FATE THERAPEUTICS INC
Form 424B4
October 01, 2013
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Filed Pursuant to Rule 424(b)(4)
Registration No. 333-190608

PROSPECTUS **Dated September 30, 2013**

6,666,667 Shares

Common Stock

This is the initial public offering of shares of our common stock. We are offering 6,666,667 shares of our common stock. Prior to this offering, there has been no public market for our common stock. Our common stock has been approved for listing on The NASDAQ Global Market under the symbol FATE. The initial public offering price of our common stock is $6.00 per share.

We are an emerging growth company under applicable Securities and Exchange Commission rules and will be subject to reduced public company reporting requirements for this prospectus and future filings. See Prospectus Summary Implications of Being an Emerging Growth Company.

Our business and an investment in our common stock involve significant risks. These risks are described under the caption Risk Factors beginning on page 11 of this prospectus.

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

	Per Share	*Total*
Public offering price	$ 6.00	$ 40,000,002
Underwriting discount from shares offered to the public[(1)]	$ 0.42	$ 1,750,001
Proceeds, before expenses, to us from shares offered to the public	$ 5.58	$ 23,250,007
Underwriting discount from shares offered to certain of our current stockholders[(1)]	$ 0.21	$ 525,000
Proceeds, before expenses, to us from shares offered to certain of our current stockholders	$ 5.79	$ 14,474,994

(1) We refer you to Underwriting beginning on page 151 of this prospectus for additional information regarding total underwriter compensation.

The underwriters may also purchase up to an additional 1,000,000 shares from us at the public offering price, less the underwriting discount, within 30 days from the date of this prospectus.

Certain holders of five percent or more of our voting securities and stockholders who are affiliated with certain of our directors have agreed to purchase an aggregate of 2,499,999 shares of our common stock in this offering at the initial public offering price.

The underwriters expect to deliver the shares against payment in New York, New York on October 4, 2013.

Cowen and Company **BMO Capital Markets**

Wedbush PacGrow Life Sciences

September 30, 2013

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Through and including October 25, 2013 (the 25th day after the date of this prospectus), all dealers effecting transactions in these securities, whether or not participating in this offering, may be required to deliver a prospectus. This delivery requirement is in addition to the obligation of dealers to deliver a prospectus when acting as underwriters and with respect to their unsold allotments or subscriptions.

You should rely only on the information contained in this prospectus or in any free writing prospectus we file with the Securities and Exchange Commission. We and the underwriters have not authorized anyone to provide you with information different from that contained in this prospectus or any free writing prospectus. We and the underwriters are offering to sell, and seeking offers to buy, common stock only in jurisdictions where offers and sales are permitted. The information contained in this prospectus is accurate only as of the date on the front cover of this prospectus, or other earlier date stated in this prospectus, regardless of the time of delivery of this prospectus or of any sale of our common stock.

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PROSPECTUS SUMMARY

Overview

We are a clinical-stage biopharmaceutical company engaged in the discovery and development of pharmacologic modulators of adult stem cells to treat orphan diseases, including certain hematologic malignancies, lysosomal storage disorders and muscular dystrophies. Our novel approaches utilize established pharmacologic modalities, including small molecules and therapeutic proteins, and target well-characterized biological mechanisms to enhance the therapeutic potential of adult stem cells. Adult stem cells play a key role in the growth, maintenance and repair of many tissues and organ systems in the body. Due to their natural ability to self-renew, and to regenerate and repair diseased or damaged tissue, adult stem cells hold considerable therapeutic promise. Based on our deep understanding of key biological mechanisms that guide the fate of adult stem cells, we have built two platforms that modulate the activity of adult stem cells using techniques that operate both outside of the body, or *ex vivo*, and within the body, or *in vivo*.

Our HSC modulation platform focuses on the *ex vivo* pharmacologic optimization of hematopoietic stem cells, or HSCs, which are adult stem cells that regenerate all types of blood cells throughout a person s lifespan. HSCs have been used for decades in a potentially curative or life-saving procedure called hematopoietic stem cell transplant, or HSCT. This procedure is most commonly performed in patients with hematologic malignancies to replace a diseased hematopoietic system with a healthy one. While over one million HSCT procedures have been performed to date, we believe HSCs have not been pharmacologically optimized to improve patient outcomes.

ProHema, the lead product candidate from our HSC modulation platform, is a pharmacologically-modulated HSC therapeutic derived from umbilical cord blood. We have established human proof-of-concept for ProHema in the clinical setting by demonstrating enhanced engraftment of HSCs within the bone marrow. Engraftment, which is the localization and integration of HSCs within a targeted tissue where they can produce new cells, is an important determinant of patient outcomes in HSCT. We are presently advancing ProHema in Phase 2 clinical development for hematologic malignancies. We are also developing pharmacologically optimized HSC therapeutics for the treatment of certain lysosomal storage disorders, or LSDs, where HSCs have demonstrated the ability to home, or migrate, to and engraft within the central nervous system, or CNS.

Our SSC modulation platform focuses on the *in vivo* pharmacologic activation of satellite stem cells, or SSCs, which are adult stem cells that regenerate muscle throughout a person s lifespan. The regenerative capacity of SSCs in skeletal muscle is exhausted both in the natural aging process and in degenerative conditions such as muscular dystrophies. We have identified Wnt7a as a natural promoter of SSCs to drive muscle regeneration and are initially focused on developing Wnt7a protein analogs for the treatment of muscular dystrophies. We believe that our regenerative approach for treating muscular dystrophies holds significant therapeutic promise and is distinct from other approaches, which focus on preventing muscle degeneration.

Our Wnt7a analogs have demonstrated proof-of-concept in animal models of muscular dystrophy. In these studies, our Wnt7a analogs were shown to drive a significant expansion of the SSC population, as well as significant increases in muscle hypertrophy and muscle strength. We are presently advancing our Wnt7a analogs in preclinical development with the goal of filing an Investigational New Drug application, or IND, in 2014.

We believe that the product candidates generated by our stem cell modulation platforms have significant potential as life-changing or curative therapeutics across a broad range of orphan indications. We plan to continue the validation of our product platforms by demonstrating the clinical benefit of our initial product candidates over the next three years in three orphan disease settings: hematologic malignancies, LSDs and muscular dystrophy.

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Our HSC Modulation Platform

Our HSC modulation platform represents a novel approach to improving patient outcomes in HSCT: we enhance the biological properties of HSCs *ex vivo* to drive well-understood biological mechanisms *in vivo* that are critical to the success of the procedure. Our novel approach encompasses the following advantages:

We optimize HSCs ex vivo to enhance their biological properties. Our strategies and methods of optimizing HSCs *ex vivo* are designed to achieve desired therapeutic effects *in vivo*. Our proprietary processes induce profound changes in the expression of key genes in HSCs that are critical for homing and engraftment.

Our platform is applicable across different stem cell sources and a broad range of diseases. We believe that our approach to the pharmacologic optimization of HSCs can be applied across various sources of HSCs in two HSCT settings: allogeneic HSCT, where healthy HSCs from a donor are transplanted into the patient, and autologous HSCT, where the patient receives his or her own HSCs collected from the patient s bone marrow or blood. Accordingly, we believe our HSC modulation platform will enable us to develop additional HSC therapeutics to treat a broad spectrum of orphan disorders.

Our proprietary HSC optimization process can be readily adopted into the HSCT standard of care. We believe we can efficiently optimize HSCs in a rapid *ex vivo* modulation process conducted onsite at the clinical center. Following this process, the enhanced cells are washed to remove the modulators and can be immediately infused into the patient within the established framework of HSCT.

Our SSC Modulation Platform

We believe we are the first company to demonstrate that SSCs can be pharmacologically modulated *in vivo* to improve muscle regeneration. We believe that our approach is novel and applicable across multiple forms of muscular dystrophies and neuromuscular disorders. The advantages of our SSC modulation platform include:

Our modulation of SSCs is receptor-mediated and highly specific. We leverage the inherent specificity conferred by the endogenous protein Wnt7a and its receptor, which is selectively expressed in muscle tissue. We believe this will enable us to develop therapeutics with a low risk of off-target effects.

Our SSC modulation platform is enabled by our expertise in the development of Wnt-based therapeutics. While the regenerative potential of the Wnt protein family is well established, Wnt proteins have not been developed as therapeutics due to challenges in the scaled manufacture and formulation of this class of proteins. We have systematically applied structural prediction, rational design and protein engineering techniques to overcome these challenges. We believe we are the first company to produce Wnt analogs that are amenable to therapeutic development and *in vivo* administration.

We drive muscle regeneration through a unique dual mechanism of action. In preclinical studies, a single injection of our Wnt7a analogs resulted in an expansion of the SSC population and an increase in muscle hypertrophy. We have demonstrated that these profound effects resulted in a significant increase in muscle strength. We believe the ability of our Wnt7a protein analogs to both activate SSC population expansion and increase muscle hypertrophy is a unique dual mechanism of action for the treatment of muscular dystrophies.

Our Wnt7a analogs have therapeutic potential as stand-alone or complementary treatments across a broad spectrum of muscular dystrophies. While most approaches to treat muscular dystrophies seek to slow the degeneration of muscle in genetically distinct subtypes of the disease, our Wnt7a analogs drive muscle regeneration and have the potential to treat a broader spectrum of muscular dystrophies either as stand-alone or complementary therapeutics.

Our SSC modulation platform has potential beyond muscular dystrophies. We believe that the regenerative potential of our Wnt7a analogs is broadly applicable to other neuromuscular disorders such as cachexia, atrophy, trauma and sarcopenia.

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Our Product Candidate Pipeline

The following table summarizes key information about our two platforms and our product candidates:

Product Candidate	Targeted Orphan Disorders[(1)]	Status
HSC Modulation Platform		
ProHema	Adult hematologic malignancies	Phase 2
ProHema	Pediatric hematologic malignancies	Preclinical
ProHema	LSDs	Preclinical
Second Generation HSC Therapeutic	LSDs	Preclinical
SSC Modulation Platform		
Wnt7a Protein Analogs	Muscular dystrophies	Preclinical
Wnt7a Protein Analogs	Neuromuscular disorders	Preclinical

(1) We have been granted orphan designation in the United States for human allogeneic HSCs *ex vivo* modulated with 16, 16-dimethyl prostaglandin E2, which we refer to as FT1050, for the enhancement of stem cell engraftment and in the European Union for ProHema for the treatment of acute myelogenous leukemia through the *ex vivo* modulation of allogeneic umbilical cord blood cells.

Our ProHema Product Candidate

ProHema is a pharmacologically-modulated HSC therapeutic derived from umbilical cord blood. It is manufactured in the transplant center by modulating an umbilical cord blood unit with FT1050 to create our final HSC therapeutic. The HSC modulation process takes only two hours, can be performed directly in the transplant center, does not require significant changes to existing infrastructure and is compatible with standard of care treatment modalities.

ProHema has the potential to improve patient outcomes by enhancing hematopoietic reconstitution through accelerated and durable engraftment, facilitating greater donor matching flexibility, reducing the risk of major side effects, and enabling the use of less toxic conditioning regimens. In a Phase 1b clinical trial conducted at the Dana Farber Cancer Institute and the Massachusetts General Hospital in adult patients with hematologic malignancies, we observed that treatment with ProHema resulted in a statistically significant improvement in time to engraftment of neutrophils, a type of white blood cell primarily involved in fighting bacterial infections, as compared to a historical control group that consisted of 53 adult patients with hematologic malignancies who underwent double umbilical cord blood HSCT procedures at these institutions. We also observed improvements in the cumulative incidence of engraftment of neutrophils and platelets, a type of blood cell involved in the prevention of bleeding; favorable 100-day survival; a low incidence of a serious complication known as graft-versus-host disease, or GvHD; and durable long-term hematopoietic reconstitution.

Based on these data, we initiated a Phase 2 clinical trial of ProHema in adult patients with hematologic malignancies in the fourth quarter of 2012. More recently, we have demonstrated that we can further enhance the potency and efficacy of ProHema by incorporating an improved nutrient-rich media formulation, which we refer to as our NRM formulation, in the manufacture of ProHema. In preclinical studies, ProHema manufactured using our NRM formulation exhibited a more than two-fold improvement in engraftment over the prior media formulation.

In order to take advantage of this recent development, we have elected to pause enrollment in our Phase 2 clinical trial to incorporate our NRM formulation. Our Phase 2 clinical trial of ProHema is currently active but

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not recruiting. On August 1, 2013, we submitted an IND amendment to the FDA, which contained preclinical and product development data to support the incorporation of our NRM formulation for the manufacture of ProHema. Based on our recent communications with the FDA regarding this amendment, we expect to resume enrollment of our Phase 2 clinical trial of ProHema incorporating our NRM formulation in the first half of 2014, with the goal of generating full data from this trial in mid-2015. We also expect to commence an additional clinical trial in children and adolescents with hematologic malignancies in 2014.

Our Phase 1b clinical trial was designed with safety as the primary endpoint and not efficacy. To support marketing approval, we will need to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that ProHema is safe and effective, and otherwise meets the appropriate standards required for approval for each targeted indication, in subsequent well-designed and conducted clinical trials, including our Phase 2 clinical trial and a Phase 3 registrational trial that we intend to initiate if our Phase 2 clinical trial is successful. We may not be able to achieve or replicate the results of our Phase 1b clinical trial in our Phase 2 clinical trial or other subsequent trials for a variety of reasons. For example, the anticipated use of our NRM formulation in our Phase 2 clinical trial may not produce the efficacy or safety benefits that we currently expect; the increase in the number of patients enrolled in later-stage trials may not produce the same or similar results as earlier trials with fewer patients; and the expansion in the number of participating clinical centers in later-stage trials may present operational and manufacturing challenges.

The therapeutic potential of HSCT procedures in LSDs has been demonstrated in clinical studies showing that many neurological manifestations of LSDs can be prevented or substantially reduced through early HSCT intervention. These effects have been attributed to the ability of HSCs to home to and engraft within the CNS, where they give rise to cells that correct the underlying enzyme deficiency in the brain. We have demonstrated in a preclinical model that the *ex vivo* modulation of HSCs increased the number of transplanted cells that home to the CNS. We plan to initiate a clinical trial of ProHema in patients with LSDs in 2014, with the goal of generating data from this trial in 2015. We are also developing second-generation HSC therapeutics to further improve the CNS-homing ability of modulated HSCs.

Our Wnt7a Product Candidates

We have identified Wnt7a, a naturally-occurring secreted protein, as a key regulator of skeletal muscle regeneration. We have demonstrated the therapeutic potential of our proprietary Wnt7a analogs in various preclinical models. In these studies, a single administration of a Wnt7a analog resulted in an approximately three-fold expansion in the population of SSCs, an approximately 20% increase in muscle hypertrophy, a reduction in disease-specific muscle fiber necrosis and an approximately 19% increase in muscle strength.

Subject to the completion of IND-enabling studies, we plan to file an IND in 2014 to evaluate a Wnt7a analog in the clinical setting, with the goal of generating data from our first clinical trials in 2015. The primary objectives of these trials will be to provide initial safety assessments and pharmacodynamic analyses, evaluate dose and treatment regimen of the Wnt7a analog in healthy volunteers and X chromosome-linked muscular dystrophy patients, and assess efficacy.

Our Strategy

Our goal is to realize the therapeutic potential of our two stem cell modulation platforms across a broad range of orphan diseases through the discovery, development and commercialization of first-in-class therapeutics. The key elements of our strategy are to:

- validate the therapeutic potential of our stem cell modulation platforms by demonstrating the clinical benefit of our initial product candidates over the next three years in three orphan disease settings: hematologic malignancies, LSDs and muscular dystrophy;

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develop and commercialize our orphan product candidates through efficient clinical development, expedited regulatory pathways and a focused and highly targeted commercial infrastructure; and

leverage lifecycle opportunities through indication expansion and the generation of additional product candidates.

We may also seek partners who can bring therapeutic, development and commercialization capabilities, geographical expertise and financial resources that allow us to leverage the potential of our product platforms within or beyond our initial clinical and commercial focus.

Our Risks

An investment in our common stock involves a high degree of risk. You should carefully consider the risks summarized below. These risks are discussed more fully in the Risk Factors section immediately following this prospectus summary. These risks include, but are not limited to, the following:

we have a limited operating history, have incurred significant losses since our inception and anticipate that we will continue to incur significant losses in the foreseeable future;

we will need to obtain additional financing to complete the clinical development of ProHema and the preclinical and clinical development of our Wnt protein analogs;

because our platforms and product candidates are based on novel technologies, it is difficult to predict the time and cost of development and commercialization. Moreover, we cannot be certain that we can successfully complete clinical development and obtain the necessary regulatory approvals for commercialization;

we may face delays in the clinical development of ProHema and the preclinical and clinical development of our Wnt protein analogs and may fail to commence, resume or complete any of our planned development activities;

we have not completed clinical development of any product candidates and do not have any products approved for sale by the FDA or any other regulatory bodies;

we rely on third parties to manufacture our product candidates and to manage various aspects of our clinical trials;

we may face difficulties in protecting and maintaining our intellectual property rights, including intellectual property rights that are licensed to us;

we currently do not have the infrastructure to commercialize any of our product candidates if such products receive regulatory approval; and

we expect to face significant uncertainty over pricing and reimbursement of any of our product candidates that are approved for commercialization.

Implications of Being an Emerging Growth Company

We qualify as an emerging growth company as defined in the Jumpstart Our Business Startups Act of 2012, as amended, or the JOBS Act. As an emerging growth company, we may take advantage of specified reduced disclosure and other requirements that are otherwise applicable generally to public companies. These provisions include:

- only two years of audited financial statements in addition to any required unaudited interim financial statements with correspondingly reduced Management s Discussion and Analysis of Financial Condition and Results of Operations disclosure;

- reduced disclosure about our executive compensation arrangements;

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no non-binding advisory votes on executive compensation or golden parachute arrangements; and

exemption from the auditor attestation requirement in the assessment of our internal control over financial reporting.

We may take advantage of these exemptions for up to five years or such earlier time that we are no longer an emerging growth company. We would cease to be an emerging growth company on the date that is the earliest of (i) the last day of the fiscal year in which we have total annual gross revenues of $1 billion or more; (ii) the last day of our fiscal year following the fifth anniversary of the date of the completion of this offering; (iii) the date on which we have issued more than $1 billion in nonconvertible debt during the previous three years; or (iv) the date on which we are deemed to be a large accelerated filer under the rules of the Securities and Exchange Commission, or SEC. We may choose to take advantage of some but not all of these exemptions. We have taken advantage of reduced reporting requirements in this prospectus. Accordingly, the information contained herein may be different from the information you receive from other public companies in which you hold stock. We have irrevocably elected to opt out of the exemption for the delayed adoption of certain 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.

Company and Other Information

We were incorporated under the laws of the State of Delaware in April 2007. Our principal executive office is located at 3535 General Atomics Court, Suite 200, San Diego, CA 92121, and our telephone number is (858) 875-1800. Our website address is www.fatetherapeutics.com. We do not incorporate the information on or accessible through our website into this prospectus, and you should not consider any information on, or that can be accessed through, our website a part of this prospectus.

We own various U.S. federal trademark registrations and applications, and unregistered trademarks, including the following marks referred to in this prospectus: Fate Therapeutics®, our corporate logo and ProHema®. All other trademarks or trade names referred to in this prospectus are the property of their respective owners. Solely for convenience, the trademarks and trade names in this prospectus are referred to without the symbols ® and , but such references should not be construed as any indicator that their respective owners will not assert, to the fullest extent under applicable law, their rights thereto.

This prospectus summary highlights information contained elsewhere in this prospectus and does not contain all of the information that you should consider in making your investment decision. Before investing in our common stock, you should carefully read this entire prospectus, including our financial statements and the related notes included elsewhere in this prospectus. You should also consider, among other things, the matters described under Risk Factors and Management s Discussion and Analysis of Financial Condition and Results of Operations in each case appearing elsewhere in this prospectus. Unless otherwise stated, all references to us, our, Fate, we, the Company and similar designations ref to Fate Therapeutics, Inc.

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The Offering

Common stock offered by us	6,666,667 shares
Common stock to be outstanding immediately after this offering	19,156,609 shares
Underwriters option to purchase additional shares	1,000,000 shares
Use of proceeds	We intend to use the proceeds from this offering for research and development activities to advance our HSC modulation platform and SSC modulation platform, the clinical and preclinical development of our product candidates and working capital and general corporate purposes. For a more complete description of our intended use of the proceeds from this offering, see Use of Proceeds.
Risk factors	You should carefully read Risk Factors on page 11 in this prospectus for a discussion of factors that you should consider before deciding to invest in our common stock.
NASDAQ Global Market symbol	FATE

Certain holders of five percent or more of our voting securities and stockholders who are affiliated with certain of our directors have agreed to purchase an aggregate of 2,499,999 shares of our common stock in this offering at the initial public offering price, as follows:

Beneficial Owner	Shares to be Purchased in Offering
ARCH Venture Fund VI, L.P.	833,333
Entities affiliated with Polaris Venture Partners	833,333
Entities affiliated with Venrock	833,333

The underwriting discount for the shares sold to these stockholders in the offering will be $0.21 per share.

The number of shares of our common stock to be outstanding after this offering is based on 12,489,942 shares of our common stock outstanding as of June 30, 2013 and excludes:

- 1,518,712 shares of common stock issuable upon the exercise of stock options outstanding as of June 30, 2013 at a weighted average exercise price of $1.46 per share;
- 36,074 shares of common stock issuable upon the exercise of warrants outstanding as of June 30, 2013 at a weighted average exercise price of $7.21 per share, which warrants prior to the completion of this offering are exercisable to purchase convertible preferred stock, assuming such warrants will not be exercised prior to the completion of this offering;

1,020,000 shares of common stock reserved for future issuance under our 2013 Stock Option and Incentive Plan, or the 2013 Plan, which will become effective immediately prior to the completion of this offering;

729,000 shares of common stock reserved for future issuance under our 2013 Employee Stock Purchase Plan, or the ESPP, which will become effective immediately prior to the completion of this offering; and

up to 480,764 shares of common stock that may be issuable to holders of exchangeable shares of Fate Therapeutics (Canada) Inc., or Fate Canada, in connection with certain milestone or change of control events that may occur after this offering, as described in Description of Capital Stock Exchangeable Shares in Canadian Subsidiary elsewhere in this prospectus.

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Unless otherwise indicated, all information in this prospectus reflects or assumes the following:

- the filing of our amended and restated certificate of incorporation and the adoption of our amended and restated bylaws, which will occur immediately prior to the completion of this offering;

- the conversion of all of our outstanding shares of convertible preferred stock into 7,229,590 shares of common stock upon the completion of this offering;

- the issuance of 3,499,319 shares of common stock upon the conversion of approximately $21.0 million in outstanding principal and accrued interest on our convertible promissory notes issued in June, July and August 2013, or our 2013 Notes, upon the completion of this offering, based on the initial public offering price per share of $6.00, and assuming that we repay in cash, on or before the date 30 days after the completion of this offering, an aggregate of approximately $2.8 million in principal and accrued interest under our 2013 Notes issued in August 2013 in lieu of the issuance of 470,252 shares of common stock upon the conversion of such notes, which, if issued, would cause the holders of such notes and their affiliates and related parties to beneficially own 15% or more of our outstanding common stock following the completion of this offering;

- the issuance of 403,841 shares of common stock pursuant to the redemption of an aggregate of 900,000 exchangeable shares of Fate Canada immediately prior to the completion of this offering, as described in Description of Capital Stock Exchangeable Shares in Canadian Subsidiary elsewhere in this prospectus;

- a one-for-6.5 reverse split of our common stock, which became effective on September 12, 2013; and

- no exercise by the underwriters of their option to purchase up to an additional 1,000,000 shares of common stock in this offering.

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Summary Consolidated Financial Data

The following summary consolidated financial information should be read together with our consolidated financial statements and accompanying notes and information under the caption Management s Discussion and Analysis of Financial Condition and Results of Operations appearing elsewhere in this prospectus. Our historical results are not necessarily indicative of 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 consolidated statement of operations data for the years ended December 31, 2011 and 2012 are derived from our audited consolidated financial statements appearing elsewhere in this prospectus. The summary consolidated statement of operations data for the six months ended June 30, 2012 and 2013 and the period from April 27, 2007 (inception) through June 30, 2013 and consolidated balance sheet data as of June 30, 2013 are derived from our unaudited consolidated financial statements and related notes appearing elsewhere in this prospectus. The unaudited consolidated financial statements have been prepared on a basis consistent with our audited consolidated financial statements included in this prospectus and, in the opinion of management, reflect all adjustments, consisting only of normal recurring adjustments, necessary to fairly state our financial position as of June 30, 2013 and results of operations for the six months ended June 30, 2012 and 2013.

	Years Ended December 31,		Six Months Ended June 30,		Period from April 27, 2007 (inception) through June 30, 2013 (unaudited)
	2011	2012	2012	2013	
			(unaudited)		
	(in thousands, except share and per share data)				
Consolidated Statement of Operations Data:					
Revenue:					
Collaboration revenue	$ 833	$ 1,268	$ 867	$ 417	$ 2,718
Grant revenue	337	1,402	632	345	2,084
Total revenue	1,170	2,670	1,499	762	4,802
Operating expenses:					
Research and development	9,858	11,999	5,281	5,598	50,577
General and administrative	4,605	4,228	2,081	2,789	26,860
Total operating expenses	14,463	16,227	7,362	8,387	77,437
Loss from operations	(13,293)	(13,557)	(5,863)	(7,625)	(72,635)
Total other income (expense)	(134)	(682)	(108)	(1,457)	(2,062)
Net loss and comprehensive loss	$ (13,427)	$ (14,239)	$ (5,971)	$ (9,082)	$ (74,697)
Net loss per common share, basic and diluted[1]	$ (16.16)	$ (13.06)	$ (5.96)	$ (7.41)	
Weighted-average shares used to compute basic and diluted net loss per share[1]	830,959	1,090,317	1,002,649	1,226,451	
Pro forma net loss per common share, basic and diluted (unaudited)[1]		$ (1.92)		$ (0.88)	
Weighted-average shares used to compute pro forma net loss per common share, basic and diluted (unaudited)[1]		7,464,793		8,866,985	

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	As of June 30, 2013		
	Actual	Pro Forma[2] (unaudited) (in thousands)	Pro Forma As Adjusted[3]
Consolidated Balance Sheet Data:			
Cash and cash equivalents	$ 3,399	$ 21,313	$ 56,433
Working capital deficit	(4,267)	16,571	52,433
Total assets	6,173	24,087	58,170
Convertible notes, net of discount	2,730		
Warrant liability[4]	194		
Long-term debt, net of current portion	746	746	746
Exchangeable share liability[4]	1,811		
Convertible preferred stock	56,526		
Deficit accumulated during the development stage	(74,697)	(75,033)	(75,033)
Total stockholders equity (deficit)	(61,411)	17,764	52,589

(1) See Note 1 to our consolidated financial statements included elsewhere in this prospectus for an explanation of the method used to calculate the historical and pro forma net loss per share, basic and diluted, and the number of shares used in the computation of the per share amounts.

(2) The pro forma information in the table gives effect to (i) the filing and effectiveness of our amended and restated certificate of incorporation, (ii) the conversion of all outstanding shares of our convertible preferred stock into an aggregate of 7,229,590 shares of common stock immediately prior to the completion of this offering, (iii) our receipt of aggregate gross proceeds of approximately $20.7 million from the sale of our 2013 Notes issued in July and August 2013, and a $0.1 million debt discount related to a beneficial conversion feature in our 2013 Notes issued in July 2013, (iv) the issuance of 3,499,319 shares of common stock upon the conversion of approximately $21.0 million in outstanding principal and accrued interest on our 2013 Notes upon the completion of this offering, based on the initial public offering price per share of $6.00, (v) our repayment in cash, on or before the date 30 days after the completion of this offering, of an aggregate of approximately $2.8 million in principal and accrued interest under our 2013 Notes issued in August 2013 in lieu of the issuance of 470,252 shares of common stock upon conversion of such notes, which, if issued, would cause the holders of such notes and their affiliates and related parties to beneficially own 15% or more of our outstanding common stock following the completion of this offering, (vi) a charge to retained earnings of $0.3 million upon the completion of this offering to reflect the accelerated amortization of debt discount to interest expense upon conversion of the 2013 Notes, (vii) the issuance of 403,841 shares of common stock pursuant to the redemption of an aggregate of 900,000 exchangeable shares of Fate Canada as described in Description of Capital Stock Exchangeable Shares in Canadian Subsidiary elsewhere in this prospectus, which will occur immediately prior to the completion of this offering, and the resultant reclassification of our exchangeable share liability to additional paid-in capital, a component of stockholders equity and (viii) the adjustment of our outstanding warrants to purchase 230,000 shares of convertible preferred stock into warrants to purchase 36,074 shares of common stock upon the completion of this offering, and the resultant reclassification of our warrant liability to additional paid-in capital, a component of stockholders equity.

