of shares made outside of the United States may be made by affiliates of the underwriters.

The underwriters have an option to buy up to 600,000 additional shares of common stock from us. The underwriters have 30 days from the date of this prospectus to exercise this option. If any shares are purchased with this option, the underwriters will purchase shares in approximately the same proportion as shown in the table above. If any additional shares of common stock are purchased, the underwriters will offer the additional shares on the same terms as those on which the shares are being offered.

The underwriting fee is equal to the public offering price per share of common stock less the amount paid by the underwriters to us and the selling stockholders per share of common stock. The underwriting fee is $3.39 per share. The following tables shows the per share and total underwriting discounts and commissions to be paid to the underwriters assuming both no exercise and full exercise of the underwriters' option to purchase additional shares.

Paid by us	Without option exercise	With full option exercise
Per Share	$ 3.39	$ 3.39
Total	$10,170,000	$12,204,000

Paid by the selling stockholders	Without option exercise	With full option exercise
Per Share	$ 3.39	$ 3.39
Total	$3,390,000	$3,390,000

Pursuant to the terms of the underwriting agreement, we have also agreed to reimburse the underwriters for certain expenses, including reasonable fees and expenses of counsel, relating to certain aspects of this offering that will not exceed $30,000. We estimate that the total expenses of this offering, including registration, filing and listing fees, printing fees and legal and accounting expenses, but excluding the underwriting discounts and commissions, will be approximately $700,000.

A prospectus in electronic format may be made available on the web sites maintained by one or more underwriters, or selling group members, if any, participating in the offering. The underwriters may agree to allocate a number of shares to underwriters and selling group members for sale to their online brokerage account holders. Internet distributions will be allocated by the representatives to underwriters and selling group members that may make Internet distributions on the same basis as other allocations.

We and the selling stockholders have agreed that we will not (i) offer, pledge, announce the intention to sell, sell, 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 or otherwise dispose of, directly or indirectly, or file with the Securities and Exchange Commission a registration statement under the Securities Act relating to, any shares of our common stock or securities convertible into or exchangeable or exercisable for any shares of our common stock, or publicly disclose the intention to make any offer, sale, pledge, disposition or filing, or (ii) enter into any swap or other arrangement that transfers, in whole or in part, any of the economic consequences of ownership of any shares of common stock or any such other securities (regardless of whether any of these transactions are to be settled by the delivery of shares of common stock or such other securities, in cash or otherwise), in each case without the prior written consent of J.P. Morgan Securities LLC for a period of 60 days after the date of this prospectus.

Our directors and executive officers, the selling stockholders, and certain of our significant shareholders have entered into lock-up agreements with the underwriters prior to the commencement of this offering pursuant to which each of these persons or entities, with limited exceptions, for a period of 60 days after the date of this prospectus, may not, without the prior written consent of J.P. Morgan Securities LLC, (1) offer, pledge, announce the intention to sell, sell, 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, or otherwise encumber, transfer or dispose of, directly or indirectly, any shares of our common stock or any securities convertible into or exercisable or exchangeable for our common stock (including, without limitation, common stock or such other securities which may be deemed to be beneficially owned by such directors, executive officers, managers and members in accordance with the rules and regulations of the SEC and securities which may be issued upon exercise of a stock option or warrant) or (2) enter into any swap or other agreement that transfers, in whole or in part, any of the economic consequences of ownership of the common stock or such other securities, whether any such transaction described in clause (1) or (2) above is to be settled by delivery of common stock or such other securities, in cash or otherwise, or (3) make any demand for or exercise any right with respect to the registration of any shares of our common stock or any security convertible into or exercisable or exchangeable for our common stock.

We and the selling stockholders have agreed to indemnify the underwriters against certain liabilities, including liabilities under the Securities Act.

Our common stock is listed on the NASDAQ Global Market under the symbol “ASPX.”

In connection with this offering, the underwriters may engage in stabilizing transactions, which involves making bids for, purchasing and selling shares of common stock in the open market for the purpose of preventing or retarding a decline in the market price of the common stock while this offering is in progress. These stabilizing transactions may include making short sales of the common stock, which involves the sale by the underwriters of a greater number of shares of common stock than they are required to purchase in this offering, and purchasing shares of common stock on the open market to cover positions created by short sales. Short sales may be "covered" shorts, which are short positions in an amount not greater than the underwriters' option referred to above, or may be "naked" shorts, which are short positions in excess of that amount. The underwriters may close out any covered short position either by exercising their option, in whole or in part, or by purchasing shares in the open market. In making this determination, the underwriters will consider, among other things, the price of shares available for purchase in the open market compared to the price at which the underwriters may purchase shares through the option. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the common stock in the open market that could adversely affect investors who purchase in this offering. To the extent that the underwriters create a naked short position, they will purchase shares in the open market to cover the position.