(3) The pro forma as adjusted information in the table gives further effect to our sale in this offering of 6,666,667 shares of common stock at the initial public offering price of $6.00 per share, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us.

(4) Upon the completion of our initial public offering, we anticipate recording a charge of approximately $0.6 million related to the final adjustment to fair value of our exchangeable share liability and warrant liability, based on the initial public offering price per share of $6.00, and further assuming all other inputs into our valuation models remain unchanged from those as of June 30, 2013.

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RISK FACTORS

Investing in our common stock involves a high degree of risk. You should carefully consider the risks described below along with all of the other information contained in this prospectus, including our financial statements and the related notes, before deciding whether to purchase our common stock. If any of the adverse events described in the following risk factors, as well as other factors which are beyond our control, actually occurs, our business, results of operations and financial condition may suffer significantly. As a result, the trading price of our common stock could decline, and you may lose all or part of your investment in our common stock.

Risks Related to Our Financial Position

We have a limited operating history, have incurred significant losses since our inception and anticipate that we will continue to incur significant losses for the foreseeable future.

We are a clinical-stage biopharmaceutical discovery and development company, formed in 2007, with a limited operating history. Since inception we have devoted substantially all of our resources to the development of our stem cell modulation platforms, the clinical and preclinical advancement of our product candidates, the creation, licensing and protection of related intellectual property rights and the provision of general and administrative support for these operations. We have not yet obtained regulatory approval for any product candidates or generated any revenues from therapeutic product sales. If ProHema or any of our other product candidates fails in clinical trials or preclinical development, or does not gain regulatory approval, or if our product candidates do not achieve market acceptance, we may never become profitable.

We have incurred net losses in each year since our inception, including net losses of approximately $13.4 million and $14.2 million for the years ended December 31, 2011 and 2012, respectively, and approximately $6.0 million and $9.1 million for the six months ended June 30, 2012 and 2013, respectively. As of June 30, 2013, we had an accumulated deficit of approximately $74.7 million. We expect to continue to incur losses for the foreseeable future, and we expect these losses to increase as we continue our development of, and seek regulatory approval for, our product candidates. In addition, if we receive approval to market any of our product candidates, we will incur additional losses as we build an internal sales and marketing organization to commercialize any approved products. We also expect our expenditures to increase as we add infrastructure and personnel to support our operations as a public company. We anticipate that our net losses for the next several years could be significant as we conduct our planned operations.

Because of the numerous risks and uncertainties associated with pharmaceutical and biological product development, we are unable to accurately predict the timing or amount of increased expenses or when, or if, we will be able to achieve profitability. In addition, our expenses could increase if we are required by the U.S. Food and Drug Administration, or FDA, or comparable foreign regulatory authorities, to perform studies or trials in addition to those currently expected, or if there are any delays in completing our clinical trials or the development of any of our product candidates. The amount of our future net losses will depend, in part, on the rate of increase in our expenses, our ability to generate revenues and our ability to raise additional capital. These net losses have had, and will continue to have, an adverse effect on our stockholders equity and working capital.

We will require substantial additional funding, which may not be available to us on acceptable terms, or at all, and, if not available, may require us to delay, scale back or cease our product development activities and operations.

We are currently advancing ProHema through clinical development and our Wnt7a analogs through preclinical development. Developing biological products, including conducting preclinical studies and clinical trials, is expensive. We will require substantial additional capital in order to complete the clinical development of, and to commercialize, ProHema, and to conduct the research and development and clinical and regulatory activities necessary to bring other product candidates to market. If the FDA or comparable foreign regulatory authorities require that we perform additional preclinical studies or clinical trials at any point, our expenses would further increase beyond what we currently expect, and the anticipated timing of any future clinical development activities

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and potential regulatory approvals would likely be delayed. Raising funds in the current economic environment may be difficult and additional funding may not be available on acceptable terms, or at all.

The amount and timing of our future funding requirements, both near- and long-term, will depend on many factors, including, but not limited to:

- the timing of, and our ability to incorporate, the use of our NRM formulation in our clinical development activities for ProHema, including our Phase 2 clinical trial in adults with hematologic malignancies;
- the agreement by the FDA and any foreign regulatory authorities to accept our protocols for clinical trials of ProHema, our Wnt7a analogs or any other product candidates that we may develop;
- the progress, costs, results and timing of our Phase 2 clinical trial and planned Phase 1b clinical trial of ProHema and our planned additional preclinical studies and Phase 1 clinical trial of our Wnt7a analogs;
- our ability to initiate, and the size, outcome, costs and timing of additional future clinical trials, including Phase 3 clinical trial costs for ProHema, that will be necessary to support any application for regulatory approval of our product candidates;
- the number and characteristics of product candidates that we pursue, including our product candidates in preclinical development;
- our need to expand our research and development activities, including the hiring of additional employees;
- the costs of acquiring, licensing or investing in complementary businesses, products, product candidates and technologies;
- our ability to maintain, expand and defend the scope of our intellectual property portfolio, including the amount and timing of any payments we may be required to make, or that we may receive, in connection with the licensing, filing, prosecution, defense and enforcement of any patents or other intellectual property rights;
- the effect of competing technological and market developments;
- our need to implement additional internal systems and infrastructure, including financial and reporting systems, as we become a public company;
- the costs associated with securing and establishing commercialization and manufacturing capabilities;
- market acceptance of any products for which we receive approval; and
- the economic and other terms, timing of and success of our existing licensing arrangements and any collaboration, licensing or other arrangements into which we may enter in the future.

Some of these factors are outside of our control. Upon the completion of this offering, based upon our currently expected level of operating expenditures, we believe that we will be able to fund our operations for at least the next 12 months. This period could be shortened if there are any significant or unanticipated increases in spending on development programs. In addition, the expected net proceeds from this offering will not be sufficient to complete the advanced clinical development, including Phase 3 clinical trials, of ProHema or clinical trials of our Wnt analogs that would be necessary to support an application for regulatory approval. Accordingly, we will continue to require substantial additional capital beyond the expected proceeds of this offering. Because successful development of our product candidates is uncertain, we are unable to estimate the actual funds we will require to complete research and development and commercialize our products under development.

If we are required to secure additional financing, such additional fundraising efforts may divert our management from our day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates. In addition, we cannot guarantee that future financing will be available in sufficient amounts or on terms acceptable to us, if at all. If we are unable to raise additional capital when required or on acceptable terms, we may be required to significantly delay, scale back or discontinue the development or commercialization of one or more of our product candidates or curtail our operations, which will have a material adverse effect on our business, operating results and prospects.

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We may sell additional equity or debt securities or enter into other arrangements to fund our operations, which may result in dilution to our stockholders and impose restrictions or limitations on our business.

We may seek additional funding through a combination of equity offerings, debt financings, government or other third-party funding and other collaborations, strategic alliances and licensing arrangements. These financing activities may have an adverse impact on our stockholders rights as well as on our operations. For example, pursuant to the terms of the convertible promissory notes issued by us in August 2013 in an aggregate principal amount of $20.0 million, based on the initial public offering price of $6.00 per share, we will be required to repay in cash, on or before the date 30 days after the completion of this offering, an aggregate of approximately $2.8 million in principal and accrued interest under these notes in lieu of the issuance of 470,252 shares of common stock upon the conversion of such notes, which, if issued, would cause the holders of the notes, together with their affiliates and related parties, to beneficially own 15% or more of our outstanding common stock following the completion of this offering. In addition, any equity financing, or any issuance of securities that may be converted, exercised or exchanged for shares of our capital stock, will result in dilution to our stockholders and may cause the market price of our stock to decline, and any debt financing may impose restrictive covenants on our operations or otherwise adversely affect the holdings or the rights of our stockholders. In addition, if we seek funds through arrangements with collaborative partners, these arrangements may require us to relinquish rights to some of our technologies or product candidates or otherwise agree to terms unfavorable to us.

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

Our audited consolidated financial statements at December 31, 2012 and for the year then ended were prepared assuming that we will continue as a going concern. However, the report of our independent registered public accounting firm included elsewhere in this prospectus contains an explanatory paragraph on our consolidated financial statements stating there is substantial doubt about our ability to continue as a going concern, meaning that we may not be able to continue in operation for the foreseeable future or be able to realize assets and discharge liabilities in the ordinary course of operations. Such an opinion could materially limit our ability to raise additional funds through the issuance of new debt or equity securities or otherwise. There is no assurance that sufficient financing will be available when needed to allow us to continue as a going concern. The perception that we may not be able to continue as a going concern may also make it more difficult to operate our business due to concerns about our ability to meet our contractual obligations.

Risks Related to the Discovery, Development and Regulation of Our Product Candidates

If we fail to complete preclinical development and clinical trials, obtain regulatory approval, or successfully commercialize our product candidates from our HSC and SSC modulation platforms, our business would be significantly harmed.

We have not completed clinical development for any of our product candidates and will only obtain regulatory approval to commercialize a product candidate if we can demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities in well-designed and conducted clinical trials that the product candidate is safe, pure and potent, or effective, and otherwise meets the appropriate standards required for approval for a particular indication. Clinical trials are lengthy, complex and extremely expensive processes with uncertain results. A failure of one or more clinical trials may occur at any stage.

We have never obtained marketing approval from the FDA or any comparable foreign regulatory authority for any product candidate. Our ability to obtain regulatory approval of our product candidates depends on, among other things, completion of additional preclinical studies and clinical trials, whether our clinical trials demonstrate statistically significant efficacy with safety issues that do not potentially outweigh the therapeutic benefit of the product candidates, and whether the regulatory agencies agree that the data from our future clinical trials are sufficient to support approval for any of our product candidates. The final results of our current and future clinical trials may not meet the FDA s or other regulatory agencies requirements to approve a product candidate for marketing, and the regulatory agencies may otherwise determine that our manufacturing processes

or facilities are insufficient to support approval. We may need to conduct more clinical trials than we currently anticipate. Even if we do receive FDA or other regulatory agency approval, we may not be successful in commercializing approved product candidates. If any of these events occur, our business could be materially harmed and the value of our common stock would likely decline.

Because our product candidates are based on novel technologies, it is difficult to predict the time and cost of development and our ability to successfully complete clinical development of these product candidates and obtain the necessary regulatory approvals for commercialization.

Our product candidates are based on our novel HSC and SSC modulation platforms, and unexpected problems related to this new technology may arise that can cause us to delay, suspend or terminate our development efforts. Regulatory approval of novel product candidates such as ours can be more expensive and take longer than for other more well-known or extensively studied pharmaceutical or biopharmaceutical product candidates due to our and regulatory agencies lack of experience with them.

Stem cell therapies represent a relatively new therapeutic area, and the FDA has cautioned consumers about potential safety risks associated with these therapies. To date, there are relatively few approved stem cell products. In addition, there are currently no approved products in any major territory throughout the world with a label designation that supports the use of a product to improve multi-lineage engraftment or survival in patients undergoing HSCT, which makes it difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for ProHema or any of our other modulated HSC product candidates in the United States and elsewhere. Furthermore, the requirement that ProHema is manufactured at cell processing facilities in close proximity to transplant centers within a short period of time before transplantation may present unprecedented additional complexities associated with ensuring consistent manufacture across all sites, the degree of qualification testing necessary for cell-based therapies both pre- and post-administration, and ProHema s rapid release nature, all of which contribute to regulatory uncertainty.

Regulatory requirements governing cell therapy products have changed frequently and may continue to change in the future. For example, the FDA established the Office of Cellular, Tissue and Gene Therapies within its Center for Biologics Evaluation and Research, or CBER, to consolidate the review of gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review. In addition, adverse developments in clinical trials of potential stem cell therapies conducted by others may cause the FDA or other regulatory bodies to change the requirements for approval of any of our product candidates. These regulatory authorities and advisory groups and the new requirements or guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our product candidates or lead to significant post-approval limitations or restrictions. As we advance our product candidates, we will be required to consult with the FDA and other regulatory authorities, and our products will likely be reviewed by the FDA s advisory committee. We also must comply with applicable requirements, and if we fail to do so, we may be required to delay or discontinue development of our product candidates. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could impair our ability to generate sufficient product revenues to maintain our business.

Our clinical development of ProHema could be substantially delayed if the FDA requires us to conduct additional studies or trials or imposes other requirements or restrictions.

We initiated a Phase 2 clinical trial of ProHema, which we refer to as our ProHema-03 trial, in December 2012 and recently notified the FDA that we have elected to pause enrollment in our ProHema-03 trial to incorporate the use of our improved NRM formulation in the manufacture of ProHema for the trial. In August 2013, we submitted to the FDA preclinical and product development data to qualify the use of our NRM formulation in our ProHema manufacturing process and support that the use of the NRM formulation should not result in additional safety risks. In addition, we submitted an amended protocol for our ProHema-03 trial that defines how we will resume enrollment with ProHema as manufactured using our NRM formulation. Based on our recent communications with the FDA, we expect to resume enrollment in our ProHema-03 trial in the first

half of 2014, with the goal of generating full data in mid-2015. Before resuming enrollment in our ProHema-03 trial, we will need to generate, and provide the FDA with, product data for ProHema as manufactured using our NRM formulation with materials intended for clinical use. The FDA may also require us to generate additional preclinical or clinical data to support the use of our NRM formulation in our ProHema-03 trial or any subsequent clinical trials for ProHema that we may plan to conduct. Additionally, the FDA may impose other requirements on the protocol for our ProHema-03 trial or our other clinical trial protocols. These additional requirements may cause further delays in our ProHema-03 trial or other clinical development activities for ProHema, which could require us to incur additional development costs, seek funding for these increased costs or delay or cease our clinical development activities for ProHema. Any inability to advance ProHema or any other product candidate through clinical development would have a material adverse effect on our business.

We may be required to file new INDs to initiate clinical trials of ProHema for the treatment of hematologic malignancies and LSDs in pediatric patients.

We plan to conduct clinical trials of ProHema for hematopoietic reconstitution in both hematologic malignancies and LSDs in pediatric patients. We believe we can conduct these trials under our current IND for ProHema, and thus we may be able to file the appropriate amendments in order to commence these planned clinical trials. Although we currently believe that these amendments will suffice, we will need to submit clinical development plans to the FDA before we can commence these trials. The FDA may disagree with our plans and require us to file new INDs for the clinical evaluation of ProHema for the treatment of pediatric patients with hematologic malignancies and LSDs. If we are required to do so, this will delay our timeline, require additional resources and data and create uncertainty and additional complexity in our ability to obtain regulatory approval for these indications.

Our Wnt7a analogs are still in preclinical development, which may not be successful. If we are unable to successfully complete preclinical studies and clinical trials of our Wnt7a analogs, our business will be harmed.

Our Wnt7a analogs are still in preclinical development. To our knowledge, there are no Wnt proteins currently undergoing clinical development, primarily due to certain molecular characteristics that prevent their effective development as biologic therapeutics. Although we believe we are the first company to produce an analog of a Wnt protein that is amenable to manufacture, formulation and administration for *in vivo* therapeutic use, we may later encounter difficulties in manufacturing, formulating or administering of our Wnt7a analogs, or we may otherwise observe undesirable safety or efficacy profiles in these product candidates as our development activities progress. Subject to the completion of IND-enabling studies and our pre-IND meeting with the FDA, we plan to select a lead candidate, file an IND with the FDA and initiate a Phase 1 clinical trial of a Wnt7a analog. If the results of our ongoing or future preclinical studies or clinical trials are not positive, or the FDA does not allow us to proceed with clinical development for any reason, we may delay or cancel our planned clinical development activities for our Wnt7a analogs, which could materially harm our business.

We may face delays in completing our clinical trials, and we may not be able to complete them at all.

We have not completed the clinical trials necessary to support an application for approval to market any of our product candidates. Our current and future clinical trials of ProHema and our other product candidates may be delayed, unsuccessful or terminated as a result of many factors, including:

- delays in our ability to resume enrollment in our ProHema-03 trial;
- the introduction of our NRM formulation into our ProHema-03 trial may not produce the benefits that we currently anticipate or may result in unanticipated adverse effects;
- delays in designing appropriate clinical trial protocols and reaching agreement on trial design with investigators and regulatory authorities;
- delays or failure in reaching agreement on acceptable clinical trial contracts or protocols with prospective sites;

governmental or regulatory delays, failure to obtain regulatory approval or changes in regulatory requirements, policy or guidelines;

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reaching agreement on acceptable terms with third-party service providers to manage various aspects of our clinical trials, the terms of which can be subject to extensive negotiation and may vary significantly among different service providers and trial sites;

the actual performance of third-party service providers and clinical trial sites in ensuring the proper and timely conduct of our clinical trials;

the ability of clinical trial sites to manufacture ProHema consistently under the correct conditions at their on-site cell processing facilities for use in our clinical trials;

delays in achieving study endpoints and completing data analysis for a trial;

regulators or institutional review boards, or IRBs, may not authorize us to commence or recommence a clinical trial;

data safety monitoring committees may recommend suspension, termination or a clinical hold for various reasons, including concerns about patient safety;

regulators or IRBs may suspend or terminate the trial or impose a clinical hold for various reasons, including noncompliance with regulatory requirements or concerns about patient safety;

patients with serious, life-threatening diseases included in our clinical trials may die or suffer other adverse medical events for reasons that may not be related to our product candidates;

participating patients may be subject to unacceptable health risks;

patients may not complete clinical trials due to safety issues, side effects, or other reasons;

our product candidates may demonstrate a lack of safety or efficacy during clinical trials; and

changes in regulatory requirements and guidance may occur, which require us to amend clinical trial protocols to reflect these changes.

The FDA has indicated that we will need to standardize the process for manufacturing ProHema across the multiple cell processing facilities at the clinical sites participating in our trials, and any delays in, or inability to, establish manufacturing protocols acceptable to the FDA will result in further delays to our clinical development plans. Any such events would increase our costs and could delay or prevent our ability to commercialize our product candidates, which could adversely impact our business, financial condition and results of operations.

Moreover, our development costs will increase because we will be required to complete additional or larger clinical trials for product candidates from our HSC and SSC modulation platforms prior to FDA or other regulatory approval. We may not have adequate capital or other resources to commence or complete these additional or larger trials. If we experience delays in the completion of, or termination of, any clinical trial of our product candidates, the commercial prospects of our product candidates will be harmed. In addition, any delays in commencing or completing our clinical trials will increase our costs, slow down our product candidate development and approval process, and jeopardize our ability to commence product sales and generate revenues. 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. Any of these occurrences may significantly harm our business, financial condition and prospects.

If we encounter difficulties enrolling patients in our clinical trials, our clinical development activities could be delayed or otherwise adversely affected.

We will be required to identify and enroll a sufficient number of patients with the disease under investigation for each of our ongoing and planned clinical trials of our product candidates. Each indication for which we plan to evaluate our current product candidates represents a rare disease or condition with limited patient populations from which to draw participants in clinical trials. Due to our focus on the development of product candidates for the treatment of orphan hematologic malignancies, rare genetic diseases and muscular dystrophies, we may not be able to identify and enroll a sufficient number of patients, or those with required or desired characteristics and criteria, in a timely manner. In addition, there are currently only a limited number of specialized transplant centers that perform

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HSCTs, and among physicians who perform HSCTs, some may not choose to perform these procedures under conditions that fall within our protocols, which would have an adverse effect on our development of ProHema. Our ability to enroll patients in our clinical trials is affected by factors including:

- severity of the disease under investigation;
- design of the trial protocol;
- the relatively small size and nature of the patient population;
- eligibility criteria for the trial in question;
- perceived risks and benefits of the product candidate under study;
- availability of competing therapies and clinical trials;
- efforts to facilitate timely enrollment in clinical trials;
- the availability of time and resources at the limited number of institutions at which clinical trials are and will be conducted;
- the ability to identify, solicit and recruit a sufficient number of patients;
- the ability to monitor patients adequately during and after treatment; and
- the proximity and availability of clinical trial sites for prospective patients.

If we have difficulty enrolling a sufficient number of patients to conduct our clinical trials as planned, we may need to delay or terminate ongoing or planned clinical trials, either of which would have an adverse effect on our business.

Results from preclinical studies and earlier clinical trials do not ensure that future clinical trials will be successful.

All of our product candidates are still in an early stage of development, and we cannot be assured that these trials will ultimately be successful. For example, although the results of our Phase 1b clinical trial of ProHema in adults with hematologic malignancies undergoing double umbilical cord blood transplant demonstrated human proof-of-concept, we may not achieve or duplicate these results in our ProHema-03 trial or planned additional clinical trials of ProHema for a variety of reasons, including:

- the anticipated use of our NRM formulation may not produce the potency and efficacy benefits observed in preclinical studies, or may result in unanticipated side effects in comparison to the standard processing media used in our Phase 1b clinical trial;

the increase in the number of patients enrolled in later-stage trials may not produce the same or similar results as earlier trials with fewer patients;

the expansion in the number of participating clinical centers and the variabilities among the centers may result in complexities in conducting clinical trials, which we may be unable to manage adequately;

we may be unable to ensure the consistent manufacture of ProHema, which is required to be manufactured at cell processing facilities at each clinical center, across all participating clinical centers once we resume our ProHema-03 trial or in any future clinical trials that we may conduct;

differences in the design of the Phase 2 clinical trial, such as additional conditioning regimens, expanded eligibility criteria, potential patient population changes based on additional clinical centers that are more geographically dispersed, the addition of a concurrent control arm and our efforts to standardize and automate our ProHema manufacturing process to make it acceptable to FDA for entry into Phase 2 clinical trials may make it less effective than the product manufactured during our Phase 1 trial or otherwise adversely affect ProHema; and

we have not previously evaluated ProHema in pediatric patients, and pediatric patients may experience side effects or other adverse events not observed in adult patients.

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Additionally, because our Wnt7a analogs are still in preclinical development, we cannot assure you that any product candidates selected from our SSC modulation platform will demonstrate the safety, purity and potency profile necessary to support further clinical development or regulatory approval.

A number of companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in later stage clinical trials even after achieving promising results in earlier stages of development. Success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful, and initial results from a clinical trial do not necessarily predict final results. Even if early stage clinical trials are successful, we may need to conduct additional clinical trials for product candidates in additional patient populations or under different treatment conditions before we are able to seek approvals from the FDA and regulatory authorities outside the United States to market and sell these product candidates. Our failure to demonstrate the required characteristics to support marketing approval for our product candidates in our planned and future clinical trials would substantially harm our business and prospects.

Our planned clinical development activities for ProHema present operational, technical and timing issues related to pediatric clinical trials.

Many clinical centers that could potentially participate in our pediatric clinical trials of ProHema are distinct and separate from the centers participating in our adult trials, and finding sufficient, capable centers that would be interested in participating in our pediatric trials may take additional time. There will be fewer eligible patients with hematologic malignancies and rare genetic disorders for our planned clinical trials in pediatric patients because the total number of pediatric patients with such diseases and disorders is lower than it is in adults. This may increase the time to enroll our planned pediatric clinical trials in hematologic malignancies and rare genetic disorders and could also further limit our ability to enroll patients in our planned Phase 1 clinical trial of ProHema in pediatric patients.

As we have not previously evaluated ProHema in pediatric patients, we will have to modify the current formulation of ProHema to one that is suitable for children, due to their smaller size and requirement for smaller infusion volume. Such a modification will require an amendment to our current IND or the filing of a new IND and may present technical challenges and may cause further delays in our planned clinical trial. In addition, any such IND amendment or new IND will require FDA review and approval of our modified formulation of ProHema for children. The FDA will need to review and approve our specific clinical plans in pediatric hematologic malignancies and rare genetic disorders, and may present additional requirements, including additional data in adult patients, before we can proceed with our planned pediatric clinical trials. Any delays in our planned clinical development activities for pediatric patients could have an adverse effect on our business operations.

We expect to rely heavily on orphan drug status to develop and commercialize our product candidates, but our orphan drug designations may not confer marketing exclusivity or other expected commercial benefits.

We expect to rely heavily on orphan drug exclusivity for our product candidates, including ProHema. Orphan drug status confers seven years of marketing exclusivity under the Federal Food, Drug, and Cosmetic Act, and up to ten years of marketing exclusivity in Europe for a particular product in a specified indication. We have been granted orphan drug designation in the United States for human allogeneic HSCs *ex vivo* modulated with FT1050 for the enhancement of stem cell engraftment and in the European Union for ProHema for the treatment of acute myelogenous leukemia through the *ex vivo* modulation of allogeneic umbilical cord blood cells. While we have been granted these orphan designations, we will not be able to rely on these designations to exclude other companies from manufacturing or selling biological products using the same principal molecular structural features for the same indication beyond these timeframes. Furthermore, the marketing exclusivity in Europe can be reduced from ten years to six years if the initial designation criteria have significantly changed since the market authorization of the orphan product.

For any product candidate for which we have been granted orphan drug designation in a particular indication, it is possible that another company also holding orphan drug designation for the same product candidate will receive marketing approval for the same indication before we do. If that were to happen, our applications for that indication may not be approved until the competing company s period of exclusivity expires. Even if we are the first to obtain

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marketing authorization for an orphan drug indication, there are circumstances under which a competing product may be approved for the same indication during the seven-year period of marketing exclusivity, such as if the later product is shown to be clinically superior to the orphan product, or if the later product is deemed a different product than ours. Further, the seven-year marketing exclusivity would not prevent competitors from obtaining approval of the same product candidate as ours for indications other than those in which we have been granted orphan drug designation, or for the use of other types of products in the same indications as our orphan product.

Even if we obtain regulatory approval for a product candidate, our products will remain subject to regulatory scrutiny.

Any product candidate for which we obtain marketing approval, along with the manufacturing processes, qualification testing, post-approval clinical data, labeling and promotional activities for such product, will be subject to continual and additional requirements of the FDA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information, reports, registration and listing requirements, good manufacturing practices, or GMP requirements relating to quality control, quality assurance and corresponding maintenance of records and documents, and recordkeeping. Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the product may be marketed or to conditions of approval, or contain requirements for costly post-marketing testing and surveillance to monitor the safety or efficacy of the product. The FDA closely regulates the post-approval marketing and promotion of pharmaceutical and biological products to ensure such products are marketed only for the approved indications and in accordance with the provisions of the approved labeling.

In addition, later discovery of previously unknown problems with our products, manufacturing processes, or failure to comply with regulatory requirements, may lead to various adverse results, including:

- restrictions on such products, manufacturers or manufacturing processes;
- restrictions on the labeling or marketing of a product;
- restrictions on product distribution or use;
- requirements to conduct post-marketing clinical trials;
- requirements to institute a risk evaluation and mitigation strategy, or REMS, to monitor safety of the product post-approval;
- requirements to individually license clinical cell processing facilities for the manufacture of ProHema;
- warning letters issued by the FDA or other regulatory authorities;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products, fines, restitution or disgorgement of profits or revenue;

suspension, revocation or withdrawal of marketing approvals;

refusal to permit the import or export of our products; and

injunctions or the imposition of civil or criminal penalties.

The FDA s policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained, which would adversely affect our business, prospects and ability to achieve or sustain profitability.

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We may be subject to certain regulations, including federal and state healthcare fraud and abuse laws and health information privacy and security laws. Any failure to comply with these regulations could have a material adverse effect on our business and financial condition.

If we obtain FDA approval for any of our product candidates and begin commercializing those products in the United States, our operations may be subject to various federal and state fraud and abuse laws, including, without limitation, the federal anti-kickback statute. 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 laws that may affect our ability to operate include:

- 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 impose certain requirements relating to the privacy, security and transmission of individually identifiable health information; and
- state law equivalents of each of the above federal laws, such as anti-kickback and false claims laws which may apply to items or services reimbursed by any third-party payer, including commercial insurers, and 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.

Because of the breadth of these laws and the narrowness of the safe harbors, it is possible that some of our business activities could be subject to challenge under one or more of these laws. If our operations are found to be in violation of any of the laws described above or any other governmental regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines 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.

We may not elect or be able to take advantage of any expedited development or regulatory review and approval processes available to product candidates granted breakthrough therapy or fast track designation by the FDA.

We are evaluating the possibility of seeking breakthrough therapy or fast track designation for some of our product candidates, although we may elect not to do so. A breakthrough therapy program is for a product candidate intended to treat a serious or life-threatening condition, and preliminary clinical evidence indicates that the product candidate may demonstrate substantial improvement on a clinically significant endpoint(s) over available therapies. In contrast, a fast track program is for a product candidate that treats a serious or life-threatening condition, and nonclinical or clinical data demonstrate the potential to address an unmet medical need. Although we believe some of our product candidates may qualify under either or both of the breakthrough therapy and fast track programs, we may elect not to pursue either of these programs, and the FDA has broad discretion whether or not to grant these designations. Accordingly, even if we believe a particular product candidate is eligible for breakthrough therapy or fast track designation, we cannot assure you that the FDA would decide to grant it. Even if we do receive breakthrough therapy or fast track designation, we may not experience a faster development process, review or approval compared to conventional FDA procedures. The FDA may withdraw breakthrough therapy or fast track designation if it believes that the product no longer meets the qualifying criteria. In addition, the breakthrough therapy program is a relatively new program. As a result, we cannot be certain whether any of our product candidates can or will qualify for breakthrough therapy designation. Our business may be harmed if we are unable to avail ourselves of these or any other expedited development and regulatory pathways.

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Risks Related to Our Reliance on Third Parties

We depend on third-party suppliers for various components of our improved NRM formulation for the manufacture of ProHema and do not have supply arrangements for certain of these components.

We currently rely, and expect to continue to rely, on third-party suppliers for components that enable us to develop and use our NRM formulation for the manufacture of ProHema in our Phase 2 clinical trial and any subsequent clinical trials. We have not entered into supply agreements for certain of the components necessary to produce our NRM formulation and may not be able to obtain clinical supply agreements that provide for an adequate and reliable supply of these components to complete our Phase 2 clinical trial or commence any future clinical trials. Even if we are able to secure such clinical supply agreements, we may be limited to a sole manufacturer for certain of the components required in our NRM formulation. Accordingly, we cannot guarantee that we will have an adequate supply of NRM to complete our Phase 2 clinical trial of ProHema as currently contemplated or to complete a Phase 3 clinical trial or any other future clinical trials. In addition, if we are unable to secure adequate quantities of these components from our preferred suppliers, we may be required to identify alternate suppliers of these components, or to modify our NRM formulation. If we are required to change suppliers of our components, or modify our NRM formulation, the efficacy and potency of ProHema may be adversely affected. We also may be required to make further changes to our trial protocol or provide additional data to the FDA regarding the use of alternative components for our NRM formulation or a modified NRM formulation, which could delay our clinical development plans for ProHema and increase the costs required to complete our Phase 2 clinical trial of ProHema or any other clinical trials.

We rely on a third-party supplier for the FT1050 starting material required for the manufacture of ProHema.

To date, we have obtained our supplies of FT1050 for the manufacture of ProHema in our preclinical studies and clinical trials from a single third-party manufacturer. This manufacturer supplies FT1050 to us for our clinical trials on a purchase order basis under a clinical supply manufacturing agreement, and we do not have any current contractual relationships for the commercial manufacture and supply of bulk FT1050 substance for manufacturing ProHema. Although we believe that we would be able to identify several alternate suppliers for FT1050 should our current third-party manufacturer become unavailable to us for any reason, we may incur delays associated with identifying and qualifying a replacement supplier and negotiating the terms of any supply contracts with the replacement supplier. These delays could adversely impact our clinical development plans and harm our business.

We face a variety of challenges and uncertainties associated with our dependence on the availability of human umbilical cord blood units, or CBUs, at cord blood banks for the manufacture of ProHema.

CBUs are one of the raw materials for the manufacture of ProHema. The CBUs currently used in the manufacture of ProHema are procured directly by the clinical cell processing facilities from cord blood banks. The availability of CBUs for the manufacture of ProHema depends on a number of regulatory, political, economic and technical factors outside of our control, including:

- government policies relating to the regulation of CBUs for clinical use;
- the availability of government funding for cord blood banks;
- individual cord blood bank policies and practices relating to CBU acquisition and banking;
- the pricing of CBUs;
- the methods used in searching for and matching CBUs to patients, which involve emerging technology related to current and future CBU parameters that guide the selection of an appropriate CBU for transplantation; and

methods for the procurement and shipment of CBUs and their handling and storage at clinical sites. Additionally, we do not have control over the supply, availability, price or types of CBUs that these third parties use in the manufacture of ProHema. We rely heavily on these clinical cell processing facilities to procure

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CBUs from cord blood banks that are compliant with government regulations and within the current standard of care. In addition, we may identify specific characteristics of CBUs, such as their volume and red blood cell content, that may limit their ability to be used to manufacture ProHema even though these CBUs may otherwise be suitable for use in allogeneic transplant. As a result, the requirement for CBUs to meet our specifications may limit the potential inventory of CBUs eligible for use in the manufacture of ProHema.

In the United States, cord blood banks are required to file a biologics license application, or BLA, and to meet certain continued regulatory requirements, in order to bank and provide CBUs for transplantation. CBUs from a cord blood bank that maintains a BLA are considered to be licensed and have a product label describing their intended use. While the FDA currently allows unlicensed CBUs to be used for transplantation and we have only used unlicensed CBUs in the manufacture of ProHema for our clinical trials, the FDA may later prohibit the use of unlicensed CBUs for transplantation. Additionally, although CBUs from foreign cord blood banks, which are generally unlicensed, are currently available in the United States for use in transplantation and we have used CBUs from foreign cord blood banks in our clinical trials, changes in U.S. and foreign regulations may prohibit or limit the future use of foreign CBUs in the United States. Any inability to procure adequate supplies of CBUs will adversely impact our ability to develop and commercialize ProHema.

We depend on facilities operated by transplant centers for the manufacture of ProHema under specific conditions. Any failure by these facilities to manufacture ProHema consistently and under the proper conditions may result in delays to our clinical development plans and impair our ability to commercialize ProHema, if approved.

ProHema is currently manufactured at clinical cell processing facilities operated by or affiliated with our clinical sites and is required to be manufactured in close proximity to the treatment site on the same day as product administration. The FDA has stated that we will be required to standardize the manufacture of ProHema to maximize consistency across the multiple clinical cell processing facilities, including our oversight for facility and raw material and vendor qualification through to final product analytical testing and release. Although we are responsible for ensuring compliance with GMPs and for overseeing all aspects of product manufacture and release prior to applying for marketing approval, we do not control the activities of these third-party cell processing facilities. The clinical cell processing facilities at which ProHema is manufactured must be approved by applicable regulatory authorities, including the FDA, pursuant to inspections that will be conducted after a BLA, or comparable foreign regulatory marketing application is submitted. We do not control the manufacturing process for ProHema and are completely dependent on third parties for compliance with the FDA s requirements and proper execution of the protocol for the manufacture of ProHema. Because of the requirement that ProHema be manufactured in close proximity to the transplant center within a short period of time before transplant, if the applicable clinical cell processing facilities are unable to manufacture ProHema in a manner that conforms to our specifications and the FDA s strict regulatory requirements, we will not be able to secure backup manufacturing capabilities. For example, to comply with GMPs and other regulatory requirements and our manufacturing protocols, the clinical cell processing facility may be required to possess or obtain certain equipment necessary for the manufacture of ProHema including but not limited to biosafety cabinets, warming devices, cell washing devices, freezers or other materials; or to modify aspects of its operations, including its physical facility or layout, environmental systems, monitoring systems, quality systems or training procedures, for the compliant manufacture of ProHema. Clinical cell processing facilities may be unwilling or unable to comply with these requirements, which could restrict or prohibit a given clinical center from manufacturing ProHema and making it available for administration to patients within the required timeframes. Any failure by these clinical cell processing facilities to properly manufacture ProHema will have an adverse impact on our business.

We will be substantially dependent on third parties for the manufacture of our clinical supplies of our Wnt7a analogs.

We expect to rely on third-party manufacturers for clinical supplies of our Wnt7a analogs and other product candidates that we may develop. These third-party manufacturers will be required to comply with current GMPs and other applicable laws and regulations. We will have no control over the ability of these third parties to

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comply with these requirements, or to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or any other applicable regulatory authorities do not approve the facilities of these third parties for the manufacture of our other product candidates or any products that we may successfully develop, or if it withdraws any such approval, or if our suppliers or contract manufacturers decide they no longer want to supply or manufacture for us, we may need to find alternative manufacturing facilities, in which case we might not be able to identify manufacturers for clinical or commercial supply on acceptable terms, or at all. In addition, Wnt proteins have specific molecular characteristics that make their manufacture for therapeutic application difficult, and it is possible that any third-party manufacturers that we engage may experience difficulties in such manufacture. Any of these factors would significantly impact our ability to develop, obtain regulatory approval for or market our Wnt7a analogs and adversely affect our business.

We currently rely on third parties to support the conduct of 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 relied upon and plan to continue to rely upon third parties to monitor and manage data for our ongoing clinical programs. We rely on these parties for execution of our clinical trials, and control only certain aspects of their activities. Nevertheless, we are responsible for ensuring that each of our studies is conducted in accordance with the applicable protocol and legal, regulatory and scientific standards, and our reliance on third parties does not relieve us of our regulatory responsibilities. We also rely on third parties to assist in conducting our nonclinical studies in accordance with good laboratory practices, or GLP. We and our third-party service providers are required to comply with good clinical practices, or GCP, which are regulations and guidelines enforced by the FDA, as well as comparable foreign regulations and guidelines, for all of our product candidates in clinical development. Regulatory authorities enforce these GCP through periodic inspections of trial sponsors, principal investigators and trial sites. If we or any of our third-party service providers or clinical trial sites fail to comply with applicable GCP, 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 by a given regulatory authority, such regulatory authority will determine that any of our clinical trials comply with GCP requirements. In addition, our clinical trials must be conducted with product produced under applicable GMP requirements. Failure to comply with these regulations may require us to repeat nonclinical and clinical trials, which would delay the regulatory approval process.

Our third-party service providers are not our employees, and except for remedies available to us under our agreements with such third parties, we cannot control whether or not they devote sufficient time and resources to our on-going clinical and nonclinical programs. If third-party service providers do not successfully carry out their contractual duties or obligations or meet expected deadlines or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols, regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated and we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. As a result, our results of operations and the commercial prospects for our product candidates would be harmed, our costs could increase and our ability to generate revenues could be delayed.

Because we have relied on third parties, our internal capacity to perform these functions is limited. Outsourcing these functions involves risk that third parties may not perform to our standards, may not produce results in a timely manner or may fail to perform at all. In addition, the use of third-party service providers requires us to disclose our proprietary information to these parties, which could increase the risk that this information will be misappropriated. We currently have a small number of employees, which limits the internal resources we have available to identify and monitor our third-party service providers. To the extent we are unable to identify and successfully manage the performance of third-party service providers in the future, our business may be adversely affected. Although we carefully manage our relationships with our third-party service providers, there can be no assurance that we will not encounter similar challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition and prospects.

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If we lose our relationships with our third-party service providers, our product development efforts could be delayed.

We rely on third-party service providers for clinical trials related to our product development efforts. Switching or adding additional third-party service providers involves additional cost and timing considerations and requires management time and focus. Some of our third-party service providers have the right to terminate their agreements with us in the event of an uncured material breach. In addition, some of our third-party service providers have an ability to terminate their respective agreements with us if it can be reasonably demonstrated that the safety of the subjects participating in our clinical trials warrants such termination, if we make a general assignment for the benefit of our creditors or if we are liquidated. Identifying, qualifying and managing performance of third-party service providers can be difficult, time consuming and cause delays in our development programs. In addition, there is a natural transition period when a new service provider commences work and the new provider may not provide the same type or level of services as the original provider. If any of our relationships with our third-party service providers terminate, we may not be able to enter into arrangements with alternative service providers or to do so on commercially reasonable terms.

Risks Related to Our Intellectual Property

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

Our commercial success will depend in part on obtaining and maintaining patent protection and trade secret protection of our current and future product candidates, the processes used to manufacture them and the methods for using them, as well as successfully defending these patents against third-party challenges. We are currently the owner of record of 14 patent applications pending in the United States and in certain foreign jurisdictions relating to ProHema and other therapeutic compositions of stem cells that have been pharmacologically modulated to enhance their therapeutic properties, and methods of manufacturing the cellular compositions. In addition, we currently own eight patent applications pending in the United States and in certain foreign jurisdictions relating to our Wnt analogs, covering compositions of matter, processes for preparing such Wnt proteins and formulations, and the modulation of SSCs. To date, no patents have been issued to us specifically covering our product candidates, and we cannot be certain that any patents will issue with claims that cover our ProHema and Wnt product candidates.

Our ability to stop third parties from making, using, selling, offering to sell or importing our product candidates is dependent upon the extent to which we have rights under valid and enforceable patents or trade secrets that cover these activities.

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

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

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

- others may be able to develop a platform similar to, or better than, ours in a way that is not covered by the claims of our patents;
- others may be able to make therapeutics or compounds that are similar to our product candidates, but that are not covered by the claims of our patents;
- we might not have been the first to make the inventions covered by our pending patent applications;
- we might not have been the first to file patent applications for these inventions;
- our pending patent applications may not result in issued patents;
- the claims of our issued patents or patent applications when issued may not cover our products or product candidates;
- others may independently develop similar or alternative technologies or duplicate any of our technologies;
- any patents that we obtain may not provide us with any competitive advantages;
- any granted patents may be held invalid or unenforceable as a result of legal challenges by third parties;
- we may not develop additional proprietary technologies that are patentable; or
- the patents of others may have an adverse effect on our business.

We depend on our licensors to prosecute and maintain patents and patent applications that are material to our business. Any failure by our licensors to effectively protect these intellectual property rights could adversely impact our business and operations.

We are currently the exclusive licensee of 45 issued or pending U.S. and non-U.S. patents or patent applications relating to ProHema and other therapeutic compositions of stem cells that have been pharmacologically modulated to enhance their therapeutic properties, methods of manufacturing the cellular compositions, and methods of promoting hematopoietic reconstitution, expansion and self-renewal using modulators that increase prostaglandin signaling activity.

We currently have exclusive licenses to 18 patents and patent applications relating to our Wnt analogs, covering compositions of matter, processes for preparing such Wnt proteins and formulations, and the modulation of SSCs.

As a licensee of third parties, we rely on these third parties to file and prosecute patent applications and maintain patents and otherwise protect the licensed intellectual property under some of our license agreements. We have not had and do not have primary control over these activities for certain of our patents or patent applications and other intellectual property rights. We cannot be certain that such activities by third parties have been or will be conducted in compliance with applicable laws and regulations or will result in valid and enforceable patents and other intellectual property rights. Pursuant to the terms of the license agreements with some of our licensors, the licensors may have the right to

control enforcement of our licensed patents or defense of any claims asserting the invalidity of these patents and even if we are permitted to pursue such enforcement or defense, we will require the cooperation of our licensors. We cannot be certain that our licensors will allocate sufficient resources or prioritize their or our enforcement of such patents or defense of such claims to protect our interests in the licensed patents. Even if we are not a party to these legal actions, an adverse outcome could harm our business because it might prevent us from continuing to license intellectual property that we may need to operate our business.

Due to the patent laws of a country, or the decisions of a patent examiner in a country, or our own filing strategies, we may not obtain patent coverage for all of our product candidates or methods involving these candidates in the parent patent application.

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

We are a party to a number of intellectual property license agreements that are important to our business and expect to enter into additional license agreements in the future. Our existing license agreements impose, and we expect that future license agreements will impose, various diligence, milestone payment, royalty and other obligations on us. For example, under our exclusive license agreements with various universities and research institutions, we are required to use commercially reasonable efforts to engage in various development and commercialization activities with respect to licensed products, and must satisfy specified milestone and royalty payment obligations. If we fail to comply with our obligations under our agreements with any of these licensors, we may be subject to termination of the license agreement in whole or in part; increased financial obligations to our licensors or loss of exclusivity in a particular field or territory, in which case our ability to develop or commercialize products covered by the license agreement will be impaired. Additionally, we may be subject to royalty obligations to multiple licensors with respect to the same product. Our material license agreements are described in greater detail in the Business Our Material Technology License Agreements section.

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

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

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

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

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

If we choose to go to court to stop another party from using the inventions claimed in any patents we obtain, that individual or company has the right to ask the court to rule that such patents are invalid or should not be enforced against that third party. These lawsuits are expensive and would consume time and resources and divert the attention of managerial and scientific personnel even if we were successful in stopping the infringement of such patents. In addition, there is a risk that the court will decide that such patents are not valid and that we do not have the right to stop the other party from using the inventions. There is also the risk that, even if the validity of such patents is upheld, the court will refuse to stop the other party on the ground that such other party s activities do not infringe our rights to such patents. In addition, the U.S. Supreme Court has recently modified some tests used by the U.S. Patent and Trademark Office, or USPTO, in granting patents over the past 20 years, which may decrease the likelihood that we will be able to obtain patents and increase the likelihood of challenge of any patents we obtain or license.

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

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

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

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

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

Our competitors may have filed, and may in the future file, patent applications covering technology similar to ours. Any such patent application may have priority over our patent applications, which could further require us to obtain rights to issued patents covering such technologies. If another party has filed a U.S. patent application on inventions similar to ours that claims priority to an application filed prior to March 16, 2013, we may have to participate in an interference proceeding declared by the USPTO to determine priority of invention in the United States. The costs of these proceedings could be substantial, and it is possible that such efforts would be unsuccessful if, unbeknownst to us, the other party had independently arrived at the same or similar invention prior to our own invention, resulting in a loss of our U.S. patent position with respect to such inventions. In addition, changes enacted on March 15, 2013 to the U.S. patent laws resulted in the United States changing from a first to invent country to a first to file country. As a result, we may lose the ability to obtain issued patent if a third party files with the patent office first. Other countries have similar laws that permit secrecy of patent applications, and may be entitled to priority over our applications in such jurisdictions.

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

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

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

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

As is common in the biotechnology and pharmaceutical industries, we employ individuals who were previously employed at other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and independent contractors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed trade secrets or other proprietary information of their former employers. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we could lose valuable intellectual property rights or personnel, which could adversely impact our business. Even if we are successful in defending against these claims, litigation could result in substantial costs and be a distraction to management.

Risks Related to the Commercialization of Our Product Candidates

If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell our product candidates, we may be unable to generate any revenues.

We have no experience selling and marketing any products. To successfully commercialize any products that may result from our development programs, we will need to develop these capabilities, either on our own or with others. If any of our initial product candidates are approved for marketing, we intend to build an internal sales and marketing organization to commercialize these products in highly specialized target markets, where patient and physician communities are concentrated and product adoption is driven by key opinion leaders. However, we may not have adequate financial resources or expertise to build an effective sales and marketing organization.

We may selectively seek to enter into partnerships with other entities to utilize their marketing and distribution capabilities in larger target markets, but we may be unable to enter into these arrangements on favorable terms, if at all. If we are unable to develop adequate marketing capabilities on our own or effectively partner with third parties, we will be unable to generate revenues from our approved products. We will be

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competing with many companies that currently have extensive and well-funded marketing and sales operations. Without an internal team or the support of a third party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies.

The commercial success of any current or future product candidates will depend upon the degree of market acceptance by physicians, patients, third-party payers and others in the medical community.

Ethical, social and legal concerns about stem cell therapies could result in additional regulations restricting or prohibiting the use of our product candidates. Even with the requisite approvals, the commercial success of our product candidates will depend in part on the medical community, patients and third-party payers accepting stem cell therapies in general, and our product candidates in particular, as medically useful, cost-effective and safe. Any product that we bring to the market may not gain market acceptance by physicians, patients, third-party payers and others in the medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenue and may not become profitable. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on a number of factors, including:

- the potential efficacy and other advantages over alternative treatments;
- the prevalence and severity of any side effects, including any limitations or warnings contained in a product s approved labeling;
- the prevalence and severity of any side effects resulting from the chemotherapy and myeloablative treatments associated with the procedure by which ProHema or any other HSC therapeutics that we may develop are administered;
- relative convenience and ease of administration;
- the willingness of the target patient population to try new therapeutics and of physicians to prescribe these therapeutics;
- the strength of marketing and distribution support and timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments; and
- sufficient third-party insurance coverage or reimbursement.

Even if a potential product displays a favorable efficacy and safety profile in preclinical studies and clinical trials, market acceptance of the product will not be known until after it is launched. Our efforts to educate the medical community and third-party payers on the benefits of our product candidates may require significant resources and may never be successful. Any failure to achieve market acceptance for our product candidates will harm our business, results and financial condition.

If we obtain approval to commercialize our product candidates outside of the United States, a variety of risks associated with international operations could materially adversely affect our business.

If any of our product candidates are approved for commercialization, we may enter into agreements with third parties to market them on a worldwide basis or in more limited geographical regions. We expect that we will be subject to additional risks related to entering into international business relationships, including:

different regulatory requirements for approval of drugs and biologics in foreign countries;

reduced or uncertain protection for our intellectual property rights;

unexpected changes in tariffs, trade barriers and regulatory requirements;

economic weakness, including inflation, or political instability in particular foreign economies and markets;

compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;

foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;

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workforce uncertainty in countries where labor unrest is more common than in the United States;

complexity and difficulty in coordinating the communications and operations of our business; and

production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad.

We expect to face uncertainty regarding the pricing of ProHema and any other product candidates that we may develop. If pricing policies for our product candidates are unfavorable, our commercial success will be impaired.

Due to the targeted indication of HSCT procedures in general and our HSC product candidates in particular, we face significant uncertainty as to the pricing of any such products for which we may receive marketing approval. While we anticipate that pricing for these candidates will be relatively high due to their anticipated use in a one-time, potentially life-saving procedure with curative intent, the biopharmaceutical industry has recently experienced significant pricing pressures, including in the area of orphan drug products. Additionally, because our target patient populations are relatively small, the pricing and reimbursement of our product candidates, if approved, must be adequate to support commercial infrastructure. In addition, there are currently no approved products for the treatment of muscular dystrophies, and it is difficult to predict the level of reimbursement, if any, that would be available for any product candidates that we may develop in these indications. If pricing is set at unsatisfactory levels, our ability to successfully market and sell our product candidates will be adversely affected.

We may experience pricing pressures in connection with the sale of any of our product candidates due to the trend toward managed healthcare, the increasing influence of health maintenance organizations and additional legislative changes. The downward pressure on healthcare costs in general, particularly in the area of orphan drug products, has become very intense. These pricing pressures have imposed significant barriers to the entry of new products.

Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost-containment initiatives in Europe, Canada and other countries has and will continue to put pressure on the pricing and usage of our product candidates. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. In general, the prices of medicines under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for medicines, but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates, which may adversely affect our ability to generate profit from the sales of our product candidates.

The insurance coverage and reimbursement status of newly-approved products is uncertain. Failure to obtain or maintain adequate coverage and reimbursement for new products could limit our ability to market any product that we may develop and decrease our ability to generate revenue.

The availability and extent of reimbursement by governmental and private payers is essential for most patients to be able to afford expensive treatments, such as stem cell transplants. Sales of our product candidates will depend substantially, both domestically and abroad, on the extent to which the costs of our product candidates will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or reimbursed by government health administration authorities, private health coverage insurers and other third-party payers. There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products. In particular, there is no body of established practices and precedents for reimbursement of modulated stem cell products, and it is difficult to predict what the regulatory authority or private payer will decide with respect to reimbursement levels for novel products such as ours. Our products may not qualify for coverage or direct reimbursement and may be subject to limited reimbursement. Stem cell transplant procedures are typically covered by one-time reimbursement, generally available for a limited number of days after transplant. If reimbursement or insurance coverage is not available, or is available only to limited levels, we may not be able to successfully commercialize our product candidates. Even if coverage is provided, the approved reimbursement amount may not be sufficient to allow us to establish or maintain pricing to generate income.

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In addition, reimbursement agencies in foreign jurisdictions may be more conservative than those in the United States. Accordingly, in markets outside the United States, the reimbursement for our products may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenues and profits. Moreover, increasing efforts by governmental and third-party payers, in the United States and abroad, to cap or reduce healthcare costs may cause such organizations to limit both coverage and level of reimbursement for new products approved and, as a result, they may not cover or provide adequate payment for our product candidates. Failure to obtain or maintain adequate reimbursement for any products for which we receive marketing approval will adversely impact our ability to achieve commercial success.

If the market opportunities for our product candidates are smaller than we believe they are, our revenues may be adversely affected and our business may suffer. Because the target patient populations of our product candidates are small, we must be able to successfully identify patients and capture a significant market share to achieve and maintain profitability.

We focus our research and product development on treatments for orphan hematologic malignancies, rare genetic disorders and muscular dystrophies. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are based on estimates. These estimates may prove to be incorrect and new studies may change the estimated incidence or prevalence of these diseases. The number of patients in the United States, Europe and elsewhere may turn out to be lower than expected or may not be otherwise amenable to treatment with our products, or new patients may become increasingly difficult to identify or gain access to, all of which would adversely affect our results of operations and our business. Additionally, because our target patient populations are small, we will be required to capture a significant market share to achieve and maintain profitability.

Risks Related to Our Business and Industry

The success of our HSC and SSC modulation platforms and our product candidates is substantially dependent on developments within the emerging field of stem cell therapies, some of which are beyond our control.

Our HSC and SSC modulation platforms and our product candidates are designed to optimize the biological activity of adult stem cells, which represents a novel development within the field of stem cell therapeutics. Stem cell therapies in turn represent a relatively new therapeutic area that presents a number of scientific, clinical, regulatory and ethical challenges. Any adverse developments in the field of stem cell therapeutics generally, and in the practice of HSCT in particular, will negatively impact our ability to develop and commercialize our product candidates. In particular, we currently anticipate that ProHema and any additional product candidates that we develop from our HSC modulation platform would be adopted into the current standard of care for HSCT procedures. If the market for HSCT procedures declines or fails to grow at anticipated levels for any reason, or if the development and commercialization of therapies targeted at the underlying cause of diseases addressed by ProHema obviate the need for patients to undergo HSCT procedures, our business prospects will be significantly harmed.

We face competition from other biotechnology and pharmaceutical 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 face competition from major multinational pharmaceutical companies, established and early-stage biotechnology companies, and universities and other research institutions. Many of our competitors have greater financial and other resources, such as larger research and development staff and more experienced marketing and manufacturing organizations. Large pharmaceutical companies, in particular, have extensive experience in clinical testing, obtaining regulatory approvals, recruiting patients and manufacturing pharmaceutical products. These companies also have significantly greater research, sales and marketing capabilities and collaborative arrangements in our target markets with leading companies and research institutions. Established pharmaceutical companies may also invest heavily to accelerate discovery and development of novel therapeutics or to in-license novel therapeutics that could make the product candidates that we develop obsolete. As a result of all of these factors, our competitors may succeed in obtaining patent

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protection or FDA approval or discovering, developing and commercializing treatments in the rare disease indications that we are targeting before we do. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large, established companies.

There are several clinical-stage development programs that seek to improve human umbilical cord blood transplantation through the use of *ex vivo* expansion technologies to increase the quantity of HSCs for use in HSCT or the use of *ex vivo* differentiation technologies to increase the quantity of hematopoietic progenitor cells for use in HSCT. Although there are currently no approved pharmaceutical products specifically for the treatment of muscular dystrophies, we are aware of several other companies with product candidates in various stages of development for the treatment of muscular dystrophies. In addition, many universities and private and public research institutes may develop technologies of interest to us, but license them to our competitors. Our competitors may succeed in developing, acquiring or licensing on an exclusive basis, technologies and drug products that are more effective or less costly than ProHema or any other product candidates that we are currently developing or that we may develop, which could render our products obsolete and noncompetitive.