The underwriters have advised us that, pursuant to Regulation M of the Securities Act, they may also engage in other activities that stabilize, maintain or otherwise affect the price of the common stock, including the imposition of penalty bids. This means that if the representatives of the underwriters purchase common stock in the open market in stabilizing transactions or to cover short sales, the representatives can require the underwriters that sold those shares as part of this offering to repay the underwriting discount received by them.

These activities may have the effect of raising or maintaining the market price of the common stock or preventing or retarding a decline in the market price of the common stock, and, as a result, the price of the common stock may be higher than the price that otherwise might exist in the open market. If the underwriters commence these activities, they may discontinue them at any time. The underwriters may carry out these transactions on the NASDAQ Global Market, in the over-the-counter market or otherwise.

In addition, in connection with this offering certain of the underwriters (and selling group members) may engage in passive market making transactions in our common stock on The Nasdaq Stock Market prior to the pricing and completion of this offering. Passive market making consists of displaying bids on The Nasdaq Stock Market no higher than the bid prices of independent market makers and making purchases at prices no higher than these independent bids and effected in response to order flow. Net purchases by a passive market maker on each day are generally limited to a specified percentage of the passive market maker's average daily trading volume in the common stock during a specified period and must be discontinued when such limit is reached. Passive market making may cause the price of our common stock to be higher than the price that otherwise would exist in the open market in the absence of these transactions. If passive market making is commenced, it may be discontinued at any time.

Other than in the United States, no action has been taken by us, the selling stockholders or the underwriters that would permit a public offering of the securities offered by this prospectus in any jurisdiction where action for that purpose is required. The securities offered by this prospectus may not be offered or sold, directly or indirectly, nor may this prospectus or any other offering material or advertisements in connection with the offer and sale of any such securities be distributed or published in any jurisdiction, except under circumstances that will result in compliance with the applicable rules and regulations of that jurisdiction. Persons into whose possession this prospectus comes are advised to inform themselves about and to observe any restrictions relating to the offering and the distribution of this prospectus. This prospectus does not constitute an offer to sell or a solicitation of an offer to buy any securities offered by this prospectus in any jurisdiction in which such an offer or a solicitation is unlawful.

This document is only being distributed to and is only directed at (i) persons who are outside the United Kingdom or (ii) to investment professionals falling within Article 19(5) of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005 (the "Order") or (iii) high net worth entities, and other persons to whom it may lawfully be communicated, falling with Article 49(2)(a) to (d) of the Order (all such persons together being referred to as "relevant persons"). The securities are only available to, and any invitation, offer or agreement to subscribe, purchase or otherwise acquire such securities will be engaged in only with, relevant persons. Any person who is not a relevant person should not act or rely on this document or any of its contents.

In relation to each Member State of the European Economic Area which has implemented the Prospectus Directive (each, a "Relevant Member State"), from and including the date on which the European Union Prospectus Directive (the ''EU Prospectus Directive'') was implemented in that Relevant Member State (the "Relevant Implementation Date") an offer of securities described in this prospectus may not be made to the public in that Relevant Member State prior to the publication of a prospectus in relation to the shares which has been approved by the competent authority in that Relevant Member State or, where appropriate, approved in another Relevant Member State and notified to the competent authority in that Relevant Member State, all in accordance with the EU Prospectus Directive, except that, with effect from and including the Relevant Implementation Date, an offer of securities described in this prospectus may be made to the public in that Relevant Member State at any time:

- to any legal entity which is a qualified investor as defined under the EU Prospectus Directive;
- to fewer than 100 or, if the Relevant Member State has implemented the relevant provision of the 2010 PD Amending Directive, 150 natural or legal persons (other than qualified investors as defined in the EU Prospectus Directive); or
- in any other circumstances falling within Article 3(2) of the EU Prospectus Directive, provided that no such offer of securities described in this prospectus shall result in a requirement for the publication by us of a prospectus pursuant to Article 3 of the EU Prospectus Directive.

For the purposes of this provision, the expression an "offer of securities to the public" in relation to any securities 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 the securities to be offered so as to enable an investor to decide to purchase or subscribe for the securities, as the same may be varied in that Member State by any measure implementing the EU Prospectus Directive in that Member State. The expression "EU Prospectus Directive" means Directive 2003/71/EC (and any 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.

Certain of the underwriters and their affiliates have provided in the past to us, our affiliates and the selling stockholders and may provide from time to time in the future certain commercial banking, financial advisory, investment banking and other services for us, our affiliates and the selling stockholders in the ordinary course of their business, for which they have received and may continue to receive customary fees and commissions. In addition, from time to time, certain of the underwriters and their affiliates may effect transactions for their own account or the account of customers, and hold on behalf of themselves or their customers, long or short positions in our debt or equity securities or loans, and may do so in the future. The underwriters of this offering, other than J.P. Morgan Securities LLC, were the underwriters of our initial public offering and our July 2014 follow-on offering.