We believe that our ability to successfully compete will depend on, among other things:

- the results of our preclinical studies and clinical trials;
- our ability to recruit and enroll patients for our clinical trials;
- the efficacy, safety and reliability of our product candidates;
- the speed at which we develop our product candidates;
- our ability to design and successfully execute appropriate clinical trials;
- our ability to protect and develop intellectual property rights related to our products;
- our ability to maintain a good relationship with regulatory authorities;
- the timing and scope of regulatory approvals, if any;
- our ability to commercialize and market any of our product candidates that receive regulatory approval;
- market perception and acceptance of stem cell therapeutics;
- acceptance of our product candidates by physicians and institutions that perform HSCTs;
- the price of our products;

adequate levels of reimbursement under private and governmental health insurance plans, including Medicare; and

our ability to manufacture and sell commercial quantities of any approved products to the market.

If our competitors market products that are more effective, safer or less expensive than our future products, if any, or that reach the market sooner than our future products, if any, we may not achieve commercial success. Any inability to successfully compete effectively will adversely impact our business and financial prospects.

We may need to expand our operations and increase the size of our company, and we may experience difficulties in managing growth.

As we increase the number of ongoing product development programs and advance our product candidates through preclinical studies and clinical trials, we will need to increase our product development, scientific and administrative headcount to manage these programs. Our management, personnel and systems currently in place may not be adequate to support this future growth. Our need to effectively manage our operations, growth and various projects requires that we:

successfully attract and recruit new employees or consultants with the requisite expertise and experience;

manage our clinical programs effectively;

if we receive regulatory approval for any product candidate, develop a marketing and sales infrastructure; and

continue to improve our operational, financial and management controls, reporting systems and procedures, including those related to being a public company.

If we are unable to successfully manage this growth and increased complexity of operations, our business may be adversely affected.

We may not be able to manage our business effectively if we are unable to attract and retain key personnel and consultants.

We may not be able to attract or retain qualified management, finance, scientific and clinical personnel and consultants due to the intense competition for qualified personnel and consultants among biotechnology, pharmaceutical and other businesses. If we are not able to attract and retain necessary personnel and consultants to accomplish our business objectives, we may experience constraints that will significantly impede the achievement of our development objectives, our ability to raise additional capital and our ability to implement our business strategy.

Our industry has experienced a high rate of turnover of management personnel in recent years. We are highly dependent on the development, regulatory, commercialization and business development expertise of Christian Weyer, our President and Chief Executive Officer; J. Scott Wolchko, our Chief Financial Officer and Chief Operating Officer; Pratik S. Multani, our Chief Medical Officer; Daniel D. Shoemaker, our Chief Technology Officer; and Peter Flynn, our Senior Vice President, Early Program Development. If we lose one or more of our executive officers or key consultants, our ability to implement our business strategy successfully could be seriously harmed. While we have entered into employment contracts with each of our executive officers, any of them could leave our employment at any time, as all of our employees are at-will employees. Replacing key personnel and consultants may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to develop, gain regulatory approval of and commercialize products successfully. Competition to hire and retain employees and consultants from this limited pool is intense, and we may be unable to hire, train, retain or motivate these additional key personnel and consultants. Our failure to retain key personnel or consultants could materially harm our business.

We rely on our scientific founders and other scientific and clinical advisors and consultants to assist us in formulating our research, development and clinical strategies. These advisors and consultants are not our employees and may have commitments to, or consulting or advisory contracts with, other entities that may limit their availability to us. In addition, these advisors and consultants typically will not enter into non-compete agreements with us. If a conflict of interest arises between their work for us and their work for another entity, we may lose their services. Furthermore, our advisors may have arrangements with other companies to assist those companies in developing products or technologies that may compete with ours. In particular, if we are unable to maintain consulting relationships with our scientific founders or if they provide services to our competitors, our development and commercialization efforts will be impaired and our business will be significantly harmed.

Failure to build our finance infrastructure and improve our accounting systems and controls could impair our ability to comply with the financial reporting and internal controls requirements for publicly traded companies.

As a public company, we will operate in an increasingly demanding regulatory environment, which requires us to comply with the Sarbanes-Oxley Act of 2002, as amended, or the Sarbanes-Oxley Act, and the related rules and regulations of the SEC, expanded disclosure requirements, accelerated reporting requirements and more complex accounting rules. Company responsibilities required by the Sarbanes-Oxley Act include establishing corporate oversight and adequate internal control over financial reporting and disclosure controls and procedures. Effective internal controls are necessary for us to produce reliable financial reports and are important to help prevent financial fraud.

We cannot assure you that we will not have material weaknesses or significant deficiencies in our internal control over financial reporting. If we are unable to successfully remediate any material weakness or significant

deficiency in our internal control over financial reporting, or identify any material weaknesses or significant deficiencies that may exist, the accuracy and timing of our financial reporting may be adversely affected, we may be unable to maintain compliance with securities law requirements regarding timely filing of periodic reports in addition to applicable stock exchange listing requirements, and our stock price may decline materially as a result.

We have begun implementing our system of internal controls over financial reporting and preparing the documentation necessary to perform the evaluation needed to comply with Section 404(a) of the Sarbanes-Oxley Act. However, we anticipate that we will need to retain additional finance capabilities and build our financial infrastructure as we transition to operating as a public company, including complying with the requirements of Section 404 of the Sarbanes-Oxley Act. As we begin operating as a public company following this offering, we will need to continue improving our financial infrastructure with the retention of additional financial and accounting capabilities, the enhancement of internal controls and additional training for our financial and accounting staff.

Section 404(a) of the Sarbanes-Oxley Act requires annual management assessments of the effectiveness of our internal control over financial reporting, starting with the second annual report that we would expect to file with the SEC. However, for as long as we remain an emerging growth company as defined in the JOBS Act, we intend to take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies including, but not limited to, not being required to comply with the auditor attestation requirements of Section 404(b) of the Sarbanes-Oxley Act. We may take advantage of these reporting exemptions until we are no longer an emerging growth company. We will remain an emerging growth company until the earliest of (i) the last day of the fiscal year in which we have total annual gross revenues of $1 billion or more; (ii) the last day of our fiscal year following the fifth anniversary of the date of the completion of this offering; (iii) the date on which we have issued more than $1 billion in nonconvertible debt during the previous three years; or (iv) the date on which we are deemed to be a large accelerated filer under the rules of the SEC.

Until we are able to expand our finance and administrative capabilities and establish necessary financial reporting infrastructure, we may not be able to prepare and disclose, in a timely manner, our financial statements and other required disclosures or comply with the Sarbanes-Oxley Act or existing or new reporting requirements. If we cannot provide reliable financial reports or prevent fraud, our business and results of operations could be harmed and investors could lose confidence in our reported financial information.

Our insurance policies are expensive and only protect us from some business risks, which will leave us exposed to significant uninsured liabilities.

We do not carry insurance for all categories of risk that our business may encounter. Some of the policies we currently maintain include general liability, employment practices liability, property, products liability and directors and officers insurance. We do not know, however, if we will be able to maintain insurance with adequate levels of coverage. Any significant uninsured liability may require us to pay substantial amounts, which would adversely affect our financial position and results of operations.

If we engage in an acquisition, reorganization or business combination, we will incur a variety of risks that could adversely affect our business operations or our stockholders.

From time to time we have considered, and we will continue to consider in the future, strategic business initiatives intended to further the expansion and development of our business. These initiatives may include acquiring businesses, technologies or products or entering into a business combination with another company. If we pursue such a strategy, we could, among other things:

- issue equity securities that would dilute our current stockholders percentage ownership;
- incur substantial debt that may place strains on our operations;
- spend substantial operational, financial and management resources to integrate new businesses, technologies and products;
- assume substantial actual or contingent liabilities;

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reprioritize our development programs and even cease development and commercialization of our product candidates; or

merge with, or otherwise enter into a business combination with, another company in which our stockholders would receive cash or shares of the other company on terms that certain of our stockholders may not deem desirable.

Although we intend to evaluate and consider acquisitions, reorganizations and business combinations in the future, we have no agreements or understandings with respect to any acquisition, reorganization or business combination at this time.

We face potential product liability and, if successful claims are brought against us, we may incur substantial liability and costs. If the use of our product candidates harms patients, or is perceived to harm patients even when such harm is unrelated to our product candidates, our regulatory approvals could be revoked or otherwise negatively impacted and we could be subject to costly and damaging product liability claims.

The use of our product candidates in clinical trials and the sale of any products for which we obtain marketing approval exposes us to the risk of product liability claims. Product liability claims might be brought against us by participants in clinical trials, consumers, healthcare providers, pharmaceutical companies or others selling or otherwise coming into contact with our product candidates and any products for which we obtain marketing approval. There is a risk that our product candidates may induce adverse events. If we cannot successfully defend against product liability claims, we could incur substantial liability and costs. In addition, regardless of merit or eventual outcome, product liability claims may result in:

impairment of our business reputation;

withdrawal of clinical trial participants;

costs due to related litigation;

distraction of management s attention from our primary business;

substantial monetary awards to patients or other claimants;

the inability to commercialize our product candidates; and

decreased demand for our product candidates, if approved for commercial sale.

We carry product liability insurance of $5.0 million per occurrence and $5.0 million aggregate limit. We believe our product liability insurance coverage is sufficient in light of our current clinical programs; however, we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses due to liability. If and when we obtain marketing approval for product candidates, we intend to expand our insurance coverage to include the sale of commercial products; however, we may be unable to obtain product liability insurance on commercially reasonable terms or in adequate amounts. On occasion, large judgments have been awarded in class action lawsuits based on drugs or medical treatments that had unanticipated adverse effects. In addition, under some of our agreements with clinical trial sites, we are required to indemnify the sites and their personnel against product liability and other claims. A successful product liability claim or series of claims brought against us or any third parties whom we are required to indemnify could cause our stock price to decline and, if judgments exceed our insurance coverage, could adversely affect our results of operations and business.

Patients with the diseases targeted by our product candidates are often already in severe and advanced stages of disease and have both known and unknown significant pre-existing and potentially life-threatening health risks. During the course of treatment, patients may suffer adverse events, including death, for reasons that may be related to our product candidates. Such events could subject us to costly litigation, require us to

pay substantial amounts of money to injured patients, delay, negatively impact or end our opportunity to receive or maintain regulatory approval to market our products, or require us to suspend or abandon our commercialization efforts. Even in a circumstance in which we do not believe that an adverse event is related to our products, the investigation into the circumstance may be time-consuming or inconclusive. These investigations may interrupt our development and commercialization efforts, delay

our regulatory approval process, or impact and limit the type of regulatory approvals our product candidates receive or maintain. As a result of these factors, a product liability claim, even if successfully defended, could have a material adverse effect on our business, financial condition or results of operations.

We may use our financial and human resources to pursue a particular research program or product candidate and fail to capitalize on programs or product candidates that may be more profitable or for which there is a greater likelihood of success.

Because we have limited resources, we may forego or delay pursuit of opportunities with certain programs or product candidates or for indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs for product candidates may not yield any commercially viable products. 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 strategic collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate, or we may allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a partnering arrangement.

We use hazardous chemicals, biological materials and infectious agents in our business. Any claims relating to improper handling, storage or disposal of these materials could be time consuming and costly.

Our research and development and manufacturing processes involve the controlled use of hazardous materials, including chemicals, biological materials and infectious disease agents. Our operations produce hazardous waste products. We cannot eliminate the risk of accidental contamination or discharge and any resultant injury from these materials. We may be sued for any injury or contamination that results from our use or the use by third parties of these materials, and our liability may exceed our insurance coverage and our total assets. Federal, state and local laws and regulations govern the use, manufacture, storage, handling and disposal of these hazardous materials and specified waste products, as well as the discharge of pollutants into the environment and human health and safety matters. Compliance with environmental laws and regulations may be expensive and may impair our research, development and production efforts. If we fail to comply with these requirements, we could incur substantial costs, including civil or criminal fines and penalties, clean-up costs or capital expenditures for control equipment or operational changes necessary to achieve and maintain compliance. In addition, we cannot predict the impact on our business of new or amended environmental laws or regulations or any changes in the way existing and future laws and regulations are interpreted and enforced.

Our business is subject to the risks of earthquakes, fire, power outages, floods and other catastrophic events, and to interruption by manmade problems such as terrorism.

A significant natural disaster, such as an earthquake, fire or a flood, or a significant power outage could have a material adverse impact on our business, operating results and financial condition. Our corporate headquarters are located in San Diego, California, a region known for seismic activity. In addition, natural disasters could affect our third-party service providers ability to perform services for us on a timely basis. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, our efforts to obtain regulatory approvals for, and to commercialize, our product candidates may be delayed or prevented. In addition, if a catastrophe such as an earthquake, fire, flood or power loss should affect one of the third parties on which we rely, our business prospects could be harmed. For example, if a central laboratory holding all of our clinical study samples were to suffer a catastrophic loss of their facility, we would lose all of our samples and would have to repeat our studies. In addition, acts of terrorism could cause disruptions in our business or the business of our third-party service providers, partners, customers or the economy as a whole.

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

We are exposed to the risk of employee fraud or other misconduct. Misconduct by employees could include intentional failures to comply with the regulations of the FDA or foreign regulators, provide accurate information to the FDA or foreign regulators, comply with healthcare fraud and abuse laws and regulations in the United States and abroad, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Employee misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and cause 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 employee misconduct, 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 comply with these 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 significant fines or other sanctions.

Risks Related to This Offering and Ownership of Our Common Stock

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

Prior to this offering, there has been no public market for shares of our common stock. Although our common stock has been approved for listing on The NASDAQ Global Market, an active trading market for our shares may never develop or be sustained following this offering. The initial public offering price of our common stock will be determined through negotiations between us and the underwriters. This initial public offering price may not be indicative of the market price of our common stock after this offering. In the absence of an active trading market for our common stock, investors may not be able to sell their common stock at or above the initial public offering price or at the time that they would like to sell.

We expect that our stock price may fluctuate significantly.

The market price of shares of our common stock could be subject to wide fluctuations as a result of many risks listed in this section, and others beyond our control, including:

- the timing of the initiation or completion of our clinical trials;
- the results of our clinical trials and preclinical studies;
- the results of clinical trials of our competitors product candidates or of other stem cell therapeutics in general;
- developments concerning our owned or licensed intellectual property rights;
- changes in laws or regulations applicable to stem cell therapeutics generally or our product candidates in particular, including but not limited to clinical trial requirements for approvals;
- changes in the markets for HSCT products and in the field of stem cell therapeutics, or changes in the markets for the treatment of muscular dystrophies and other diseases targeted by our product candidates;

actual or anticipated fluctuations in our financial condition and operating results;

actual or anticipated changes in our growth rate relative to our competitors;

competition from existing products or new products that may emerge;

announcements by us or our competitors of significant acquisitions, strategic partnerships, joint ventures, collaborations or capital commitments;

failure to meet or exceed financial estimates and projections of the investment community or that we provide to the public;

issuance of new or updated research or reports by securities analysts;

fluctuations in the valuation of companies perceived by investors to be comparable to us;

share price and volume fluctuations attributable to inconsistent trading volume levels of our shares;

additions or departures of key management or scientific personnel;

disputes or other developments related to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;

announcements or expectations of additional debt or equity financing efforts;

sales of our common stock by us, our insiders or our other stockholders; and

general economic and market conditions.

These and other market and industry factors may cause the market price and demand for our common stock to fluctuate substantially, regardless of our actual operating performance, which may limit or prevent investors from readily selling their shares of common stock and may otherwise negatively affect the liquidity of our common stock. In addition, the stock market in general, and The NASDAQ Global Market and pharmaceutical companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. In the past, when the market price of a stock has been volatile, holders of that stock have instituted securities class action litigation against the company that issued the stock. If any of our stockholders brought a lawsuit against us, we could incur substantial costs defending the lawsuit and divert the time and attention of our management.

Our principal stockholders will exercise significant control over our company.

Immediately after this offering, our executive officers, directors and entities affiliated with our five percent stockholders will beneficially own, in the aggregate, shares representing approximately 64.4% of our outstanding voting stock, taking into account shares of common stock that certain existing holders of five percent or more of our voting securities and stockholders who are affiliated with certain of our directors have agreed to purchase in this offering, as described under Certain Relationships and Related Party Transactions Participation in this Offering. The above percentage assumes the conversion of approximately $21.0 million in principal and accrued interest on our 2013 Notes upon the completion of this offering at the initial public offering price of $6.00 per share, and that we repay in cash an aggregate of approximately $2.8 million in principal and accrued interest under our 2013 Notes issued in August 2013 in lieu of the issuance of 470,252 shares of common stock upon conversion of such notes, which, if issued, would cause the holders of such notes, together with their affiliates and related entities, to beneficially own 15% or more of our outstanding common stock following the completion of this offering. Although we are not aware of any voting arrangements that will be in place among these stockholders following this offering, if these stockholders were to choose to act together, as a result of their stock ownership, they would be able to influence our management and affairs and control all matters submitted to our stockholders for approval, including the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. This concentration of ownership may have the effect of delaying or preventing a change in control of our company and might affect the market price of our common stock.

Future sales of shares by existing stockholders could cause our stock price to decline.

If our existing stockholders sell, or indicate an intent to sell, substantial amounts of our common stock in the public market after the 180-day contractual lock-up and other legal restrictions on resale discussed in this

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prospectus lapse, the trading price of our common stock could decline significantly and could decline below the initial public offering price. Based on shares outstanding as of June 30, 2013, and assuming the conversion of approximately $21.0 million in principal and accrued interest on our 2013 Notes upon the completion of this offering at the initial public offering price of $6.00 per share, and assuming that we repay in cash an aggregate of approximately $2.8 million in principal and accrued interest under our 2013 Notes issued in August 2013 in lieu of the issuance of 470,252 shares of common stock upon the conversion of such notes, which, if issued, would cause the holders of such notes, together with their affiliates and related entities, to beneficially own 15% or more of our outstanding common stock following the completion of this offering, we will have outstanding 19,156,609 shares of common stock, assuming no exercise of outstanding warrants or options. Of these shares, approximately 6,976,559 shares of common stock, plus any shares sold pursuant to the underwriters option to purchase additional shares, will be immediately freely tradable, without restriction, in the public market except for any shares purchased in this offering by certain of our stockholders or held by our affiliates as that term is defined under Rule 144 of the Securities Act of 1933, as amended, or the Securities Act. The underwriters may, in their discretion and under the terms of the lock-up agreements, permit our officers, directors, employees and current stockholders to sell some or all of their shares prior to the expiration of the lock-up agreements. We cannot predict the effect, if any, that public sales of these shares or the availability of these shares for sale will have on the market price of our common stock.

After the lock-up agreements pertaining to this offering expire and based on shares outstanding as of June 30, 2013 and assuming the conversion of our 2013 Notes as described above, an additional approximately 12,180,050 shares will be eligible for sale in the public market, although a portion of such shares held by our affiliates will be subject to volume limitations and other conditions pursuant to Rule 144 of the Securities Act. In addition, the shares subject to outstanding options under our equity incentive plans and the shares reserved for future issuance under our equity incentive plans will become eligible for sale in the public market in the future, subject to certain legal and contractual limitations. Moreover, 180 days after the completion of this offering, certain holders of our common stock will have the right to require us to register these shares under the Securities Act pursuant to an investor rights agreement. If our existing stockholders sell substantial amounts of our common stock in the public market, or if the public perceives that such sales could occur, this could have an adverse impact on the market price of our common stock, even if there is no relationship between such sales and the performance of our business.

We will have broad discretion in how we use the proceeds of this offering. We may not use these proceeds effectively, which could affect our results of operations and cause our stock price to decline.

We will have considerable discretion in the application of the net proceeds of this offering. As a result, investors will be relying upon management s judgment with only limited information about our specific intentions for the use of the balance of the net proceeds of this offering. We may use the net proceeds for purposes that do not yield a significant return or any return at all for our stockholders. In addition, pending their use, we may invest the net proceeds from this offering in a manner that does not produce income or that loses value.

Being a public company will increase our expenses and administrative burden.

As a public company, we will incur significant legal, insurance, accounting and other expenses that we did not incur as a private company. In addition, our administrative staff will be required to perform additional tasks. For example, in anticipation of becoming a public company, we will need to adopt additional internal controls and disclosure controls and procedures, retain a transfer agent, adopt an insider trading policy and bear all of the internal and external costs of preparing and distributing periodic public reports in compliance with our obligations under the securities laws.

In addition, laws, regulations and standards applicable to public companies relating to corporate governance and public disclosure, including the Sarbanes-Oxley Act and related regulations implemented by the SEC and The NASDAQ Stock Market, are creating uncertainty for public companies, increasing legal and financial compliance costs and making some activities more time consuming. These laws, regulations and standards are subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their application in practice

may evolve over time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices. We intend to invest resources to comply with evolving laws, regulations and standards, and this investment will result in increased general and administrative expenses and may divert management s time and attention from product development activities. If our efforts to comply with new laws, regulations and standards differ from the activities intended by regulatory or governing bodies due to ambiguities related to practice, regulatory authorities may initiate legal proceedings against us and our business may be harmed. In connection with this offering, we are increasing our directors and officers insurance coverage, which will increase our insurance cost. In the future, it will be more expensive for us to obtain director and officer liability insurance, and we may be required to accept reduced coverage or incur substantially higher costs to obtain coverage. These factors could also make it more difficult for us to attract and retain qualified members of our board of directors, particularly to serve on our audit committee and compensation committee, and qualified executive officers.

Provisions of Delaware law or our charter documents could delay or prevent an acquisition of our company, even if the acquisition would be beneficial to our stockholders, and could make it more difficult for you to change management.

Provisions of Delaware law and our amended and restated certificate of incorporation and amended and restated bylaws, which will be effective upon the completion of this offering, may discourage, delay or prevent a merger, acquisition or other change in control that stockholders may consider favorable, including transactions in which stockholders might otherwise receive a premium for their shares. These provisions may also prevent or delay attempts by stockholders to replace or remove our current management or members of our board of directors. These provisions include:

a classified board of directors with limitations on the removal of directors;

advance notice requirements for stockholder proposals and nominations;

the inability of stockholders to act by written consent or to call special meetings;

the ability of our board of directors to make, alter or repeal our amended and restated bylaws: and

the authority of our board of directors to issue preferred stock with such terms as our board of directors may determine.

The affirmative vote of the holders of at least 75% of our shares of capital stock entitled to vote, and not less than 75% of the outstanding shares of each class entitled to vote thereon as a class, is generally necessary to amend or repeal the above provisions that are contained in our amended and restated certificate of incorporation. In addition, absent approval of our board of directors, our amended and restated bylaws may only be amended or repealed by the affirmative vote of the holders of at least 75% of our shares of capital stock entitled to vote.

In addition, upon the completion of this offering, we will be subject to the provisions of Section 203 of the Delaware General Corporation Law, which limits business combination transactions with stockholders of 15% or more of our outstanding voting stock that our board of directors has not approved. These provisions and other similar provisions make it more difficult for stockholders or potential acquirers to acquire us without negotiation. These provisions may apply even if some stockholders may consider the transaction beneficial to them.

As a result, these provisions could limit the price that investors are willing to pay in the future for shares of our common stock. These provisions might also discourage a potential acquisition proposal or tender offer, even if the acquisition proposal or tender offer is at a premium over the then-current market price for our common stock.

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

We are an emerging growth company, as defined in the JOBS Act, and we intend to take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are not

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emerging growth companies, including not being required to comply with the auditor attestation requirements of Section 404(b) of the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and shareholder approval of any golden parachute payments not previously approved. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

Investors in this offering will pay a higher price than the book value of our common stock.

If you purchase common stock in this offering, you will pay more for your shares than the amounts paid by existing stockholders for their shares. You will incur immediate and substantial dilution of $3.25 per share, representing the difference between our pro forma as adjusted net tangible book value per share after giving effect to this offering at the initial public offering price of $6.00 per share. In the past, we have issued restricted stock and options and warrants to acquire shares of our capital stock at prices significantly below the initial public offering price. In addition, we will be obligated, upon the occurrence of certain milestone events or a change of control, to issue additional shares of common stock to the holders of exchangeable shares of our subsidiary, Fate Canada, in the future for no additional consideration. To the extent any outstanding options or warrants are ultimately exercised or we issue additional shares of common stock to the holders of exchangeable shares of our subsidiary, you will sustain further dilution.

If equity research analysts do not publish research or reports about our business or if they issue unfavorable commentary or downgrade our common stock, the price of our common stock could decline.

The trading market for our common stock will rely in part on the research and reports that equity research analysts publish about us and our business. We do not control these analysts. The price of our common stock could decline significantly if one or more equity analysts downgrade our common stock or if analysts issue other unfavorable commentary or cease publishing reports about us or our business.

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SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus, including the sections entitled Prospectus Summary, Risk Factors, Management s Discussion and Analysis of Financial Condition and Results of Operations and Business contains forward-looking statements that are based on our management s belief and assumptions and on information currently available to our management. Although we believe that the expectations reflected in these forward-looking statements are reasonable, these statements relate to future events or our future financial performance, and involve known and unknown risks, uncertainties and other factors that may cause our actual results, levels of activity, performance or achievements to be materially different from any future results, levels of activity, performance or achievements expressed or implied by these forward-looking statements. Forward-looking statements in this prospectus include, but are not limited to, statements about:

- the projected timing of the initiation of our clinical trials for our product candidates;
- the timing of, and our ability to incorporate, the use of our NRM formulation in our Phase 2 clinical trial of ProHema in adults undergoing double umbilical cord blood transplant, or UCBT, and in any subsequent clinical trials of ProHema;
- the timing of our submission to the FDA of, and any review or comments on, product data that we will need to generate for ProHema as manufactured using our NRM formulation with materials intended for clinical use;
- our ability to obtain consent from the FDA and applicable IRBs to our amended protocol for our Phase 2 clinical trial of ProHema;
- our plans to resume enrollment in our Phase 2 clinical trial of ProHema or to commence other clinical trials of ProHema;
- our plans to complete the preclinical development of and to submit an IND for our Wnt7a analogs;
- our ability to satisfy regulatory requirements with respect to ProHema and our other product candidates, many of which are new and still evolving;
- the ability of cell processing facilities operated by transplant centers to consistently manufacture ProHema under the proper conditions;
- the performance of third-party service providers and independent contractors upon whom we rely to conduct our clinical trials and to manufacture our product candidates or certain components of our product candidates;
- our ability to discover, develop and commercialize innovative therapeutics using our proprietary platforms;
- our ability to develop sales and marketing capabilities or to enter into strategic partnerships to develop and commercialize ProHema or any of our other product candidates;
- the timing and success of the commercialization of ProHema or any of our other product candidates;

the degree of market acceptance of stem cell based therapies in general and our product candidates in particular;

the size and growth of the potential markets for our product candidates and our ability to serve those markets;

regulatory developments in the United States and foreign countries;

our ability to obtain, maintain, defend and enforce intellectual property rights protecting our product candidates;

our obligations to make payments under the 2013 Notes; and

the accuracy of our estimates regarding expenses and capital requirements and the use of proceeds from this offering.

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In some cases, you can identify forward-looking statements by terminology such as may, will, should, expects, intends, plans, anticipate believes, estimates, predicts, potential, continue or the negative of these terms or other comparable terminology. These statements are only predictions. You should not place undue reliance on forward-looking statements because they involve known and unknown risks, uncertainties and other factors, which are, in some cases, beyond our control and which could materially affect results. Factors that may cause actual results to differ materially from current expectations include, among other things, those listed under Risk Factors and elsewhere in this prospectus. If one or more of these risks or uncertainties occur, or if our underlying assumptions prove to be incorrect, actual events or results may vary significantly from those implied or projected by the forward-looking statements. No forward-looking statement is a guarantee of future performance. You should read this prospectus and the documents that we reference in this prospectus and have filed with the SEC as exhibits to the registration statement, of which this prospectus is a part, completely and with the understanding that our actual future results may be materially different from any future results expressed or implied by these forward-looking statements.

The forward-looking statements in this prospectus represent our views as of the date of this prospectus. We anticipate that subsequent events and developments will cause our views to change. However, while we may elect to update these forward-looking statements at some point in the future, we have no current intention of doing so except to the extent required by applicable law. You should therefore not rely on these forward-looking statements as representing our views as of any date subsequent to the date of this prospectus.

The market data and certain other statistical information used throughout this prospectus are based on independent industry publications, governmental publications, reports by market research firms or other independent sources. Some data are also based on our good faith estimates.

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USE OF PROCEEDS

We estimate that the net proceeds to us from the sale of 6,666,667 shares of common stock in this offering will be approximately $34.8 million based upon the initial public offering price of $6.00 per share, and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. If the underwriters option to purchase additional shares in this offering is exercised in full, we estimate that our net proceeds will be approximately $40.4 million, after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

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

- approximately $20.0 million to fund research and development activities to advance our HSC modulation platform and the clinical and preclinical development of its product candidates, including the conduct of our Phase 2 clinical trial of ProHema in patients with orphan hematologic malignancies;

- approximately $12.0 million to fund research and development activities to advance our SSC modulation platform and the preclinical development of its product candidates, including the conduct of preclinical studies of our Wnt7a analog product candidates; and

- the remainder for working capital and general corporate purposes, including funding the costs of operating as a public company.

The expected use of net proceeds from this offering represents our intentions based upon our current plans and business conditions, which could change in the future as our plans and business conditions evolve. The amounts and timing of our actual expenditures may vary significantly depending on numerous factors, including the progress of our development, the status of and results from clinical trials and preclinical studies, as well as any collaborations that we may enter into with third parties for our product candidates, the amount of cash available from other sources and any unforeseen cash needs. As a result, our management will retain broad discretion over the allocation of the net proceeds from this offering.

Pending these uses, we intend to invest the net proceeds in high quality, investment grade instruments, certificates of deposit or direct or guaranteed obligations of the U.S. government, or hold as cash.

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DIVIDEND POLICY

We have never declared or paid any dividends on our capital stock. We currently intend to retain all available funds and any future earnings, if any, to fund the development and expansion of our business and we do not anticipate paying any cash dividends in the foreseeable future. Any future determination to pay dividends will be made at the discretion of our board of directors.

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CAPITALIZATION

The following table sets forth our cash and cash equivalents and capitalization as of June 30, 2013:

- on an actual basis;
- on a pro forma basis to give effect to (i) the filing and effectiveness of our amended and restated certificate of incorporation, (ii) the conversion of all outstanding shares of our convertible preferred stock into an aggregate of 7,229,590 shares of common stock immediately prior to the completion of this offering, (iii) our receipt of aggregate gross proceeds of approximately $20.7 million from the sale of our 2013 Notes issued in July and August 2013 and a $0.1 million debt discount related to a beneficial conversion feature in our 2013 Notes issued in July 2013, (iv) the issuance of 3,499,319 shares of common stock upon the conversion of approximately $21.0 million in outstanding principal and accrued interest on our 2013 Notes upon the completion of this offering, based on the initial public offering price per share of $6.00, (v) our repayment in cash of an aggregate of approximately $2.8 million in principal and accrued interest under our 2013 Notes issued in August 2013 in lieu of the issuance of 470,252 shares of common stock upon the conversion of such notes, which, if issued, would cause the holders of such notes, together with their affiliates and related entities, to beneficially own 15% or more of our outstanding common stock following the completion of this offering, (vi) a charge to retained earnings of $0.3 million upon the completion of this offering to reflect the accelerated amortization of debt discount to interest expense upon conversion of the 2013 Notes, (vii) the issuance of 403,841 shares of common stock pursuant to the redemption of an aggregate of 900,000 exchangeable shares of Fate Canada as described in Description of Capital Stock Exchangeable Shares in Canadian Subsidiary elsewhere in this prospectus, which will occur immediately prior to the completion of this offering, and the resultant reclassification of our exchangeable share liability to additional paid-in capital, a component of stockholders equity and (viii) the adjustment of our outstanding warrants to purchase 230,000 shares of convertible preferred stock into warrants to purchase 36,074 shares of common stock upon the completion of this offering, and the resultant reclassification of our warrant liability to additional paid-in capital, a component of stockholders equity; and
- on a pro forma as adjusted basis to give further effect to our sale in this offering of 6,666,667 shares of common stock at the initial public offering price of $6.00 per share, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us.

Pursuant to the terms of our 2013 Notes issued in August 2013, in no event may the aggregate number of shares issued upon the completion of this offering pursuant to the conversion of such notes cause the holders of such notes, together with their affiliates and related parties, to beneficially own 15% or more of our outstanding common stock immediately following the completion of this offering. Unless earlier repaid by us, any balance remaining outstanding under our convertible promissory notes issued in August 2013 following the completion of this offering may be converted into additional shares of common stock on the date 30 days after the completion of this offering if the conversion of such remaining balance outstanding would not cause the holders of such notes, together with their affiliates and related parties, to beneficially own 15% or more of our outstanding common stock immediately following the conversion of the remaining balance on such notes; otherwise we will be required to repay such outstanding balance on the date 30 days after the completion of this offering.

You should read the following table together with Management s Discussion and Analysis of Financial Condition and Results of Operations, Description of Capital Stock, and the financial statements and related notes appearing elsewhere in this prospectus.

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	As of June 30, 2013		
	Actual	Pro Forma (unaudited)	Pro Forma As Adjusted
	(in thousands, except per share data)		
Cash and cash equivalents	$ 3,399	$ 21,313	$ 56,433
Capitalization:			
Long-term debt (including current portion)	$ 2,708	$ 2,708	$ 2,708
Convertible notes, net of discount	2,730		
Warrant liability[(1)]	194		
Exchangeable share liability[(1)]	1,811		
Convertible preferred stock, $0.001 par value; 62,200,000 shares authorized, 44,967,690 shares issued and outstanding, actual; no shares issued and outstanding, pro forma and pro forma as adjusted.	56,526		
Stockholders (deficit) equity:			
Preferred stock, $0.001 par value; no shares authorized, issued and outstanding, actual; 5,000,000 shares authorized and no shares issued and outstanding, pro forma and pro forma as adjusted.			
Common stock, $0.001 par value; 100,000,000 shares authorized, 1,357,192 shares issued and outstanding, actual; 150,000,000 shares authorized and 12,489,942 shares issued and outstanding, pro forma; and 150,000,000 shares authorized and 19,156,609 shares issued and outstanding, pro forma as adjusted	1	12	19
Additional paid-in capital	13,285	92,785	127,603
Deficit accumulated during the development stage	(74,697)	(75,033)	(75,033)
Total stockholders (deficit) equity	(61,411)	17,764	52,589
Total capitalization	$ 2,558	$ 20,472	$ 55,297

(1) Upon the completion of our initial public offering, we anticipate recording a charge of approximately $0.6 million related to the final adjustment to fair value of our exchangeable share liability and warrant liability, based on the initial public offering price per share of $6.00, and further assuming all other inputs into our valuation models remain unchanged from those as of June 30, 2013.

The actual, pro forma and pro forma as adjusted information set forth in the table excludes:

1,518,712 shares of common stock issuable upon the exercise of stock options outstanding as of June 30, 2013 at a weighted average exercise price of $1.46 per share;

36,074 shares of common stock issuable upon the exercise of warrants outstanding as of June 30, 2013 at a weighted average exercise price of $7.21 per share, which warrants prior to the completion of this offering are exercisable to purchase preferred stock, assuming such warrants will not be exercised prior to the completion of this offering;

1,020,000 shares of common stock reserved for future issuance under the 2013 Plan, which will become effective immediately prior to the completion of this offering;

729,000 shares of common stock reserved for future issuance under the ESPP, which will become effective immediately prior to the completion of this offering; and

up to 480,764 shares of common stock that may be issuable to holders of exchangeable shares of Fate Canada in connection with certain milestone or change of control events that may occur after this offering, as described in Description of Capital Stock Exchangeable Shares in Canadian Subsidiary elsewhere in this prospectus.

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DILUTION

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

Our historical net tangible book deficit of our common stock as of June 30, 2013 was approximately $(61.4) million, or $(45.25) per share. Historical net tangible book deficit per share represents our total tangible assets less our total liabilities and convertible preferred stock, divided by the number of shares of common stock outstanding at June 30, 2013.

Our pro forma net tangible book value of our common stock as of June 30, 2013 was $17.8 million, or approximately $1.42 per share. Pro forma net tangible book value gives effect to (i) the conversion of all outstanding shares of our convertible preferred stock into an aggregate of 7,229,590 shares of common stock upon the completion of this offering, (ii) the sale of our 2013 Notes in July and August 2013 for aggregate gross proceeds of $20.7 million, a $0.1 million debt discount related to a beneficial conversion feature in the notes issued in July 2013, and the issuance of 3,499,319 shares of common stock upon the conversion of approximately $21.0 million in outstanding principal and accrued interest on our 2013 Notes upon the completion of this offering, based on the initial public offering price per share of $6.00, (iii) our repayment in cash of an aggregate of approximately $2.8 million in principal and accrued interest under our 2013 Notes issued in August 2013 in lieu of the issuance of 470,252 shares of common stock upon conversion of such notes, which, if issued, would cause the holders of such notes and their affiliates and related parties to beneficially own 15% or more of our outstanding common stock following the completion of this offering, (iv) a charge to retained earnings of $0.3 million upon the completion of this offering to reflect the accelerated amortization of debt discount to interest expense upon conversion of the 2013 Notes, (v) the issuance of 403,841 shares of common stock pursuant to the redemption of an aggregate of 900,000 exchangeable shares of Fate Canada as described in Description of Capital Stock Exchangeable Shares in Canadian Subsidiary elsewhere in this prospectus, which will occur immediately prior to the completion of this offering, and the resultant reclassification of our exchangeable share liability to additional paid-in capital, a component of stockholders equity and (vi) the adjustment of our outstanding warrants to purchase 230,000 shares of convertible preferred stock into warrants to purchase 36,074 shares of common stock upon the completion of this offering and the resultant reclassification of our warrant liability to additional paid-in capital, a component of stockholders equity.

Net tangible book value dilution per share to new investors represents the difference between the amount per share paid by purchasers of common stock in this offering and the pro forma as adjusted net tangible book value per share of our common stock immediately after the completion of this offering. After giving effect to (i) the pro forma transactions described in the preceding paragraph and (ii) our sale of 6,666,667 shares of common stock in this offering at the initial public offering price of $6.00 per share, and after deducting estimated underwriting discounts and commissions and estimated offering expenses, our pro forma as adjusted net tangible book value as of June 30, 2013 would have been $2.75 per share. This represents an immediate increase in net tangible book value of $1.33 per share to existing stockholders and an immediate dilution in net tangible book value of $3.25 per share to purchasers of common stock in this offering, as illustrated in the following table:

Initial public offering price per share		$ 6.00
Historical net tangible book deficit per share as of June 30, 2013	$ (45.25)	
Pro forma increase in net tangible book value per share attributable to pro forma transactions described in preceding paragraphs	46.67	
Pro forma net tangible book value per share as of June 30, 2013	$ 1.42	
Increase per share attributable to new investors	1.33	
Pro forma as adjusted net tangible book value per share at June 30, 2013 after giving effect to this offering		2.75
Dilution per share to new investors		$ 3.25

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If the underwriters exercise their option to purchase additional shares of common stock in this offering in full, our pro forma as adjusted net tangible book value per share after the offering would be $2.89 per share, the increase in the pro forma as adjusted net tangible book value per share to existing stockholders would be $1.47 per share and the dilution to investors participating in this offering would be $3.11 per share.

The following table summarizes, on a pro forma as adjusted basis, as of June 30, 2013, the differences between the number of shares of common stock purchased from us, the total consideration paid to us and the average price per share paid by existing stockholders and by new investors in this offering at the initial public offering price of $6.00 per share, before deducting estimated underwriting discounts and commissions and estimated offering expenses.

	Shares Purchased		Total Consideration		Average Price Per Share
	Number	Percent	Amount	Percent	
Existing stockholders	12,489,942	65%	$ 91,035,000	69%	$ 7.29
New investors	6,666,667	35	40,000,002	31	6.00
Total	19,156,609	100%	$ 131,035,002	100%	

The above discussion and tables are based on 12,489,942 shares of common stock issued and outstanding as of June 30, 2013 and also reflects (i) the conversion of all outstanding shares of convertible preferred stock into an aggregate of 7,229,590 shares of common stock immediately prior to the completion of this offering, (ii) the sale of our 2013 Notes in June, July and August 2013 for aggregate gross proceeds of $23.7 million and the issuance of 3,499,319 shares of common stock upon the conversion of approximately $21.0 million in outstanding principal and accrued interest on our 2013 Notes upon the completion of this offering, based on the initial public offering price per share of $6.00, and assuming that we repay in cash an aggregate of approximately $2.8 million in principal and accrued interest under the 2013 Notes issued in August 2013 in lieu of the issuance of 470,252 shares of common stock upon the conversion of such notes, which, if issued, would cause the holders of such notes, together with their affiliates and related entities, to beneficially own 15% or more of our outstanding common stock following the completion of this offering, and (iii) the issuance of 403,841 shares of common stock pursuant to the redemption of an aggregate of 900,000 exchangeable shares of Fate Canada as described in Description of Capital Stock Exchangeable Shares in Canadian Subsidiary elsewhere in this prospectus, which will occur immediately prior to the completion of this offering, and excludes:

- 1,518,712 shares of common stock issuable upon the exercise of stock options outstanding as of June 30, 2013 at a weighted average exercise price of $1.46 per share;
- 36,074 shares of common stock issuable upon the exercise of warrants outstanding as of June 30, 2013 at a weighted average exercise price of $7.21 per share, which warrants prior to the completion of this offering are exercisable to purchase convertible preferred stock, assuming such warrants will not be exercised prior to the completion of this offering;
- 1,020,000 shares of common stock reserved for future issuance under the 2013 Plan, which will become effective immediately prior to the completion of this offering;
- 729,000 shares of common stock reserved for future issuance under the ESPP, which will become effective immediately prior to the completion of this offering; and
- up to 480,764 shares of common stock that may become issuable to holders of exchangeable shares of Fate Canada in connection with certain milestone or change of control events that may occur after this offering, as described in Description of Capital Stock Exchangeable Shares in Canadian Subsidiary elsewhere in this prospectus.

To the extent that outstanding options and warrants are exercised or additional shares of common stock are issued to holders of exchangeable shares of Fate Canada upon the achievement of milestones or the occurrence of a change of control, you will experience further dilution. In

addition, we may choose to raise additional capital

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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 that additional capital is raised through the sale of equity or convertible debt securities, the issuance of these securities may result in further dilution to our stockholders.

Certain holders of five percent or more of our voting securities and stockholders who are affiliated with certain of our directors have agreed to purchase an aggregate of 2,499,999 shares of our common stock in this offering at the initial public offering price. The foregoing discussion and tables do not reflect any purchases in this offering by these stockholders.

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SELECTED CONSOLIDATED FINANCIAL DATA

The following selected consolidated financial information should be read together with our consolidated financial statements and accompanying notes and information under the caption Management s Discussion and Analysis of Financial Condition and Results of Operations appearing elsewhere in this prospectus. Our historical results are not necessarily indicative of 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 selected consolidated statements of operations data for the years ended December 31, 2011 and 2012 and the summary consolidated balance sheet data as of December 31, 2011 and 2012 are derived from our audited consolidated financial statements appearing elsewhere in this prospectus. The summary consolidated statements of operations data for the six months ended June 30, 2012 and 2013 and the period from April 27, 2007 (inception) through June 30, 2013 and balance sheet data as of June 30, 2013 are derived from our unaudited consolidated financial statements and related notes appearing elsewhere in this prospectus. The unaudited consolidated financial statements have been prepared on a basis consistent with our audited consolidated financial statements included in this prospectus and, in the opinion of management, reflect all adjustments, consisting only of normal recurring adjustments, necessary to fairly state our financial position as of June 30, 2013 and results of operations for the six months ended June 30, 2012 and 2013.

	Years Ended December 31,		Six Months Ended June 30,		Period from April 27, 2007 (inception) through June 30, 2013 (unaudited)
	2011	2012	2012	2013	
			(unaudited)		
	(in thousands, except share and per share data)				
Consolidated Statements of Operations Data:					
Revenue:					
Collaboration revenue	$ 833	$ 1,268	$ 867	$ 417	$ 2,718
Grant revenue	337	1,402	632	345	2,084
Total revenue	1,170	2,670	1,499	762	4,802
Operating expenses:					
Research and development	9,858	11,999	5,281	5,598	50,577
General and administrative	4,605	4,228	2,081	2,789	26,860
Total operating expenses	14,463	16,227	7,362	8,387	77,437
Loss from operations	(13,293)	(13,557)	(5,863)	(7,625)	(72,635)
Total other income (expense)	(134)	(682)	(108)	(1,457)	(2,062)
Net loss and comprehensive loss	$ (13,427)	$ (14,239)	$ (5,971)	$ (9,082)	$ (74,697)
Net loss per common share, basic and diluted[1]	$ (16.16)	$ (13.06)	$ (5.96)	$ (7.41)	
Weighted-average shares used to compute basic and diluted net loss per share [1]	830,959	1,090,317	1,002,649	1,226,451	
Pro forma net loss per common share, basic and diluted (unaudited)[1]		$ (1.92)		$ (0.88)	
Weighted-average shares used to compute pro forma net loss per common share, basic and diluted (unaudited)[1]		7,464,793		8,866,985	

(1) See Note 1 to our financial statements included elsewhere in this prospectus for an explanation of the method used to calculate the historical and pro forma net loss per share, basic and diluted, and the number of shares used in the computation of the per share amounts.

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	As of December 31, 2011	2012	As of June 30, 2013 (unaudited)
		(in thousands)	
Consolidated Balance Sheet Data:			
Cash and cash equivalents	$ 6,387	$ 9,087	$ 3,399
Working capital deficit	3,013	4,943	(4,267)
Total assets	7,852	11,076	6,173
Convertible notes, net of discount	1,000		2,730
Warrant liability	221	184	194
Long-term debt, net of current portion	3,591	1,732	746
Exchangeable share liability	563	551	1,811
Convertible preferred stock	50,309	56,526	56,526
Deficit accumulated during the development stage	(51,376)	(65,615)	(74,697)
Total stockholders deficit	(50,683)	(52,825)	(61,411)

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MANAGEMENT S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion and analysis of our financial condition and results of operations together with our consolidated financial statements and related notes appearing in this prospectus. Some of the information contained in this discussion and analysis or set forth elsewhere in this prospectus, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the Risk Factors section of this prospectus, our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Overview

We are a clinical-stage biopharmaceutical company engaged in the discovery and development of pharmacologic modulators of adult stem cells to treat orphan diseases, including certain hematologic malignancies, lysosomal storage disorders and muscular dystrophies. Our novel approaches utilize established pharmacologic modalities, including small molecules and therapeutic proteins, and target well-characterized biological mechanisms to enhance the therapeutic potential of adult stem cells. Based on our deep understanding of key biological mechanisms that guide the fate of adult stem cells, we have built two platforms that optimize the activity of adult stem cells using both *ex vivo* and *in vivo* techniques: our HSC modulation platform and SSC modulation platform. We believe that the product candidates generated by our platforms have significant potential as life-changing or curative therapeutics across a broad range of orphan indications.

Our HSC modulation platform focuses on the *ex vivo* pharmacologic optimization of hematopoietic stem cells, or HSCs. Our lead product candidate from this platform, ProHema, is a pharmacologically modulated HSC therapeutic derived from umbilical cord blood. We have established human proof-of-concept for ProHema in a Phase 1b clinical trial by demonstrating enhanced engraftment. We are presently advancing ProHema in Phase 2 clinical development for hematologic malignancies. Our SSC modulation platform focuses on the *in vivo* pharmacologic activation of satellite stem cells, or SSCs. We have identified Wnt7a as a natural promoter of SSCs to drive muscle regeneration, and we have demonstrated proof-of-concept of Wnt7a analogs as potential therapeutics for muscular dystrophy in preclinical animal studies. We are presently advancing our Wnt7a analogs in preclinical development with the goal of filing an IND in 2014.

Since our inception in 2007, we have devoted substantially all of our resources to the development of our stem cell modulation platforms, the clinical and preclinical advancement of our product candidates, the creation, licensing and protection of related intellectual property and the provision of general and administrative support for these operations. We have generated revenues from collaboration activities and grants, but have not generated any revenues from therapeutic product sales. We have funded our operations primarily through the private placement of preferred stock and convertible notes and through commercial bank debt. We continue to be classified as a development stage company for financial reporting purposes.

We have never been profitable and have incurred net losses in each year since inception. Our net losses were $13.4 million and $14.2 million for the years ended December 31, 2011 and 2012, respectively, and $6.0 million and $9.1 million for the six months ended June 30, 2012 and 2013, respectively. Substantially all of our net losses resulted from costs incurred in connection with our research and development programs and from general and administrative costs associated with our operations. We expect to continue to incur significant expenses and increasing operating losses for at least the next several years. Our net losses may fluctuate significantly from quarter to quarter and year to year. We expect our expenses will increase substantially in connection with our ongoing activities as we:

- conduct clinical trials of our initial product candidates;
- continue our research and development efforts;
- manufacture preclinical study and clinical trial materials;
- maintain, expand and protect our intellectual property portfolio;

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seek regulatory approvals for our product candidates that successfully complete clinical trials;

hire additional clinical, quality control and technical personnel to conduct our clinical trials;

hire additional scientific personnel to support our product development efforts;

implement operational, financial and management systems; and

add general and administrative personnel to operate as a public company.

We do not expect to generate any revenues from therapeutic product sales until we successfully complete development and obtain regulatory approval for one or more of our product candidates, which we expect will take a number of years. If we obtain regulatory approval for any of our product candidates, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution. Accordingly, we will seek to fund our operations through public or private equity or debt financings or other sources. However, we may be unable to raise additional funds or enter into such other arrangements when needed on favorable terms or at all. Our failure to raise capital or enter into such other arrangements when needed would have a negative impact on our financial condition and ability to develop our product candidates. Our recurring losses from operations and negative cash flows raise substantial doubt about our ability to continue as a going concern and, as a result, our independent registered public accounting firm included an explanatory paragraph in its report on our financial statements as of and for the year ended December 31, 2012 with respect to this uncertainty.

In June, July and August 2013, we issued convertible promissory notes in an aggregate principal amount of $23.7 million to certain existing stockholders and new investors. See Liquidity and Capital Resources 2013 Convertible Note Financings for additional details regarding these transactions.

Financial Operations Overview

We conduct substantially all of our activities through Fate Therapeutics, Inc., a Delaware corporation, at our facility in San Diego, California. Fate Therapeutics, Inc. owns 100% of the voting shares of Fate Canada that were outstanding at June 30, 2013 and directs all of its operational activities, which are insignificant as compared to the operations of Fate Therapeutics, Inc. The following information is presented on a consolidated basis to include the accounts of Fate Therapeutics, Inc. and Fate Canada. All intercompany transactions and balances are eliminated in consolidation.

Revenue

To date, we have not generated any revenues from therapeutic product sales. Our revenues have been derived from collaboration activities and grant revenues.

Collaboration revenues are generated exclusively from our collaboration arrangement with Becton, Dickinson and Company, or BD. In September 2010, we entered into a worldwide exclusive license and collaboration agreement with BD for the joint development and worldwide commercialization of certain induced pluripotent stem cell, or iPSC, tools and technologies for use in drug discovery and development. The license and collaboration agreement was assigned by BD to Corning Incorporated in October 2012. In connection with the agreement, we received an upfront, non-refundable license payment, and are entitled to receive research funding for the conduct of joint development activities for a period of three years ending in September 2013. In addition, we are eligible to receive certain commercialization milestones and royalties on the sale of iPSC reagent products. In connection with the arrangement with BD, we recognized $0.8 million, $1.3 million, $0.9 million and $0.4 million for the years ended December 31, 2011 and 2012 and the six months ended June 30, 2012 and 2013, respectively, as collaboration revenue in our consolidated statements of operations. Our three-year joint development period under our license and collaboration agreement with BD concluded in September 2013. We do not currently anticipate generating any significant revenues associated with iPSC tools and technologies thereafter.

Grant revenue is primarily generated through research and development grant programs offered by the U.S. government and its agencies. In April 2011, we were awarded a $2.1 million grant from the U.S. Army Telemedicine & Advanced Technology Research Center, or TATRC, to identify and develop regenerative medicines for acute sound-inducing hearing loss. All funding under the TATRC grant was expended in full as

of May 2013.

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Research and Development Expenses

Research and development expenses consist of development costs associated with our platforms and programs. These costs are expensed as incurred and include:

- compensation and employee-related costs;
- costs associated with conducting our preclinical, clinical and regulatory activities, including fees paid to third-party professional consultants and service providers;
- costs incurred under clinical trial agreements with investigative sites;
- costs for laboratory supplies;
- costs to acquire, develop and manufacture preclinical study and clinical trial materials;
- charges associated with the achievement of certain preclinical and financial milestones pursuant to our asset acquisition of Verio Therapeutics Inc., or Verio, that was completed in April 2010; and
- facilities, depreciation and other expenses including allocated expenses for rent and maintenance of facilities.

From inception through June 30, 2013, we incurred $50.6 million in research and development expenses. We plan to increase our current level of research and development expenses for the foreseeable future as we continue the development of our stem cell modulation platforms and our initial therapeutic product candidates. Our current planned research and development activities include the following:

- advancing ProHema in a Phase 2 clinical trial in the setting of adult patients with orphan hematologic malignancies in 2014 to examine its safety and its curative potential in allogeneic HSCT;
- initiating in 2014 a clinical trial of a pharmacologically-modulated HSC product candidate in pediatric patients with lysosomal storage disorders, or LSDs, to evaluate its safety and its curative potential in allogeneic HSCT; and
- conducting IND-enabling studies, filing an IND in 2014 and initiating a clinical trial of a Wnt7a protein analog product candidate to evaluate its safety and its potential to promote muscle regeneration.

We cannot determine with certainty the timing of initiation, the duration and the completion costs of current or future preclinical studies and clinical trials of our therapeutic product candidates. At this time, due to the inherently unpredictable nature of preclinical and clinical development and given the early stage of our programs, we are unable to estimate with any certainty the costs we will incur and the timelines we will require in the continued development of our product candidates, including ProHema. Clinical and preclinical development timelines, the probability of success and development costs can differ materially from expectations. In addition, we cannot forecast which product candidates may be subject to future collaborations, when such arrangements will be secured, if at all, and to what degree such arrangements would affect our development plans and capital requirements.

The following table summarizes our research and development expenses by major programs for the periods indicated:

	Years Ended December 31,		Six Months Ended June 30,	
	2011	2012	2012	2013
			(unaudited)	
	(in thousands)			
HSC modulation platform	$ 3,084	$ 5,869	$ 2,291	$ 2,514
Other preclinical programs and technologies	3,379	3,589	1,833	1,751
Total direct research and development expenses	6,463	9,458	4,124	4,265
Unallocated expenses	3,395	2,541	1,157	1,333
Total research and development expenses	$ 9,858	$ 11,999	$ 5,281	$ 5,598

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Prior to 2011, we did not track research and development expenses by program. We do not allocate general equipment and supply costs, or facilities, depreciation and other miscellaneous expenses to specific programs as these expenses are deployed across all of our programs.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries and employee-related costs, including stock-based compensation and travel expenses for our employees in executive, operational, finance and human resource functions. Other general and administrative expenses include facility-related costs and professional fees for directors, accounting and legal services and expenses associated with obtaining and maintaining patents.

We anticipate that our general and administrative expenses will increase in the future as we increase our headcount to support our continued research and development and potential commercialization of our product candidates. We also anticipate increased expenses related to audit, legal, regulatory, and tax-related services associated with maintaining compliance with exchange listing and SEC requirements, director and officer insurance premiums, and investor relations costs associated with being a public company.

Other Income (Expense), Net

Other income (expense), net consists primarily of interest income earned on cash and cash equivalents; interest expense on convertible notes and on amounts outstanding under our credit facility; change in fair value of the exchangeable share liability relating to the total exchangeable shares held by the prior stockholders of Verio; change in fair value of the warrant liability relating to our outstanding preferred stock warrants; and amounts received related to a therapeutic discovery project tax credit under Section 48D of the Internal Revenue Code of 1986, as amended, or the Code.

Critical Accounting Policies and Significant Judgments and Estimates

Our management s discussion and analysis of our financial condition and results of operations are based on our financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles. The preparation of these financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, and expenses and the disclosure of contingent assets and liabilities in our financial statements. On an ongoing basis, we evaluate our estimates and judgments, including those related to accrued expenses and stock-based compensation. We base our estimates on historical experience, known trends and events, and various other factors that are believed to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are described in more detail in the notes to our financial statements appearing elsewhere in this prospectus, we believe the following accounting policies to be most critical to the judgments and estimates used in the preparation of our financial statements.