In addition, Maxim Group LLC is acting as our advisor.

Legal matters

The validity of the shares of common stock being offered by this prospectus will be passed upon for us by Cooley LLP, San Diego, California. The underwriters are being represented by Latham & Watkins LLP, San Diego, California.

Experts

Ernst & Young LLP, independent registered public accounting firm, has audited our financial statements included in our Annual Report on Form 10-K for the year ended December 31, 2013, as set forth in their report, which is incorporated by reference in this prospectus and elsewhere in the registration statement. Our financial statements are incorporated by reference in reliance on Ernst & Young LLP's report, given on their authority as experts in accounting and auditing.

Where you can find additional information

We have filed with the SEC a registration statement on Form S-1 under the Securities Act, with respect to the shares of common stock being offered by this prospectus. This prospectus does not contain all of the information in the registration statement and its exhibits. For further information with respect to us and the common stock offered by this prospectus, we refer you to the registration statement and its exhibits. Statements contained in this prospectus as to the contents of any contract or any other document referred to are not necessarily complete, and in each instance, we refer you to the copy of the contract or other document filed as an exhibit to the registration statement. Each of these statements is qualified in all respects by this reference.

You can read our SEC filings, including the registration statement, over the Internet at the SEC's website at www.sec.gov. You may also read and copy any document we file with the SEC at its public reference facilities at 100 F Street NE, Washington, D.C. 20549. You may also obtain copies of these documents at prescribed rates by writing to the Public Reference Section of the SEC at 100 F Street N.E., Washington, D.C. 20549. Please call the SEC at 1-800-SEC-0330 for further information on the operation of the public reference facilities. You may also request a copy of these filings, at no cost, by writing us at 3333 N. Torrey Pines Court, Suite 400, San Diego, California 92037 or telephoning us at (858) 558-2401.

We are subject to the information and periodic reporting requirements of the Exchange Act, and we file periodic reports, proxy statements and other information with the SEC. These periodic reports, proxy statements and other information are available for inspection and copying at the public reference room and website of the SEC referred to above. We maintain a website at http://www.auspexpharma.com. You may access our annual reports on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K and amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Exchange Act with the SEC free of charge at our website as soon as reasonably practicable after such material is electronically filed with, or furnished to, the SEC. The information contained in, or that can be accessed through, our website is not incorporated by reference in, and is not part of, this prospectus.

Incorporation of certain information by reference

The SEC allows us to "incorporate by reference" information from other documents that we file with it, which means that we can disclose important information to you by referring you to those documents. The information incorporated by reference is considered to be part of this prospectus. Information in this prospectus supersedes information incorporated by reference that we filed with the SEC prior to the date of this prospectus.

We incorporate by reference into this prospectus and the registrations statement of which this prospectus is a part the information or documents listed below that we have filed with the SEC (Commission File No. 001-36292):

- our annual report on Form 10-K for the year ended December 31, 2013, filed with the SEC on March 28, 2014;
- our quarterly reports on Form 10-Q for the quarters ended March 31, 2014, June 30, 2014 and September 30, 2014, filed with the SEC on May 14, 2014, August 7, 2014 and November 10, 2014;
- the description of our common stock contained in our Registration Statement on Form 8-A filed on January 30, 2014, including any amendment or report filed for the purpose of updating such description; and
- our current reports on Form 8-K filed with the SEC on February 11, 2014, April 1, 2014, May 7, 2014, June 13, 2014, July 25, 2014, December 16, 2014, and January 12, 2015 (other than portions of those documents not deemed to be filed).

We will furnish without charge to you, on written or oral request, a copy of any or all of the documents incorporated by reference, including exhibits to these documents. You should direct any requests for documents by writing us at 3333 N. Torrey Pines Court, Suite 400, San Diego, California 92037 or telephoning us at (858) 558-2400.

Any statement contained in a document incorporated or deemed to be incorporated by reference in this prospectus will be deemed modified, superseded or replaced for purposes of this prospectus to the extent that a statement contained in this prospectus modifies, supersedes or replaces such statement.

4,000,000 shares

Common shares

Prospectus

Sole Book-Running Manager

J.P. Morgan

Co-Lead Managers

Stifel **BMO Capital Markets**

Co-Managers

Baird **William Blair**

January 22, 2015

You should rely only on the information contained in this prospectus. Neither we nor the selling stockholders have authorized anyone to provide you with information different from that contained in this prospectus. We and the selling stockholders are offering to sell, and seeking offers to buy, common shares only in jurisdictions where offers and sales are permitted. 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 of any sale of our common shares.

No action is being taken in any jurisdiction outside the United States to permit a public offering of the common shares or possession or distribution of this prospectus in that jurisdiction. Persons who come into possession of this prospectus in jurisdictions outside the United States are required to inform themselves about and to observe any restrictions as to this offering and the distribution of this prospectus applicable to that jurisdiction.