Revenue Recognition

Our revenues have principally consisted of license fees, periodic research and development funding and milestone payments under our September 2010 license and collaboration agreement with BD, as well as funding received under government grants. Our license and collaboration agreement contains multiple elements, all of which are accounted for as collaboration revenue. We recognize revenues when all four of the following criteria are met: (i) persuasive evidence that an agreement exists; (ii) delivery of the products and/or services has occurred; (iii) the selling price is fixed or determinable; and (iv) collectibility is reasonably assured.

Collaboration Revenues

Agreements entered into prior to 2011. For multiple-element agreements entered into prior to January 1, 2011 and not materially modified thereafter, such as our agreement with BD, we analyzed the agreement to

determine whether the elements within the agreement could be separated or whether they must be accounted for as a single unit of accounting. If the delivered element, which for us is commonly a license, has stand-alone value and the fair value of the undelivered elements, which for us are generally collaboration research activities, can be determined, we recognized revenue separately under the residual method as the elements under the agreement are delivered. If the delivered element does not have stand-alone value or if the fair value of the undelivered element cannot be determined, the agreement is then accounted for as a single unit of accounting, with consideration received under the agreement recognized as revenue on the straight-line basis over the estimated period of performance, which for us is generally the expected term of the research and development plan.

Agreements entered into or materially modified after December 31, 2010. In October 2009, the Financial Accounting Standards Board, or FASB, issued a new accounting standard which amended the guidance on accounting for arrangements involving the delivery of more than one element. This standard addresses the determination of the unit(s) of accounting for multiple-element arrangements and how the arrangement s consideration should be allocated to each unit of accounting. In January 2011, we adopted new authoritative guidance on revenue recognition for milestone payments related to agreements under which we have continuing performance obligations. As required under the new literature, we evaluate all milestones at the beginning of the agreement to determine if they meet the definition of a substantive milestone.

We recognize revenue from milestone payments when earned, provided that (i) the milestone event is substantive in that it can only be achieved based in whole or in part on either our performance or on the occurrence of a specific outcome resulting from our performance and its achievability was not reasonably assured at the inception of the agreement; (ii) we do not have ongoing performance obligations related to the achievement of the milestone and (iii) it would result in the receipt of additional payments. A milestone payment is considered substantive if all of the following conditions are met: (i) the milestone payment is non-refundable; (ii) achievement of the milestone was not reasonably assured at the inception of the arrangement; (iii) substantive effort is involved to achieve the milestone and (iv) the amount of the milestone payment appears reasonable in relation to the effort expended, the other milestones in the arrangement and the related risk associated with the achievement of the milestone.

Collaboration arrangements providing for payments to us upon the achievement of research and development milestones generally involve substantial uncertainty as to whether any such milestone would be achieved. In the event a milestone is considered to be substantive, we expect to recognize future payments as revenue in connection with the milestone as it is achieved. Collaboration arrangements providing for payments to us upon the achievement of milestones that are solely contingent upon the performance of a collaborator also involve substantial uncertainty as to whether any such milestone would be achieved. For such contingent milestones, even if they do not meet the definition of a substantive milestone, since they are based solely upon a collaborator s effort, we expect to recognize future payments as revenue when earned under the applicable arrangement, provided that collection is reasonably assured.

Government Grant Revenue

Revenue from government grants is recorded when reimbursable expenses are incurred under the grant in accordance with the terms of the grant award. The receivable for reimbursable amounts that have not been collected is reflected in prepaid and other current assets on our consolidated balance sheets.

Deferred Revenue

Amounts received prior to satisfying the above revenue recognition criteria are recorded as deferred revenue. Amounts not expected to be recognized within the next 12 months are classified as non-current deferred revenue on our consolidated balance sheets.

Accrued Research and Development Expenses

As part of the process of preparing our financial statements, we are required to estimate our accrued expenses. This process involves reviewing open contracts and purchase orders, communicating with our personnel to identify services that have been performed on our behalf and estimating the level of service

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performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of the actual cost. The majority of our service providers invoice us monthly in arrears for services performed or when contractual milestones are met. We make estimates of our accrued expenses as of each balance sheet date in our financial statements based on facts and circumstances known to us at that time. We periodically confirm the accuracy of our estimates with the service providers and make adjustments if necessary. Examples of estimated accrued research and development expenses include fees paid to investigative sites in connection with clinical trials; service providers in connection with preclinical development activities; and service providers related to product manufacturing, development and distribution of clinical supplies.

We base our expenses related to clinical trials on our estimates of the services received and efforts expended pursuant to our contract arrangements. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to our service providers will exceed the level of services provided and result in a prepayment of the clinical expense. Payments under some of these contracts depend on factors such as the successful enrollment of patients and the completion of clinical milestones. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from our estimate, we adjust the accrual or prepaid accordingly.

Although we do not expect our estimates to be materially different from amounts actually incurred, if our estimates of the status and timing of services performed differs from the actual status and timing of services performed, we may report amounts that are too high or too low in any particular period. To date, there have been no material differences from our estimates to the amount actually incurred.

Stock-Based Compensation

Stock-based compensation expense represents the grant date fair value of employee stock option grants recognized over the requisite service period of the awards (usually the vesting period) on a straight-line basis, net of estimated forfeitures. For stock option grants with performance-based milestones, the expense is recorded over the remaining service period after the point when the achievement of the milestone is probable or the performance condition has been achieved. For stock option grants with both performance-based milestones and market conditions, expense is recorded over the derived service period after the point when the achievement of the performance-based milestone is probable or the performance condition has been achieved. We estimate the fair value of stock option grants using the Black-Scholes option pricing model, with the exception of option grants with both performance-based milestones and market conditions, which are valued using a lattice based model.

We account for stock options and restricted stock awards to non-employees using the fair value approach. Stock options and restricted stock awards to non-employees are subject to periodic revaluation over their vesting terms. For stock option grants with performance-based milestones, the expense is recorded over the remaining service period after the point when the performance condition has been achieved.

We generally estimate the fair value of our stock-based awards to employees and non-employees using the Black-Scholes option pricing model, which requires the input of highly subjective assumptions, including (a) the risk-free interest rate, (b) the expected volatility of our stock, (c) the expected term of the award and (d) the expected dividend yield. Due to the lack of a public market for the trading of our common stock and a lack of company specific historical and implied volatility data, we have based our estimate of expected volatility on the historical volatility of a group of similar companies that are publicly traded. For these analyses, we have selected companies with comparable characteristics to ours including enterprise value, risk profiles, position within the industry, and with historical share price information sufficient to meet the expected life of the stock-based awards. We compute the historical volatility data using the daily closing prices for the selected companies shares during the equivalent period of the calculated expected term of our stock-based awards. We will continue to apply this process until a sufficient amount of historical information regarding the volatility of our own stock price becomes available. We have estimated the expected life of our employee stock options using the simplified method, whereby, the expected life equals the average of the vesting term and the original contractual term of the option. The risk-free interest rates for periods within the expected life of the option are based on the yields of zero-coupon U.S. Treasury securities.

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The weighted-average assumptions used in the Black-Scholes option pricing model to determine the fair value of employee stock option grants were as follows:

	Years Ended December 31,		Six Months Ended June 30,	
	2011	2012	2012	2013
Risk-free interest rate	1.1%	1.0%	1.0%	1.1%
Expected volatility	90%	94%	94%	90%
Expected term (in years)	6.1	6.1	6.1	6.1
Expected dividend yield	0.0%	0.0%	0.0%	0.0%

The weighted-average assumptions used in the Black-Scholes option pricing model to determine the fair value of non-employee stock option grants were as follows:

	Years Ended December 31,		Six Months Ended June 30,	
	2011	2012	2012	2013
Risk-free interest rate	1.1%	1.2%	1.2%	1.5%
Expected volatility	90%	94%	94%	90%
Expected term (in years)	6.1	7.5	6.9	8.1
Expected dividend yield	0.0%	0.0%	0.0%	0.0%

The following table summarizes by grant date the number of shares of common stock underlying stock options granted from January 1, 2012 through August 12, 2013, as well as the associated per share exercise price and the estimated fair value per share of our common stock on the grant date:

Grant Dates	Number of Common Shares Underlying Options Granted	Exercise Price per Common Share	Estimated Fair Value per Common Share
February 9, 2012	248,027	$ 1.63	$ 1.63
March 13, 2012	31,563	1.63	1.63
March 23, 2012	57,308	1.63	1.63
July 24, 2012	123,872	1.37	1.37
October 10, 2012	701,214	1.37	1.37
December 12, 2012	26,923	1.37	1.37
January 14, 2013	19,154	1.37	1.37
February 6, 2013	56,923	1.37	1.37
May 13, 2013	41,538	1.63	1.63
August 12, 2013	214,257	7.87	7.87

Total employee stock-based compensation expense related to unvested stock option grants not yet recognized as of June 30, 2013 was approximately $0.7 million and the weighted-average period over which these grants are expected to vest is 2.9 years.

Based on the initial public offering price of $6.00 per share, the intrinsic value of stock options outstanding as of June 30, 2013 would be $6.9 million, of which $1.1 million and $5.8 million would have been related to stock options that were vested and unvested, respectively, at that date.

Determination of the Fair Value of Common Stock

We are required to estimate the fair value of the common stock underlying our stock-based awards when performing fair value calculations. The fair value of the common stock underlying our stock-based awards was determined on each grant date by our board of directors, taking into account input from management and independent third-party valuation analysis. All options to purchase shares of our common stock are intended to be granted with an exercise price per share no less than the fair value per share of our common stock underlying

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those options on the date of grant, based on the information known to us on the date of grant. In the absence of a public trading market for our common stock, on each grant date we develop an estimate of the fair value of our common stock in order to determine an exercise price for the option grants. Our determinations of the fair value of our common stock were made using methodologies, approaches and assumptions consistent with the American Institute of Certified Public Accountants, or AICPA, Audit and Accounting Practice Aid Series: Valuation of Privately Held Company Equity Securities Issued as Compensation, or the Practice Aid.

Our board of directors considered various objective and subjective factors, along with input from management, to determine the fair value of our common stock, including:

- contemporaneous valuations prepared by an independent third-party valuation specialist effective as of August 31, 2011, July 3, 2012, March 31, 2013, June 30, 2013 and August 12, 2013;
- the prices of our convertible preferred stock sold to investors in arm s length transactions, and the rights, preferences and privileges of our convertible preferred stock as compared to those of our common stock, including the liquidation preferences of our convertible preferred stock;
- our results of operations, financial position and the status of research and development efforts and achievement of enterprise milestones;
- the composition of, and changes to, our management team and board of directors;
- the lack of liquidity of our common stock as a private company;
- our stage of development and business strategy and the material risks related to our business and industry;
- the valuation of publicly traded companies in the life sciences and biotechnology sectors, as well as recently completed mergers and acquisitions of peer companies;
- external market conditions affecting the life sciences and biotechnology industry sectors;
- the likelihood of achieving a liquidity event for the holders of our common stock, such as an initial public offering, or IPO, or a sale of our company, given prevailing market conditions; and
- the state of the IPO market for similarly situated privately held biotechnology companies.

There are significant judgments and estimates inherent in the determination of the fair value of our common stock. These judgments and estimates include assumptions regarding our future operating performance, the time to completing an IPO or other liquidity event and the determination of the appropriate valuation methods. If we had made different assumptions, our stock-based compensation expense, net loss and net loss per common share could have been significantly different.

Common Stock Valuation Methodologies

Our valuations were prepared in accordance with the guidelines in the Practice Aid, which prescribes several valuation approaches for setting the value of an enterprise, such as the cost, income and market approaches, and various methodologies for allocating the value of an enterprise to its

common stock. The cost approach establishes the value of an enterprise based on the cost of reproducing or replacing the property less depreciation and functional or economic obsolescence, if present. The income approach establishes the value of an enterprise based on the present value of future cash flows that are reasonably reflective of our company s future operations, discounting to the present value with an appropriate risk adjusted discount rate or capitalization rate. The market approach is based on the assumption that the value of an asset is equal to the value of a substitute asset with the same characteristics. The following market approaches were utilized in our various valuations:

Guideline public company method. The guideline public company market approach estimates the value of a business by comparing a company to similar publicly-traded companies.

Guideline transaction method. The guideline transaction market approach estimates the value of a business based on valuations from selected mergers and acquisitions transactions for companies with similar characteristics.

Precedent transaction method. The precedent transaction market approach estimates the value of a business based on the utilization of a company s own relevant stock transactions.

Each valuation methodology was considered in our valuations. We elected not to utilize the cost approach in any of our valuations since our value relates primarily to our intangible assets.

Methods Used to Allocate Our Enterprise Value to Classes of Securities

In accordance with the Practice Aid, we considered the various methods for allocating the enterprise value across our classes and series of capital stock to determine the fair value of our common stock at each valuation date. The methods we considered consisted of the following:

Current value method. Under the current value method, once the fair value of the enterprise is established, the value is allocated to the various series of preferred and common stock based on their respective seniority, liquidation preferences or conversion values, whichever is greatest. This method was considered but not utilized in any of the valuations discussed below.

Option pricing method. Under the option pricing method, shares are valued by creating a series of call options with exercise prices based on the liquidation preferences and conversion terms of each equity class. The values of the preferred and common stock are inferred by analyzing these options.

Probability-weighted expected return method, or PWERM. The PWERM is a scenario-based analysis that estimates the value per share based on the probability-weighted present value of expected future investment returns, considering each of the possible outcomes available to us, as well as the economic and control rights of each share class.

February 2012 and March 2012 grants. On each of February 9, 2012, March 13, 2012 and March 23, 2012, our board of directors determined that the fair value of our common stock was $1.63 per share in connection with the grant of stock options. As part of each determination, our board of directors concluded that no significant internal or external value-generating events had taken place between the August 2011 valuation analysis and the dates of these stock option grants.

The common stock fair value in August 2011 was estimated to be $1.63 per share by our board of directors, with input from both management and an independent third-party valuation specialist. It was believed that a precedent transaction market approach was most reliable to determine our enterprise value because we completed a third closing of our Series B preferred stock financing in March 2011 with a new strategic investor. The guideline public company market approach and the guideline transaction market approach were also considered, but not utilized due to our early stage of development. We did not identify any major operational events between March 2011 and August 2011 that would cause a change in our overall enterprise value.

The option pricing method was utilized to allocate the enterprise value to our common stock. It was determined that the option pricing method was the most reliable given the expectation of various potential liquidity outcomes and the difficulty of selecting and supporting appropriate enterprise values given our early stage of development and financial position. The calculation of the fair value of our common stock included a probability of success factor of 35% based on success rates for drugs entering clinical trials as reported in the 2011 Pharmaceutical Industry Profile issued by the Pharmaceutical Research and Manufacturers of America; a discount for lack of control of 5% due to the protective provisions afforded the preferred stockholders that were not afforded to the common stockholders; and a discount for lack of marketability, or DLOM, of 50% based on several empirical restricted stock studies and mathematical models for calculating illiquidity discounts. Because the enterprise value was established relative to the sale price of an illiquid security, the DLOM reflected only an incremental discount for lack of marketability attributed to the illiquidity of the common stock relative to that of the Series B preferred stock.

July 2012 valuation and grants. The common stock fair value was estimated by our board of directors to be $1.37 per share in July 2012, with input from both management and an independent third-party valuation specialist, in connection with the grant of stock options. The fair value per share of $1.37 represented a decrease of $0.26 per share from the $1.63 per share utilized for the March 2012 option grants. The decrease in fair value was primarily related to our issuance in May 2012 of Series C preferred stock at a price per share reflecting an

enterprise value below that of our most recent preferred stock financing. This valuation utilized a market approach to determine our enterprise value. It was believed that a precedent transaction market approach was most reliable to determine our enterprise value since we completed closings of our Series C preferred stock financing in May and July 2012. The guideline public company market approach and the guideline transaction market approach were also considered, but not utilized due to our early stage of development.

We issued an aggregate of 9,244,679 shares of Series C convertible preferred stock at $1.00 per share in the May 2012 and July 2012 closings. The Series C preferred stock financing included what is commonly referred to as a pay-to-play provision, pursuant to which each existing holder of over 750,000 shares of our convertible preferred stock was required to participate, on a pro rata preferred stock ownership basis, in the Series C preferred stock financing. If such a stockholder elected not to participate in the Series C preferred stock financing, every ten shares of convertible preferred stock held by such stockholder would be converted into one share of common stock. Two of the nine investors subject to the pay-to-play provision elected not to participate in the Series C preferred stock financing including one investor, which owned approximately 13.0% of the outstanding convertible preferred stock, and a second investor, which owned approximately 4.6% of the outstanding convertible preferred stock, prior to such Series C preferred stock financing.

The option pricing method was utilized to allocate the enterprise value to our common stock. It was determined that the option pricing method was the most reliable given the expectation of various potential liquidity outcomes and the difficulty of selecting and supporting appropriate enterprise values given our early stage of development and financial position. The calculation of the fair value of our common stock included a probability of success factor of 65% based on success rates for drugs in clinical trials as reported in the 2011 Pharmaceutical Industry Profile issued by the Pharmaceutical Research and Manufacturers of America, where the probability of success was increased from 35% in the August 2011 valuation due to the continued clinical progression of ProHema; a discount for lack of control of 5% due to the protective provisions afforded the preferred stockholders that were not afforded to our common stockholders; and a DLOM of 25% based on several empirical restricted stock studies and mathematical models for calculating illiquidity discounts. Because the enterprise value was established relative to the sale price of an illiquid security, the DLOM reflected only an incremental discount for lack of marketability attributed to the illiquidity of the common stock relative to that of the Series C convertible preferred stock. The decline in DLOM relative to prior fair value estimates reflects a reduction in the expected time to a liquidity event.

October 2012, December 2012, January 2013 and February 2013 grants. On each of October 10, 2012, December 12, 2012, January 14, 2013 and February 6, 2013, our board of directors determined that the fair value of our common stock was $1.37 per share in connection with the grant of stock options. As part of this determination, our board of directors concluded that no significant internal or external value-generating events had taken place between the July 2012 valuation analysis date and the dates of these stock option grants.

March 2013 valuation. The common stock fair value was estimated to be $1.63 per share in March 2013, with input from both management and an independent third-party valuation specialist. The fair value per share of $1.63 represented an increase of $0.26 per share from the $1.37 per share utilized for the February 2013 option grants. The significant internal and external events between February 2013 and March 2013 that either directly impacted our estimated enterprise value or contributed to our updated conclusions regarding the timing and nature of future liquidity events were as follows:

- In February and March 2013, we met with several investment banks to evaluate the IPO market for emerging biotechnology companies and to explore our potential to pursue an IPO;

- In February and March 2013, we were introduced to several public market investors and obtained their feedback on our financing alternatives; and

- In March 2013, we completed enrollment of, and initiated the collection of monitored data on, the first eight patients in our Phase 2 clinical trial of ProHema.

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To determine our enterprise value, our valuation utilized both an income approach based on a discounted cash flow method and a market approach based on several methods including a guideline public company market approach, a guideline transaction market approach and a precedent transaction market approach. In addition, we utilized the PWERM to determine the per share common stock value using the following probability-weighted liquidity event scenarios:

Scenario	Weighting
IPO using Guideline Public Company Market Approach	10%
Merger or Sale using Income Approach and Guideline Transaction Market Approach(1)	10%
Liquidity Event using Precedent Transaction Market Approach	40%
No Value to Common	40%
Total	100%

(1) Based on equal weighting of each approach.

The change in valuation methodologies was made because we believed that there was a higher probability of a liquidity event in the following 12 to 18 months. For each liquidity event scenario under the PWERM, the rights and preferences of each class of our capital stock were considered in order to determine the appropriate allocation of our enterprise value to the shares of our common stock.

At the time of the March 2013 valuation, we considered the potential of an IPO in late 2013, as this opportunity to raise capital was then considered a possibility based on our stage of development. However, there continued to be a significant likelihood that success would not be achieved for our Phase 2 clinical trial of ProHema, and that we would be unable to raise additional capital in order to sustain operations including through the completion of our Phase 2 clinical trial or to an IPO. For the IPO liquidity event scenario, we used pre-money IPO valuations of recent initial public offerings of biotechnology companies, under the guideline public company market approach, to determine our enterprise value. We reduced this median implied value by (i) the cost of completing an IPO and (ii) the estimated amount of bridge financing required to complete an IPO, and then calculated the common stock value on a fully diluted basis. We then discounted the common stock value to present value using a cost of capital of 25%, based on several empirical studies assessing cost of capital for venture-backed pre-IPO companies and applied a DLOM of 20% based on several empirical restricted stock studies and mathematical models for calculating illiquidity discounts. Because the enterprise value in the IPO liquidity event scenario was established relative to the sale price of registered common stock on the eve of public trading, the DLOM reflected only an incremental discount for lack of marketability attributed to the illiquidity of unregistered common stock.

We also considered the potential of a merger or sale. However, there continued to be a significant likelihood that success would not be achieved for our Phase 2 clinical trial of ProHema, and that we would be unable to raise additional capital in order to sustain operations including through the completion of our Phase 2 clinical trial or to a merger or sale. For the merger or sale liquidity event scenario, we used the income approach based on a discounted cash flow method and the market approach based on a guideline transaction market approach, giving equal weight to both approaches, to determine our enterprise value. The discounted cash flow method was based on management forecasts for our company under the assumption that we would succeed with our clinical trials and product commercialization. The guideline transaction market approach was based on the enterprise price paid in emerging pharmaceutical and biotechnology acquisitions between 2011 and 2012, where the enterprise price paid included any contingent consideration at its minimum and maximum values. Based on an equal weighting of these two approaches (income and market), the option pricing method was utilized to allocate the enterprise value to the shares of our common stock. To determine the fair value of our common stock, a DLOM of 50% was used based on several empirical restricted stock studies and mathematical models for calculating illiquidity discounts. Since neither market approach accounted for the illiquidity of the common stock, the DLOM represented the full discount for lack of marketability of the common stock.

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We also utilized a precedent transaction market approach because we completed a second tranche of our Series C preferred stock financing in October 2012. We issued 7,563,825 shares of Series C convertible preferred stock at $1.00 per share in the October 2012 closing. The option pricing method was utilized to allocate the enterprise value to our common stock. It was determined that the option pricing method was the most reliable given the expectation of various potential liquidity outcomes and the difficulty of selecting and supporting appropriate exit values given our early stage of development and financial position. To determine the fair value of our common stock, a discount for lack of control of 5% was used due to the protective provisions afforded the preferred stockholders not afforded to the common stockholders, and a DLOM of 30% was used based on several empirical restricted stock studies and mathematical models for calculating illiquidity discounts. Because the enterprise value in the precedent transaction market approach was established relative to the sale price of an illiquid security, the DLOM reflected only an incremental discount for lack of marketability attributed to the illiquidity of the common stock relative to that of the Series C convertible preferred stock.

Finally, we utilized the no value to common scenario that contemplated circumstances resulting from a failure of our Phase 2 clinical trial of ProHema or from our inability to raise additional funding in order to sustain operations. For the no value to common scenario, we used the precedent transaction market approach and an assumed liquidation for net asset value, giving equal weight to both approaches, to determine our enterprise value. This scenario assumes a liquidation of the business, where our preferred stockholders would recover a portion of their original investment through a sale of our assets, but no value would remain available for distribution to holders of our common stock.

May 2013 grants. On May 13, 2013, our board of directors determined that the fair value of our common stock was $1.63 per share in connection with the grant of stock options. As part of this determination, our board of directors concluded that no significant internal or external value-generating events had taken place between the March 2013 valuation analysis date and the date of these stock option grants.

June 2013 valuation. The fair value of our common stock was estimated to be $4.49 per share in June 2013, with input from both management and an independent third-party valuation specialist. The fair value per share of $4.49 represented an increase of $2.86 per share from the March 2013 valuation. The significant internal and external events between May 2013 and June 2013 that either directly impacted our estimated enterprise value or contributed to our updated conclusions regarding the timing and nature of future liquidity events were as follows:

In the second quarter of 2013, we completed a series of *in vitro* and *in vivo* studies demonstrating that the potency and efficacy profile of ProHema can be significantly improved using our NRM formulation, and we established our internal timeline for amending our IND to incorporate our NRM formulation for the manufacture of ProHema;

In late May 2013, we selected a lead banker for a potential IPO effort and held an organizational meeting to formally initiate the IPO process;

On June 20, 2013, we confidentially submitted to the SEC a draft registration statement on Form S-1 for an IPO; and

On June 24, 2013, we completed a bridge financing in which we sold $3.0 million in convertible notes to certain existing investors.

As of June 30, 2013, as a result of the factors described above, we updated the weighting of the scenarios used in our PWERM as follows:

Scenario	Weighting
IPO using Guideline Public Company Market Approach	50%
Merger or Sale using Income Approach and Guideline Transaction Market Approach[(1)]	10%
Liquidity Event using Precedent Transaction Market Approach	20%
No Value to Common	20%
Total	100%

(1) Based on equal weighting of each approach.

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Based on the events described above, at the time of the June 2013 valuation, we considered the potential of an IPO in late 2013 to have substantially increased from the time of our March 2013 valuation. Additionally, we believed that the IPO market for emerging biotechnology companies significantly improved during the second quarter of 2013 because over ten emerging biotechnology companies completed IPOs during this three-month period. The increased weighting on the IPO scenario using a guideline public company market approach from 10% in March 2013 to 50% in June 2013 was balanced by a decrease in weighting of the liquidity event scenario using a precedent transaction market approach from 40% in March 2013 to 20% in June 2013, and a decrease in the weighting of the no value to common scenario from 40% in March 2013 to 20% in June 2013. While the aggregate weighting for these scenarios decreased from 80% to 40%, we believed that a range of liquidity outcomes occurring beyond 2013, including liquidity outcomes that would result in no value to the common stock, was still probable because the filing of our IND amendment with the FDA and our ability to resume enrollment of our Phase 2 clinical trial of ProHema remained uncertain.

To determine our enterprise value, we continued to use the same approaches described above in the discussion of our March 2013 valuation. Although we continued to use the median implied value to set the enterprise value under the guideline public company market approach, we noted a 35% increase in our estimated enterprise value based on the valuations of the underlying market comparable transactions. The enterprise values derived from the income approach, guideline transaction market approach, precedent transaction market approach and no value to common as of June 30, 2013 remained substantially consistent with the values used in our March 2013 valuation. In addition, the various assumptions regarding DLOM, cost of capital and discounts for lack of control remained substantially the same as of March 2013 and June 2013.

August 2013 valuation and grants. The fair value of our common stock was estimated by our board of directors to be $7.87 per share on August 12, 2013, with input from both management and an independent third-party valuation specialist, in connection with the grant of stock options. The fair value per share of $7.87 represented an increase of $3.38 per share from the June 2013 valuation. The significant internal and external events between June 2013 and August 2013 that either directly impacted our estimated enterprise value or contributed to our updated conclusions regarding the timing and nature of future liquidity events were as follows:

- On August 1, 2013, we submitted an IND amendment to the FDA, which contained preclinical and product development data to support the incorporation of our NRM formulation for the manufacture of ProHema and that the use of the NRM formulation should not result in additional safety risks. In addition, we submitted an amended protocol for our Phase 2 clinical trial of ProHema that defines how we will resume enrollment with ProHema as manufactured using our NRM formulation; and

- On August 8, 2013, we completed a bridge financing in which we sold $20.0 million in convertible notes to certain new investors advised by Fidelity Management & Research Company.

As of August 12, 2013, as a result of the factors noted above, we updated the weighting of the scenarios used in our PWERM as follows:

Scenario	Weighting
IPO using Guideline Public Company Market Approach	70%
Merger or Sale using Income Approach and Guideline Transaction Market Approach [(1)]	10%
No Value to Common	20%
Total	100%

(1) Based on equal weighting of each approach.

Based on the events described above, at the time of the August 2013 valuation, we considered the potential of an IPO in 2013 to have substantially increased from the time of our June 2013 valuation. We also believed that the IPO market for emerging biotechnology companies remained robust. Therefore, we increased the weighting

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on the IPO scenario using a guideline public company market approach from 50% in June 2013 to 70% in August 2013. This increase was balanced by a decrease in weighting of the liquidity event scenario using a precedent transaction market approach, which considered a range of liquidity outcomes occurring beyond 2013, from 20% in June 2013 to 0% in August 2013. We believed a liquidity outcome that would result in no value to the common stock was still possible due to the uncertainty involving the FDA s response to our IND amendment and, therefore, our ability to resume enrollment of our Phase 2 clinical trial of ProHema.

To determine our enterprise value, we continued to use the same approaches described above in the discussion of our June 2013 valuation. Although we continued to use the median implied value to set the enterprise value under the guideline public company market approach, we noted an 11% increase in our estimated enterprise value based on the valuations of the underlying market comparable transactions. Additionally, we decreased the DLOM from 20% in June 2013 to 15% in August 2013 under the guideline public company market approach to reflect our more aggressive internal timeline for the completion of an IPO. We also noted a 40% increase in our estimated enterprise value derived from the income approach and the guideline transaction market approach, which increase primarily resulted from our additional cash resources as of the August 2013 valuation date. Under the income and guideline transaction market approaches, we decreased the DLOM from 50% in June 2013 to 30% in August 2013 to reflect a reduction in our expected time to liquidity. The no value to common scenario remained substantially unchanged from our June 2013 valuation.

Initial public offering price

Our initial public offering price is $6.00 per share. In comparison, our estimate of the fair value of our common stock was determined to be $7.87 per share as of August 12, 2013 using a contemporaneous valuation prepared by management and an independent third-party valuation specialist.

Warrant Liability

Freestanding warrants for the purchase of convertible preferred stock are classified as liabilities on the consolidated balance sheets at their estimated fair value since the underlying convertible preferred stock has been classified as temporary equity. At the end of each reporting period, changes in the estimated fair value during the period are recorded as a component of other income (expense). We will continue to adjust the fair value of these warrants until the earlier of the exercise of the warrants or the time at which the underlying securities are no longer classified as temporary equity, including the completion of an IPO. We estimate the fair values of the convertible preferred stock warrants using the Black-Scholes option pricing model based on inputs as of the valuation measurement dates for: the estimated fair value of the underlying convertible preferred stock; the remaining contractual terms of the warrants; the risk-free interest rates; the expected dividend yield and the estimated volatility of the price of the convertible preferred stock.

The following assumptions were used in the Black-Scholes option pricing model to determine the fair value of the preferred stock warrant liability:

	As of December 31,		As of June 30,
	2011	2012	2013
Assumed risk-free interest rate	1.9%	1.2%	1.7%
Assumed volatility	90.0%	93.5%	85.9%
Remaining contractual term (in years)	9.31	8.31	7.81
Expected dividend yield	0.0%	0.0%	0.0%
Series B preferred stock	$ 2.00	$ 0.92	$ 1.03
Series C preferred stock	$	$ 0.99	$ 1.09

The above assumptions remained relatively consistent for the periods presented as a result of only minor changes in the remaining contractual term of the underlying warrants due to the passage of time, as well as minor changes in the underlying volatility and risk-free interest rate. The fair values per share of our underlying preferred stock were estimated using the same methodologies described above for the valuation of our common stock.

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The completion of this offering will result in the conversion of our convertible preferred stock into common stock and the warrants will become exercisable into common stock. Upon such conversion, the preferred stock warrants will be classified as a component of stockholders equity (deficit) and will no longer be subject to remeasurement. Based on the initial public offering price of $6.00 per share, and assuming all other inputs into our valuation model remain unchanged from those as of June 30, 2013, we do not expect to record a charge to adjust the warrant liability to its then-current fair value upon the closing of our initial public offering.

Exchangeable Share Liability

In April 2010, we acquired Verio Therapeutics Inc., or Verio, a development stage company headquartered in Ottawa, Ontario. In connection with the acquisition, the stockholders of Verio received 900,000 non-voting shares of Fate Canada that were initially exchangeable into 138,462 shares of our common stock and, subject to the validation of certain scientific data and the achievement of certain preclinical, clinical, commercial and financial milestones, may be exchangeable for up to 884,605 shares of our common stock. As of June 30, 2013, these shares were exchangeable for 403,841 shares of our common stock upon the completion of an IPO. Additionally, the holders of the exchangeable shares of Fate Canada may be entitled to receive up to 480,764 shares of our common stock upon the satisfaction of applicable performance milestones or the occurrence of a change of control. Any issuance of such shares will be recorded as research and development expense based on the then-current fair value of our common stock. These exchangeable shares are exchangeable into our common stock at any time by the election of the holders of a majority of the exchangeable shares and are further described in Description of Capital Stock Exchangeable Shares in Canadian Subsidiary elsewhere in this prospectus.

Based on our evaluation of the set of activities and assets of Verio, at the acquisition date, we determined that Verio did not meet the definition of a business. In addition, we determined that Verio was a development stage enterprise without any material inputs; without any processes that create, or have the ability to create, outputs; and without any outputs. As such, the Verio acquisition was accounted for as an asset acquisition and we charged the $0.4 million purchase price to research and development expense. The initial purchase price of the Verio assets consisted of $0.2 million of assumed net liabilities and an initial exchangeable share liability of $0.2 million. This amount represents the estimated fair value of purchased in-process technology for projects that, as of the acquisition date, had not yet reached technological feasibility and had no alternative future use.

The fair value of all previously earned exchange shares is re-measured at each reporting date, with any changes in fair value being recognized in the change in fair value of exchangeable share liability, a component of other income (expense), in the accompanying consolidated statements of operations. The fair value of the exchangeable share liability is equal to the fair value of the number of shares of our common stock into which the exchangeable shares would convert. The fair value of our underlying common stock used to determine the fair value of the exchangeable share liability was estimated as described above. We will continue to re-measure the fair value of the exchangeable share liability until the exchange for shares of our common stock is complete. Upon such exchange, the then-current fair value of the exchangeable share liability will be classified as a component of stockholders equity and will no longer be subject to remeasurement. Based on the initial public offering price of $6.00 per share, we would expect to record a charge of approximately $0.6 million to adjust the exchangeable share liability to its then-current fair value upon the closing of our initial public offering.

Subsequent to our initial charge of $0.2 million to research and development expense in 2010 for the exchangeable share liability, we have recorded charges to research and development expense of $0.4 million in 2011 and $0.1 million in 2012 related to increases in the number of shares of common stock issuable upon the exchange of the exchangeable shares of 207,688 shares and 57,691 shares, respectively. We recorded other income (expense) related to the change in fair value of the exchangeable shares for the years ended December 31, 2011 and 2012 and the six months ended June 30, 2012 and 2013 of $(5,000), $0.1 million, $0.1 million and $(1.3) million, respectively.

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Other Company Information

Net Operating Loss Carryforwards

At December 31, 2012, we had federal, California and Canadian net operating loss, or NOL, carryforwards of approximately $34.6 million, $32.2 million and $0.4 million, respectively, which may be available to offset future taxable income. The federal, California and Canadian NOL carryforwards begin to expire in 2027, 2028 and 2029, respectively, unless previously utilized. At December 31, 2012, we had federal and California research and development, or R&D, credit carryforwards of approximately $1.0 million and $1.3 million, respectively. The federal R&D tax credit carryforwards will begin to expire in 2027 unless previously utilized. The California R&D credit carryforwards will carry forward indefinitely.

Utilization of the NOL and R&D credit carryforwards may be subject to a substantial annual limitation due to ownership change limitations that may have occurred or that could occur in the future, as required by Section 382 of the Code, as well as similar state and foreign provisions. These ownership changes may limit the amount of NOL and R&D credit carryforwards that can be utilized annually to offset future taxable income and tax, respectively. In general, an ownership change as defined by Section 382 of the Code results from a transaction or series of transactions over a three-year period resulting in an ownership change of more than 50 percentage points of the outstanding stock of a company by certain stockholders. Since our formation, we have raised capital through the issuance of capital stock on several occasions, which on its own or combined with the purchasing stockholders subsequent disposition of those shares, may have resulted in such an ownership change, or could result in an ownership change in the future.

We have not completed a study to assess whether an ownership change has occurred or whether there have been multiple ownership changes since our formation due to the complexity and cost associated with such a study and the fact that there may be additional such ownership changes in the future. If we have experienced an ownership change at any time since our formation, utilization of the NOL or R&D credit carryforwards would be subject to an annual limitation under Section 382 of the Code, which is determined by first multiplying the value of our stock at the time of the ownership change by the applicable long-term, tax-exempt rate, and then could be subject to additional adjustments, as required. Any limitation may result in expiration of a portion of the NOL or R&D credit carryforwards before utilization. Further, until a study is completed and any limitation known, no amounts are being considered as an uncertain tax position or disclosed as an unrecognized tax benefit. Due to the existence of the valuation allowance, future changes in our unrecognized tax benefits will not impact our effective tax rate. Any carryforwards that will expire prior to utilization as a result of such limitations will be removed from deferred tax assets, with a corresponding reduction of the valuation allowance.

JOBS Act

On April 5, 2012, the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, was enacted. Section 107 of the JOBS Act provides that an emerging growth company can take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act for complying with new or revised accounting standards. In other words, an emerging growth company can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this extended transition period and, as a result, we will adopt new or revised accounting standards on the relevant dates on which adoption of such standards is required for other public companies.

We are in the process of evaluating the benefits of relying on other exemptions and reduced reporting requirements provided by the JOBS Act. Subject to certain conditions set forth in the JOBS Act, as an emerging growth company, we intend to rely on certain of these exemptions, including without limitation, (i) providing an auditor s attestation report on our system of internal controls over financial reporting pursuant to Section 404(b) of the Sarbanes-Oxley Act and (ii) complying with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor s report providing additional information about the audit and the financial statements, known as the auditor discussion and analysis. We will remain an emerging growth company until the earliest of (a) the last day of the fiscal year in which we have total annual gross revenues of $1 billion or more, (b) the last day of our fiscal year

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following the fifth anniversary of the date of the completion of this offering, (c) the date on which we have issued more than $1 billion in non-convertible debt during the previous three years or (d) the date on which we are deemed to be a large accelerated filer under the rules of the SEC.

Recently Adopted Accounting Pronouncements

In February 2013, the FASB issued guidance to provide information about the amounts reclassified out of accumulated other comprehensive income, or AOCI, by component. An entity is required to present, either on the face of the financial statements or in the notes, significant amounts reclassified out of AOCI by the respective line items of net income, but only if the amount reclassified is required to be reclassified in its entirety in the same reporting period. For amounts that are not required to be reclassified in their entirety to net income, an entity is required to cross-reference to other disclosures that provide additional details about those amounts. On January 1, 2013, we adopted this standard, which had no impact on our financial position or results of operations.

In June 2011, the FASB issued an amendment to the accounting guidance for presentation of comprehensive income. Under the amended guidance, a company may present the total of comprehensive income, the components of net income, and the components of other comprehensive income either in a single continuous statement of comprehensive income or in two separate but consecutive statements. In either case, a company is required to present each component of net income along with total net income, each component of other comprehensive income along with a total for other comprehensive income, and a total amount for comprehensive income. The amendment is effective for fiscal years ending, and interim periods within those years, beginning after December 15, 2011, and is applied retrospectively. We adopted this amendment in the accompanying financial statements by presenting comprehensive loss in one consecutive statement along with net loss.

In May 2011, the FASB issued amended guidance on fair value measurements. This newly issued accounting standard clarifies the application of certain existing fair value measurement guidance and expands the disclosures for fair value measurements that are estimated using significant unobservable (Level 3) inputs. This accounting standard was effective on a prospective basis for annual and interim reporting periods beginning on or after December 15, 2011. The adoption of this standard has not had a material impact on our financial position or results of operations.

Results of Operations

Six Months Ended June 30, 2012 and 2013 (unaudited)

The following table summarizes the results of our operations for the six months ended June 30, 2012 and 2013:

	Six Months Ended June 30,	
	2012	2013
	(unaudited)	
	(in thousands)	
Collaboration revenue	$ 867	$ 417
Grant revenue	632	345
Research and development expenses	5,281	5,598
General and administrative expenses	2,081	2,789
Total other income (expense), net	(108)	(1,457)

Revenue. Revenue was $0.8 million for the six months ended June 30, 2013, compared to $1.5 million for the six months ended June 30, 2012. The decrease of $0.7 million was due to the completion of our TATRC grant in May 2013 and the receipt of a $0.5 million commercialization milestone payment in 2012 that did not recur in 2013.

Research and development expenses. Research and development expenses were $5.6 million for the six months ended June 30, 2013, compared to $5.3 million for the six months ended June 30, 2012. The increase in spending was primarily due to a $0.8 million increase in employee compensation-related expenses associated with additional headcount to support the Phase 2 clinical development of ProHema and the preclinical

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development of our other product candidates, which was partially offset by a $0.3 million reduction in expenditures for laboratory supplies and fees paid to third-party professional consultants and service providers relating to our TATRC grant and a $0.2 million reduction in expenditures for laboratory supplies relating to other preclinical programs and technologies.

General and administrative expenses. General and administrative expenses were $2.8 million for the six months ended June 30, 2013, compared to $2.1 million for the six months ended June 30, 2012. The increase in spending was primarily due to a $0.4 million increase in employee compensation-related expenses associated with the expansion of our executive management team and a $0.3 million increase in professional service provider fees.

Other income (expense), net. Other income (expense), net was $(1.5) million for the six months ended June 30, 2013, compared to $(0.1) million for the six months ended June 30, 2012. The increase in other expense was primarily due to an increase in the fair value of the exchangeable share liability.

Years Ended December 31, 2011 and 2012

The following table summarizes the results of our operations for the years ended December 31, 2011 and 2012:

	Years Ended December 31,	
	2011	2012
	(in thousands)	
Collaboration revenue	$ 833	$ 1,268
Grant revenue	337	1,402
Research and development expenses	9,858	11,999
General and administrative expenses	4,605	4,228
Total other income (expense), net	(134)	(682)

Revenue. Revenue was $2.7 million for the year ended December 31, 2012, compared to $1.2 million for the year ended December 31, 2011. The increase of $1.5 million is due to an increase in reimbursable expenses related to our TATRC grant and the achievement of a commercial milestone under our iPSC technology collaboration with BD.

Research and development expenses. Research and development expenses were $12.0 million for the year ended December 31, 2012, compared to $9.9 million for the year ended December 31, 2011. The increase of $2.1 million was primarily due to: an increase in headcount resulting in an increase of $0.4 million in employee compensation-related expense, a $2.0 million increase in external costs for professional consultants, clinical site start-up and clinical supply manufacture and a $0.4 million increase in equipment and supplies, and to support clinical development and regulatory activities for ProHema, and an increase in headcount resulting in an increase of $0.2 million in employee compensation-related expense to support the development of our other programs and technologies, which was partially offset by a $0.9 million decrease in unallocated research and development costs.

General and administrative expenses. General and administrative expenses were $4.2 million for the year ended December 31, 2012, compared to $4.6 million for the year ended December 31, 2011. The decrease of $0.4 million was due primarily to a $0.4 million decrease in employee-related costs and a $0.2 million decrease in intellectual property maintenance and prosecution costs, partially offset by a $0.2 million increase in market research related expenses.

Other income (expense), net. Other income (expense), net, was $(0.7) million for the year ended December 31, 2012, compared to $(0.1) million for the year ended December 31, 2011, an increase in other expense of approximately $0.6 million. The increase was primarily due to a $0.3 million loss on extinguishment of debt recognized in 2012 relating to a transaction with a strategic investor pursuant to our Series C preferred stock financing, whereby we issued shares of Series B-1 convertible preferred stock in exchange for shares of our common stock owned by the strategic investor and for the forgiveness of a note payable by us to the strategic investor, and a $0.4 million increase in interest expense as a result of higher average debt balances outstanding in 2012.

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Liquidity and Capital Resources

We have incurred losses and negative cash flows from operations since inception. As of June 30, 2013, we had an accumulated deficit of $74.7 million and anticipate that we will continue to incur net losses for the foreseeable future.

From our inception through June 30, 2013, we have funded our consolidated operations primarily through the private placement of preferred stock and convertible notes, commercial bank debt and revenues from collaboration activities and grants. As of June 30, 2013, we had cash and cash equivalents of approximately $3.4 million.

In 2009, we entered into a $3.0 million loan and security agreement collateralized by substantially all of our assets, excluding certain intellectual property. We drew the full $3.0 million available under the loan and security agreement in 2009. In August 2011, the loan and security agreement was amended to: (i) increase the available credit under the loan and security agreement to $4.0 million, (ii) add an additional payment upon maturity equal to 5% of the maximum loan amount and (iii) repay the remaining $0.6 million of outstanding principal related to the original $3.0 million loan. We accessed the full $4.0 million of available credit under the amended loan and security agreement by taking a term advance of $2.0 million in August 2011 and a term advance of $2.0 million in December 2011, each of which are scheduled to be fully paid by August 2014 and December 2014, respectively. The term advances require interest-only payments during the first 12 months from access and equal monthly principal and interest payments during the final 24 months from access. The interest rate on the term advances is fixed at 7.0% per annum for their entire 36-month term of the debt. As of June 30, 2013, the aggregate outstanding principal was $2.8 million.

The following table sets forth a summary of the net cash flow activity for each of the periods indicated (in thousands):

	Years Ended December 31,		Six Months Ended June 30,	
	2011	2012	2012	2013
			(unaudited)	
Net cash used in operating activities	$ (12,145)	$ (13,274)	$ (7,112)	$ (7,323)
Net cash provided by (used in) investing activities	200	(709)	(59)	(71)
Net cash provided by financing activities	7,107	16,683	6,246	1,706
Net (decrease) increase in cash and cash equivalents	$ (4,838)	$ 2,700	$ (925)	$ (5,688)

Operating Activities

Cash used in operating activities increased $0.2 million from $7.1 million for the six months ended June 30, 2012 to $7.3 million for the six months ended June 30, 2013. The primary driver of operating cash requirements was our net loss in each period. During the six months ended June 30, 2012, we used cash from operating activities of $7.1 million while our net loss was $6.0 million. The difference was a result of $1.4 million net change in our operating assets and liabilities, offset by $0.3 million of non-cash charges and deferrals, including depreciation expense and stock-based compensation. During the six months ended June 30, 2013, we used cash from operating activities of $7.3 million while our net loss was $9.1 million. The difference was a result of $0.1 million net change in our operating assets and liabilities and $1.7 million of non-cash charges and deferrals, including depreciation expense, stock-based compensation and the change in fair value of exchangeable shares.

Cash used in operating activities increased $1.2 million from $12.1 million for the year ended December 31, 2011 to $13.3 million for the year ended December 31, 2012. The primary driver of operating cash requirements was our net loss in each period. During the year ended December 31, 2011, we used cash from operating activities of $12.1 million while our net loss was $13.4 million. The difference was a result of $0.3 million net change in our operating assets and liabilities and $0.9 million of non-cash charges and deferrals, including depreciation expense, stock-based compensation deferred revenue and other. During the year ended December 31, 2012, we used cash from operating activities of $13.3 million while our net loss was $14.2 million.

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The difference was a result of $1.0 million of non-cash charges and deferrals, including depreciation expense, stock-based compensation, deferred rent, loss on extinguishment of debt and other.

Investing Activities

During both the six months ended June 30, 2012 and 2013, investing activities used cash of $0.1 million for the purchase of property and equipment. During the year ended December 31, 2011, investing activities provided cash of $0.2 million, primarily due to our sale of property and equipment. During the year ended December 31, 2012, investing activities used cash of $0.7 million for the purchase of property and equipment.

Financing Activities

Financing activities provided cash of $6.2 million for the six months ended June 30, 2012 and provided cash of $1.7 million for the six months ended June 30, 2013. During the six months ended June 30, 2012, we received $0.2 million of proceeds from the issuance of restricted stock awards and the exercise of common stock options and received $6.1 million in net proceeds from the sale of Series C preferred stock. During the six months ended June 30, 2013, we paid down principal of $1.0 million on our outstanding long-term debt. No equivalent principal amounts were paid down in 2012 as the debt was still in its interest-only period. In addition, we received $3.0 million of proceeds from the sale of convertible notes and paid $0.3 million for initial public offering costs during the six months ended June 30, 2013.

Financing activities provided cash of $7.1 million for the year ended December 31, 2011 and $16.7 million during the year ended December 31, 2012. During the year ended December 31, 2011, we sold $1.0 million of convertible debt and $3.5 million of Series B convertible preferred stock. In addition, we paid down $0.8 million of principal under our loan and security agreement prior to its amendment, under which we received net cash of an additional $3.4 million. During the year ended December 31, 2012, we issued $16.7 million of Series C convertible preferred stock.

2013 Convertible Note Financings

In June and July 2013, we issued convertible promissory notes in an aggregate principal amount of $3.7 million to certain existing stockholders. The notes accrue interest at 2% per year and are due on June 24, 2014. The principal balance and all accrued and unpaid interest due on the notes will become due in cash or will be converted into shares of our capital stock as follows:

- upon the closing of an initial public offering which either (i) results in gross proceeds to us of at least $40.0 million (including amounts from conversion of the June and July 2013 notes) or (ii) is approved by the holders of a majority of the aggregate principal amount of all June and July 2013 notes then outstanding, the notes will automatically convert into shares of our common stock at a per share price equal to the initial public offering price;

- upon the completion of an equity financing other than an initial public offering as described above with gross proceeds to us of at least $5.0 million (excluding amounts from conversion of the June and July 2013 notes), at the election of the holders of a majority of the aggregate principal amount of the notes, the notes will automatically convert into either (i) shares of the securities issued in the equity financing at the per share price of the securities issued in such equity financing or (ii) shares of our Series C convertible preferred stock at $1.00 per share;

- on or after June 24, 2014, at the election of the holders of a majority of the aggregate principal amount of the notes, the notes will convert into shares of our Series C convertible preferred stock at $1.00 per share; or

- upon a sale of our company, transfer of all or substantially all of our assets, or the voluntary or involuntary liquidation, dissolution or winding up of our company, at the election of the holders of the notes, the notes will either (i) become due and payable in cash or (ii) convert into shares of our Series C convertible preferred stock at $1.00 per share.

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In August 2013, we issued convertible promissory notes in an aggregate principal amount of $20.0 million to certain new investors. The notes accrue interest at 2% per year and are due on August 8, 2016. The principal balance and all accrued and unpaid interest due on the notes will become due in cash or will be converted into shares of our capital stock as follows:

- upon the closing of an initial public offering pursuant to which all of the preferred stock and all of the convertible promissory notes issued in June and July 2013 are converted into shares of our common stock, the notes will automatically convert into shares of our common stock at a per share price equal to the initial public offering price, subject to the limitations described below;

- upon the completion of an equity financing (other than an initial public offering as described above) with gross proceeds to us of at least $10.0 million (excluding amounts from conversion of the convertible promissory notes issued in June and July 2013), at the election of the holder of each note, such holder s note will automatically convert into either (i) shares of the securities issued in the equity financing at the per share price of the securities issued in such equity financing or (ii) shares of our Series C-1 convertible preferred stock at $1.90 per share;

- on or after August 8, 2016, at the election of the holder of each note, such holder s note will convert into shares of our Series C-1 convertible preferred stock at $1.90 per share; or

- upon a sale of our company, transfer of all or substantially all of our assets, or the voluntary or involuntary liquidation, dissolution or winding up of our company, at the election of the holder of each note, such holder s note will either (i) become due and payable in cash or (ii) convert into shares of our Series C-1 convertible preferred stock at $1.90 per share.

In the event the full conversion upon the completion of this offering of all outstanding principal and accrued interest under our convertible promissory notes issued in August 2013 would cause the holders of the August 2013 notes, together with their affiliates and related parties, to beneficially own 15% or more of our outstanding common stock immediately following the completion of this offering, or the percentage cap, only a portion of the outstanding balance up to the percentage cap will be converted into shares of common stock upon the completion of this offering. We will be required to repay any remaining unconverted balance under the August 2013 notes (i) upon the request of the holders of the August 2013 notes made on or prior to the date 30 days after the completion of this offering or (ii) on the date 30 days after the completion of this offering, in the event any balance remains outstanding under the August 2013 notes following their conversion in an amount up to the percentage cap on such date. Unless earlier repaid by us, on the date 30 days after the completion of this offering, the remaining unconverted balance under the August 2013 notes will be converted into shares of common stock up to the percentage cap, as measured on the date 30 days after the completion of this offering.

We refer to the convertible promissory notes issued in June, July and August 2013 as our 2013 Notes.

Operating Capital Requirements

To date, we have not generated any revenues from therapeutic product sales. We do not know when, or if, we will generate any revenue from therapeutic product sales. We do not expect to generate significant revenue from therapeutic product sales unless and until we obtain regulatory approval of and commercialize one of our current or future product candidates. We anticipate that we will continue to incur losses for the foreseeable future, and we expect the losses to increase as we continue the development of, and seek regulatory approvals for, our product candidates, and begin to commercialize any approved products. We are subject to all of the risks incident in the development of new therapeutic products, and we may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. Upon the completion of this offering, we expect to incur additional costs associated with operating as a public company. We anticipate that we will need substantial additional funding in connection with our continuing operations.

We believe that the net proceeds from this offering and our existing cash and cash equivalents, including the proceeds from the July and August 2013 notes, will be sufficient to fund our projected operating requirements

through at least the end of 2014. However, we may require additional capital for the further development of our existing product candidates and may also need to raise additional funds sooner to pursue other development activities related to additional product candidates.

Until we can generate a sufficient amount of revenue from our therapeutic products, if ever, we expect to finance future cash needs through public or private equity or debt offerings. In any event, we do not expect to achieve significant revenue from therapeutic product sales prior to the use of the net proceeds from this offering. Additional capital may not be available on reasonable terms, if at all. 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 one or more of our product candidates. If we raise additional funds through the issuance of additional debt or equity securities, it could result in dilution to our existing stockholders, increased fixed payment obligations and the existence of securities with rights that may be senior to those of our common stock. If we incur indebtedness, we could become subject to covenants that would restrict our operations and potentially impair our competitiveness, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. Any of these events could significantly harm our business, financial condition and prospects.

Our forecast of the period of time through which our financial resources will be adequate to support our operations is a forward-looking statement and involves risks and uncertainties, and actual results could vary as a result of a number of factors. We have based this estimate on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we currently expect. The amount and timing of future funding requirements, both near- and long-term, will depend on many factors, including, but not limited to:

- the design, initiation, progress, size, timing, costs and results of preclinical studies and clinical trials for our product candidates;
- the outcome, timing and cost of regulatory approvals by the FDA and comparable foreign regulatory authorities, including the potential for the FDA or comparable foreign regulatory authorities to require that we perform more studies than, or evaluate clinical endpoints other than those that we currently expect;
- the timing and costs associated with manufacturing our product candidates for clinical trials, preclinical studies and, if approved, for commercial sale;
- the number and characteristics of product candidates that we pursue;
- the extent to which we are required to pay milestone or other payments under our in-license agreements and the timing of such payments;
- the cost of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights;
- our need to expand our research and development activities, including our need and ability to hire additional employees;
- our need to implement additional infrastructure and internal systems and hire additional employees to operate as a public company;
- the effect of competing technological and market developments; and
- the cost of establishing sales, marketing and distribution capabilities for any products for which we may receive regulatory approval.

If we cannot expand our operations or otherwise capitalize on our business opportunities because we lack sufficient capital, our business, financial condition and results of operations could be materially adversely affected.

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Contractual Obligations and Commitments

The following table summarizes our contractual obligations and commitments as of December 31, 2012 that will affect our future liquidity (in thousands):

	Total	Less than 1 Year	1-3 Years	3-5 Years	More than 5 Years
Long-term debt (including interest)	$ 4,212	$ 2,201	$ 2,011	$	$
Operating lease obligation [(1)]	1,331	883	448		
Total	$ 5,543	$ 3,084	$ 2,459	$	$

(1) On December 3, 2009, we entered into a multi-year building lease for our facility in San Diego, California. The operating lease is noncancelable and expires on June 30, 2014. In September 2013, we exercised an option to extend this lease by an additional two years.

The table above excludes convertible promissory notes we issued to certain existing stockholders in June and July 2013 for an aggregate principal amount of $3.7 million. These notes accrue interest at 2% per year and are due on June 24, 2014. The table above also excludes convertible promissory notes we issued to certain new investors in August 2013 for an aggregate principal amount of $20.0 million. These notes accrue interest at 2% per year and are due on August 8, 2016. Upon the completion of this offering, all outstanding principal and accrued interest under the convertible promissory notes issued in June, July and August 2013 will automatically convert into shares of our common stock, except that under certain circumstances, a portion of our 2013 Notes issued in August 2013 may remain outstanding following the completion of this offering, and we may be required to repay such portion in cash. See Liquidity and Capital Resources 2013 Convertible Note Financings for a description of the terms of the convertible promissory notes issued in June, July and August 2013.

We also have obligations under various license agreements to make future payments to third parties that become due and payable on the achievement of certain development, regulatory and commercial milestones (such as the start of a clinical trial, filing for product approval with the FDA or other regulatory agencies, product approval by the FDA or other regulatory agencies, product launch or product sales) or on the sublicense of our rights to another party. We have not included these commitments on our balance sheet or in the table above because the achievement and timing of these events is not fixed and determinable. Certain milestones are in advance of receipt of revenue from the sale of products and, therefore, we may require additional debt or equity capital to make such payments. These commitments include:

Under an exclusive license agreement with Children s Medical Center Corporation pursuant to which we license certain patents for use in our HSC modulation platform and our pharmacologically-modulated HSC product candidates, including ProHema, we are required to make annual maintenance payments and payments based upon development, regulatory and commercial milestones for any products covered by the in-licensed intellectual property. The maximum aggregate milestone payments we may be obligated to make per product are $5.0 million. We will also be required to pay a royalty on net sales of products covered by the in-licensed intellectual property in the low to mid single digits. The royalty is subject to reduction for any third-party payments required to be made, with a minimum floor in the low single digits. We have the right to sublicense our rights under this agreement, and we will be required to pay a percentage of any sublicense income.

Under an exclusive license agreement with the Board of Trustees of the Leland Stanford Junior University pursuant to which we license certain patents relating to the use of Wnt proteins, we are required to make annual maintenance payments and payments based upon development, regulatory and commercial milestones for any products covered by the in-licensed intellectual property. The maximum aggregate milestone payments we may be obligated to make are $0.9 million. We will also be required to pay a royalty on net sales of products covered by the in-licensed intellectual property in the low to mid single digits. The royalty is subject to reduction for any third-party payments required to be made, with a minimum floor in the low single digits. We have the right to sublicense our rights under this agreement so

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long as we are actively pursuing the development or commercialization of products covered by the patent rights, and we will be required to pay a percentage of any sublicense income.

Under an exclusive license agreement with the Ottawa Hospital Research Institute pursuant to which we license certain patents relating to the use of Wnt7a proteins, we are required to make annual maintenance payments and payments based upon development, regulatory and commercial milestones for any products covered by the in-licensed intellectual property. The maximum aggregate milestone payments we may be obligated to make per product are $1.4 million. We will also be required to pay a royalty on net sales of products covered by the in-licensed intellectual property in the low single digits. The royalty is subject to reduction for any third-party payments required to be made, with a minimum floor in the low single digits. We have the right to sublicense our rights under this agreement, and we will be required to pay a percentage of any sublicense income.

We enter into contracts in the normal course of business with clinical sites for the conduct of clinical trials, contract research service providers for preclinical research studies, professional consultants for expert advice and other vendors for laboratory and research supplies and services. These contracts generally provide for termination on notice, and therefore are cancelable contracts and not included in the table of contractual obligations and commitments.

Off-Balance Sheet Arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined in the rules and regulations of the SEC.

Quantitative and Qualitative Disclosures about Market Risks

We are exposed to market risk related to changes in interest rates. As of June 30, 2013, we had cash and cash equivalents of $3.4 million, including $0.3 million of money market mutual funds. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because our investments are in short-term securities. Our available for sale securities are subject to interest rate risk and will fall in value if market interest rates increase. Due to the short-term duration of our investment portfolio and the low risk profile of our investments, an immediate 100 basis point change in interest rates would not have a material effect on the fair market value of our portfolio.

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BUSINESS

Overview

We are a clinical-stage biopharmaceutical company engaged in the discovery and development of pharmacologic modulators of adult stem cells to treat orphan diseases, including certain hematologic malignancies, lysosomal storage disorders and muscular dystrophies. Our novel approaches utilize established pharmacologic modalities, including small molecules and therapeutic proteins, and target well-characterized biological mechanisms to enhance the therapeutic potential of adult stem cells. Adult stem cells play a key role in the growth, maintenance and repair of many tissues and organ systems in the body. Due to their natural ability to self-renew, and to regenerate and repair diseased or damaged tissue, adult stem cells also hold considerable therapeutic promise.

Based on our deep understanding of key biological mechanisms that guide the fate of adult stem cells, we have built two modulation platforms that optimize the activity of adult stem cells using techniques that operate both outside of the body, or *ex vivo*, and within the body, or *in vivo*. We believe that the product candidates generated by our stem cell modulation platforms have significant potential as life-changing or curative therapeutics across a broad range of orphan indications.

Our HSC modulation platform focuses on the *ex vivo* pharmacologic optimization of hematopoietic stem cells, or HSCs, which are adult stem cells that regenerate all types of blood cells throughout a person s lifespan. HSCs have been used for decades in a potentially curative procedure called hematopoietic stem cell transplant, or HSCT. This procedure is most commonly used in patients with hematologic malignancies to replace a diseased hematopoietic system with a healthy one. While over one million HSCT procedures have been performed to date, we believe HSCs have not been pharmacologically optimized to improve patient outcomes. Our HSC modulation platform has the potential to generate products that will improve patient outcomes in orphan indications by enhancing hematopoietic reconstitution through accelerated, durable engraftment, permitting greater donor matching flexibility, reducing the risk of major side effects and enabling the use of less toxic conditioning regimens.

Our lead product candidate, ProHema, is a pharmacologically modulated HSC therapeutic derived from umbilical cord blood. We have established human proof-of-concept for ProHema in the clinical setting by demonstrating enhanced and durable engraftment of HSCs within the bone marrow. Engraftment, which is the localization and integration of HSCs within a targeted tissue where they can produce new cells, is an important determinant of patient outcomes in HSCT. We are presently advancing ProHema in Phase 2 clinical development for hematologic malignancies. We are also pursuing the development of pharmacologically optimized HSC therapeutics for the treatment of certain lysosomal storage disorders, or LSDs, where HSCs have demonstrated the ability to home, or migrate, to and engraft within the central nervous system, or CNS.

Our SSC modulation platform focuses on the *in vivo* pharmacologic activation of satellite stem cells, or SSCs, which are adult stem cells that regenerate muscle throughout a person s lifespan. The regenerative capacity of SSCs in skeletal muscle is exhausted both in the natural aging process and in degenerative conditions, such as muscular dystrophies. We have identified Wnt7a as a natural promoter of SSCs to drive muscle regeneration, and we are initially focused on developing Wnt7a analogs for the treatment of muscular dystrophies.

Using our expertise in Wnt protein chemistry, we have engineered pharmacologically optimized analogs of the Wnt protein class. Wnts comprise a family of 19 secreted proteins known to play a key physiological role in developmental and regenerative processes. We have developed injectable analogs of Wnt7a as recombinant human protein therapeutics with muscle regenerative activity. In preclinical models of muscular dystrophies, our Wnt7a protein analogs demonstrated an expansion of the SSC population, an increase in muscle hypertrophy, a reduction in disease-specific muscle fiber necrosis and inflammation, and an increase in muscle strength, all of which were statistically significant. We are presently advancing our Wnt7a analogs in preclinical development with the goal of filing an IND in 2014.

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The following table summarizes key information about our platforms and our product candidates:

Product Candidate	Targeted Orphan Disorders[(1)]	Status
HSC Modulation Platform		
ProHema	Adult hematologic malignancies	Phase 2
ProHema	Pediatric hematologic malignancies	Preclinical
ProHema	LSDs	Preclinical
Second Generation HSC Therapeutic	LSDs	Preclinical
SSC Modulation Platform		
Wnt7a Protein Analogs	Muscular dystrophies	Preclinical
Wnt7a Protein Analogs	Neuromuscular disorders	Preclinical

(1) We have been granted orphan designation in the United States for human allogeneic HSCs *ex vivo* modulated with 16, 16-dimethyl prostaglandin E2, which we refer to as FT1050, for the enhancement of stem cell engraftment and in the European Union for ProHema for the treatment of acute myelogenous leukemia through the *ex vivo* modulation of allogeneic umbilical cord blood cells.

We plan to continue the validation of our two platforms by demonstrating the clinical benefit of our initial product candidates over the next three years in three orphan disease settings: hematologic malignancies, LSDs and muscular dystrophy. We believe both of our platforms have the ability to generate additional products with therapeutic utility beyond their initial target indications. We also intend to expand our initial indications across a broader spectrum of orphan diseases, including those where allogeneic HSCT holds curative potential and those where muscle regeneration holds disease-modifying potential.

Our platforms and product candidates are based on the research of our scientific founders, all of whom are internationally recognized experts in the field of adult stem cell biology and have contributed significant intellectual capital to our efforts. Our stem cell modulation platforms and our proprietary product candidates are protected by a strong intellectual property position. We have retained worldwide therapeutic rights to product candidates generated by each of our platforms.

Our Novel Approach to *Ex Vivo* HSC Modulation

While over one million HSCT procedures have been performed to date with curative intent, we believe HSCs administered to patients undergoing HSCT have not been pharmacologically optimized to improve patient outcomes. Our HSC modulation platform pioneers a novel approach to improving patient outcomes in HSCT: we enhance the biological properties of HSCs *ex vivo* to drive well-understood biological mechanisms *in vivo* that are critical to the success of the procedure.

We believe our product candidates can significantly improve and enable the curative potential of HSCT in patients with orphan hematologic malignancies and rare genetic disorders. Our HSC modulation platform encompasses the following advantages:

We optimize HSCs *ex vivo* to enhance their biological properties. Our strategies and methods of optimizing HSCs *ex vivo* are designed to specifically enhance the ability of HSCs to achieve desired therapeutic effects *in vivo*. Our proprietary processes induce profound changes in gene expression that are critical to HSC homing and engraftment, which are required for successful patient outcomes.

Our platform is applicable across different stem cell sources and a broad range of diseases. We believe that our approach to the pharmacological enhancement of certain biological properties of HSCs can be applied across various sources of HSCs, such as mobilized peripheral blood, bone marrow and umbilical cord blood. Furthermore, we believe our technology can be employed in both the allogeneic and autologous HSCT settings, independent of the underlying cause of disease. Accordingly, we believe our

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HSC modulation platform will enable us to develop additional HSC therapeutics for the treatment of a broad spectrum of hematologic malignancies and rare genetic diseases.

Our proprietary HSC optimization process can be readily adopted into the HSCT standard of care. We believe we can efficiently optimize HSCs in a rapid *ex vivo* treatment process conducted on site at the clinical center. Following this process, the enhanced cells are washed to remove the modulators and can be immediately infused into the patient within the established framework of HSCT.

Our Novel Approach to *In Vivo* SSC Modulation

We are applying our knowledge of stem cell modulation to develop novel biologic therapeutics based on the natural signals that stimulate SSCs *in vivo*. Our SSC modulation platform enables us to evaluate multiple opportunities in skeletal muscle biology and neuromuscular disease. Our initial focus is on the treatment of muscular dystrophies. We believe we are the first company to demonstrate in preclinical studies that SSCs can be pharmacologically modulated *in vivo* to improve muscle regeneration.

Our SSC modulation platform seeks to stimulate the intrinsic regenerative capacity of skeletal muscle. While several promising product candidates have emerged for the treatment of genetically distinct subtypes of muscular dystrophies, such as Duchenne muscular dystrophy, these therapeutics are generally focused on preventing further muscle degeneration. We are not aware of any clinical-stage programs focused on driving the natural regenerative process to increase muscle strength. We believe that our approach is novel and applicable across multiple forms of muscular dystrophies.

We believe that our proprietary Wnt7a analogs validate our therapeutic strategy for the pharmacologic modulation of SSCs and represent a novel and promising approach for the treatment of muscular dystrophies. The advantages of our approach include:

Our means of SSC intervention are receptor-mediated and highly-specific. We leverage the inherent specificity conferred by the endogenous protein Wnt7a and its receptor, Fzd7, which is selectively expressed in muscle tissue. We believe this inherent specificity will enable us to develop therapeutics with a low risk of off-target effects.

Our SSC modulation platform is enabled by our expertise in the development of Wnt-based therapeutics. The therapeutic and regenerative potential of the Wnt protein family is well known. However, Wnt proteins have not been developed as therapeutics because their molecular characteristics prevent their scaled production, formulation, functional specificity and administration for human use. We have systematically applied structural prediction, rational design and protein engineering techniques to overcome these challenges. We believe we are the first company to produce Wnt protein analogs that are amenable to therapeutic development and *in vivo* administration.

We drive muscle regeneration through a unique dual mechanism of action. We have established preclinical proof-of-concept for our Wnt7a protein analogs in models of muscular dystrophy. These studies demonstrate that a single injection of our Wnt7a analogs induced an expansion of the SSC population, an increase in muscle hypertrophy and a decrease in muscle inflammation and damage, all of which were statistically significant. We have demonstrated in preclinical studies that these profound effects result in a significant increase in muscle strength. We believe the ability of our Wnt7a protein analogs to both activate SSC population expansion and increase muscle hypertrophy is a unique dual mechanism of action for the treatment of muscular dystrophies.

Our Wnt7a analogs have therapeutic potential as stand-alone or complementary treatments across a broad spectrum of muscular dystrophies. Most approaches to treat muscular dystrophies seek to slow the degeneration of muscle in genetically distinct subtypes of the disease. In contrast, because our Wnt7a protein analogs enable muscular regeneration, they have the potential to treat a broader spectrum of muscular dystrophies either as stand-alone or complementary therapeutics. We believe that our Wnt-based protein analogs are the only therapeutics in development that actively promote the regeneration of muscle for the treatment of muscular dystrophies.

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Our SSC modulation platform has potential beyond muscular dystrophies. Our Wnt7a analogs target the biological mechanisms underlying the body s intrinsic muscle regenerative process. We believe that enhancing these mechanisms can restore the balance between muscle degeneration and regeneration for other neuromuscular disorders. Accordingly, our Wnt protein analogs have the potential to treat a wide range of conditions, such as cachexia, atrophy, trauma and sarcopenia.

Our Strategy

Our goal is to realize the therapeutic potential of our two stem cell modulation platforms across a broad range of orphan diseases through the discovery, development and commercialization of first-in-class products. The key elements of our strategy are to:

Validate the transformative therapeutic potential of our platforms. We plan to validate our two stem cell modulation platforms over the next three years by demonstrating the clinical benefit of our initial product candidates in three orphan disease settings: hematologic malignancies, LSDs and muscular dystrophy. We believe the data generated from our planned clinical trials will enable us to establish stem cell modulation as a new treatment modality with application across a broad range of orphan diseases.

Efficiently develop and commercialize our orphan therapeutic candidates. We plan to pursue a fast-to-market strategy through efficient clinical development and expedited regulatory pathways. Due to the nature of our target indications, we believe our pivotal clinical trials will generally require relatively small numbers of patients and measure relatively short-term efficacy endpoints. We also intend to pursue, where possible, expedited regulatory pathways such as fast track or breakthrough therapy designations, which are available for therapies that address serious conditions and provide a major advance in treatment. In addition, because our target markets are highly specialized and concentrated within a limited number of centers of excellence, we intend to build our own focused sales and marketing capabilities to commercialize any products that we may successfully develop in a cost-efficient manner.

Leverage lifecycle opportunities. We believe that our therapeutic approach provides a unique opportunity for strategic lifecycle management and indication expansion. First, because our product candidates have broad therapeutic utility, clinical validation in their initial target indications may allow for the development of these product candidates for the treatment of additional diseases. Second, we intend to leverage both of our platforms to generate additional product candidates to further exploit the therapeutic potential of HSCs and SSCs.

We may also seek partners who can bring therapeutic, development and commercialization capabilities, geographical expertise and financial resources that allow us to leverage the potential of our product platforms within or beyond our initial clinical and commercial focus.

Our HSC Modulation Platform and Product Candidates

Background on Hematopoietic Stem Cells

HSCs are adult stem cells that have the ability to regenerate all types of blood cells throughout a person s lifespan. HSCs have been used for decades in HSCT, a potentially curative or life-saving procedure that is most commonly performed in patients with hematologic malignancies to replace a diseased hematopoietic system with a healthy one. There are two types of HSCT procedures, autologous and allogeneic transplant. In the autologous setting, a patient s own HSCs are recovered from bone marrow aspirates or are mobilized and recovered from peripheral blood for transplant. In the allogeneic setting, matched HSCs are recovered from a related or unrelated donor, or from umbilical cord blood. The standard of care for HSCT in both of these settings uses HSCs that have not been pharmacologically optimized.

The number of HSCT procedures performed has increased steadily over the past two decades and continues to grow. According to a global survey conducted by the Worldwide Network for Blood and Marrow Transplantation, a total of 56,739 HSCT procedures were performed worldwide in 2010, including 26,241 such procedures in the allogeneic setting. It is estimated that approximately 95% of HSCT procedures are performed

for the treatment of hematologic malignancies. Additionally, it is estimated that allogeneic HSCT procedures have been used in the treatment of over 50 rare genetic disorders, many of which are life-threatening and lack alternative therapeutic options.

Limitations of Allogeneic HSCT

While allogeneic HSCT is a proven therapeutic intervention strategy with curative potential, it is associated with significant treatment-related limitations and 100-day mortality rates between 20% to 30%. Treatment-related morbidity and mortality for patients undergoing allogeneic HSCT are significantly influenced by several key factors, including:

HLA matching. The ability to achieve human leukocyte antigen, or HLA, matching, or the degree to which a donor s and recipient s tissues are immunologically compatible, is a critical determinant of clinical outcomes. If the donor-derived immune system is not sufficiently compatible with the recipient s tissue, a serious complication known as graft-versus-host disease, or GvHD, may occur. Chronic GvHD occurs in 25-50% of patients who undergo HSCT procedures. Greater HLA mismatch also increases the risk of failure to engraft.

Cell dose. Successful transplants require an adequate dose of HSCs in order to ensure timely reconstitution. While a sufficient number of HSCs can usually be collected from healthy adults donating bone marrow or mobilized peripheral blood, some HSC collections may be suboptimal, which increases the risk of delayed or failed engraftment. Despite many advantages, cord blood units generally contain fewer HSCs than traditional HSC sources, which translates into delayed engraftment and a higher risk of failed engraftment. Graft failure rates can be as high as 23% after double umbilical cord blood unit transplant and 27% after single umbilical cord blood unit transplant in adults. As a result, many of the banked cord blood units are deemed to contain an insufficient number of HSCs for adult transplant.

Patient conditioning. Prior to allogeneic HSCT, chemotherapy or radiation therapy and immunotherapy are administered to eradicate a patient s diseased hematopoietic system and enable donor-derived HSCs to reconstitute a healthy hematopoietic system. HSCT has traditionally required intense myeloablative conditioning, or MAC, which is highly toxic and associated with high rates of transplant-related morbidity. As a result, only younger and healthier patients are typically considered eligible for MAC. More recently, investigators have developed reduced-intensity conditioning, or RIC, regimens that employ significantly lower doses of chemotherapy or radiation and are less toxic. Despite their safety advantages, RIC regimens are associated with lower rates of engraftment and higher rates of relapse.

Reconstitution. The process by which a patient s hematopoietic system reconstitutes, which occurs over the course of several weeks and months after HSCT, is also critical to patient outcomes. Importantly, the components of the hematopoietic system do not return to normal levels at the same rate. Time to engraftment, particularly as measured by time to the engraftment of neutrophils, a type of white blood cell involved in fighting bacterial infections, correlates with key clinical outcomes including the length of hospital stays, rates of serious infections, and overall transplant-related morbidity and mortality.

Advantages of Our HSC Modulation Platform

Our HSC modulation platform is designed to address the current limitations of allogeneic HSCT and enhance its curative potential across a broad range of orphan hematologic malignancies and rare genetic disorders. Since our inception, we have worked closely with our scientific founders, who are internationally-recognized leaders in HSC biology, to gain a deep understanding of the molecular pathways involved in homing and engraftment. Extensive genome-wide expression studies have provided key insights that allow us to modulate these signaling networks using a proprietary pathway screening approach. We have also developed sophisticated assays to characterize the molecular and functional properties of HSCs following the *ex vivo* modulation process. These tools have enabled us to optimize the *ex vivo* modulation process by systematically and precisely evaluating key parameters of the incubation conditions, including time, dose, temperature and media. Our HSC modulation platform also utilizes established *in vivo* models of hematopoiesis to rapidly assess and quantify the enhanced properties of our product candidates.

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Our scientific founders were the first to demonstrate preclinical proof-of-concept for the *ex vivo* pharmacologic modulation of HSCs using prostaglandin E2 receptor agonists in 2007. Dr. Leonard Zon identified FT1050 to be a potent regulator of hematopoiesis. Since then, we have systematically applied our HSC modulation platform to translate this initial academic discovery into the clinical setting. This involved optimizing the incubation conditions and performing extensive preclinical characterization studies. By modulating HSCs derived from umbilical cord blood with FT1050, we generated our initial product candidate, which we refer to as ProHema. The figure below shows the enhanced homing and engraftment properties of the *ex vivo* modulated human HSCs in a mouse model of HSCT:

We also performed a series of mouse transplantation experiments to determine whether the improved homing and engraftment properties of ProHema translated into improved survival outcomes following transplants with suboptimal HSC numbers. The figure below shows that the majority of lethally irradiated mice in the control group (seven out of ten) died during the 30-day observation period due to insufficient HSC dose, while all of the mice in the ProHema group survived.

Our HSC modulation platform has the potential to enhance the biological properties of HSCs from any source, including umbilical cord blood, peripheral blood and bone marrow, and addresses many of the limitations of the current standard of care for HSCT as follows:

Expand the pool of HSC sources. We believe that the use of HSC sources that are immunologically naïve, such as umbilical cord blood, can increase the likelihood of identifying an HLA-compatible HSC source for allogeneic HSCT and reduce the incidence and severity of GvHD. It is believed that most patients have the chance to rapidly find a well HLA-matched umbilical cord blood unit for use in allogeneic HSCT, given that

there are currently over 600,000 publicly-banked cord blood units available worldwide. Enhancing the biological properties of cord blood derived HSCs has the potential to significantly broaden the pool of viable banked cord blood units, and thereby improve the odds of finding the best or a better HLA-matched unit.

Overcome cell dose limitations. We believe that the optimization of HSCs can improve the engraftment potential of allogeneic HSCT, particularly when performed with umbilical cord blood, in which the HSC dose is lower than with other allogeneic HSC sources. As a result, we believe this will enable patients who are potential candidates for HSCT to have greater access to HSC sources, such as umbilical cord blood units that previously would have been considered to contain HSC doses insufficient for HSCT.

Enable the use of less toxic conditioning regimens. By enhancing the biological properties of HSCs, we believe that we can improve the rate of engraftment in the safer RIC setting as compared to MAC. We believe that improving the viability of RIC regimens will widen the adoption of, and broaden the eligible patient populations for, allogeneic HSCT.

Enhance HSC engraftment and reconstitution. We believe that the pharmacologic modulation of HSCs can improve patient outcomes across HSCT by increasing engraftment success rates, accelerating the time to reconstitution and improving the durability of engraftment. In addition, we believe that improving engraftment success rates and accelerating the time to reconstitution will lead to improved patient outcomes and the broader adoption of allogeneic HSCT.

We believe ProHema is the first *ex vivo* modulated HSC product candidate to be evaluated in a clinical trial in patients undergoing HSCT. We have established human proof-of-concept for ProHema in the clinical setting by demonstrating enhanced and durable engraftment, which are important determinants of patient outcomes. The HSC modulation process used in the manufacture of ProHema takes only two hours, can be performed directly in the transplant center, does not require significant changes to existing infrastructure and is compatible with standard of care treatment modalities.

Phase 1b Clinical Proof-of-Concept for ProHema

In September 2011, we completed a Phase 1b clinical trial of ProHema in adult patients with hematologic malignancies undergoing double umbilical cord blood transplant, or UCBT, after a RIC regimen. The primary objective of our Phase 1b clinical trial, referred to as the ProHema-01 trial, was to evaluate the safety of allogeneic HSCT using ProHema plus an unmanipulated cord blood unit. Secondary objectives of the trial included the assessment of time to engraftment and 100-day survival.

The ProHema-01 trial consisted of two cohorts of patients with acute leukemia, non-Hodgkin s lymphoma and myelodysplastic syndrome:

an inactive cohort of nine patients who received an unmanipulated cord blood unit and a cord blood unit modulated with FT1050 under biologically inactive conditions; and

the ProHema cohort of 12 patients who received ProHema and an unmanipulated cord blood unit.

The trial was conducted at the Dana Farber Cancer Institute and the Massachusetts General Hospital, and the results were compared against recent historical results from a control group of 53 adult patients with hematologic malignancies undergoing double UCBT at these institutions.

Key Clinical Observations

We observed the following potential clinical benefits in our ProHema-01 trial:

Treatment with ProHema demonstrated a statistically significant improvement in time to neutrophil engraftment, as compared to the historical control (p=0.043). Neutrophil engraftment was defined as peripheral blood neutrophil count above 500 cells per microliter. A p-value is a probability with a value ranging from 0 to 1, which indicates the likelihood that the results of a study are different between

treatment and control groups. P-values below 0.05 are typically referred to as statistically significant;

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ProHema improved the cumulative incidence of neutrophil engraftment and the cumulative incidence of platelet engraftment, as defined by peripheral blood platelet count above 20,000 platelets per microliter;

100-day survival in the ProHema cohort compared favorably to both the inactive cohort and the historical control;

there was a low incidence of acute and chronic GvHD in the ProHema cohort; and

ProHema contributed to durable long-term hematopoietic reconstitution in a significant majority of the patients in the ProHema cohort and compared favorably to the historical control.

The following table shows the results observed in the ProHema-01 trial with respect to the key measures of time to engraftment, cumulative incidence of neutrophil engraftment, rate of failure to achieve neutrophil engraftment and 100-day survival:

Cohort	Median Time to Engraftment	Cumulative Incidence of Neutrophil Engraftment by Day 26	Rate of Failure to Achieve Neutrophil Engraftment	100-Day Survival
ProHema	17.5 days (range 14 31 days)	83%	0%	100%
Inactive	22.0 days (range 14 40 days)	67%	11%	89%
Historical	20.5 days (range 13 70 days)	70%	6%	87%

The ProHema cohort also compared favorably to both the inactive cohort and the historical control in other measures of engraftment, including the cumulative incidence of platelet engraftment by Day 100 and the rate and incidence of cumulative engraftment as defined by absolute neutrophil count and platelet count. The following graphs show the rate and incidence of absolute neutrophil count and platelet count in the ProHema cohort, as compared to the historical control:

Rate and Incidence of Neutrophil Engraftment

Rate and Incidence of Platelet Engraftment

We also evaluated the incidence of GvHD and observed a low incidence of acute GvHD in the twelve subjects in the ProHema cohort. By Day 100, there was an 8% incidence of Grade II-IV acute GvHD in the ProHema cohort, as compared to 17% in the historical control group. One patient in the ProHema cohort experienced mild chronic GvHD.

Additionally, we performed an assessment of the ProHema cohort and the historical control to determine which of the two cord blood units contributed to long-term hematopoietic reconstitution. This analysis determined that, at Day 100, 83% of patients (10 of 12) in the ProHema cohort had achieved predominant

hematopoietic reconstitution with ProHema as opposed to the unmodulated cord blood unit. In contrast, at Day 100, the profile of hematopoietic reconstitution in the historical control was substantially diverse: 34% of patients engrafted with the first cord administered to the patient; 34% of patients engrafted with the second cord administered to the patient; and 8% of patients persisted in a state referred to as dual chimerism, where both cords contributed to hematopoietic reconstitution, and the remainder either experienced graft failure or died prior to Day 100. With a median follow-up among survivors of 24.6 months, no patient in the ProHema cohort experienced secondary graft failure, or graft failure following an initial period of engraftment. In addition, the one-year and two-year progression-free survival rates in the ProHema cohort were 61.7% and 31.3%, respectively. The corresponding one- and two-year overall survival rates in the ProHema cohort were 75% and 38.9%, respectively. Post-100 day survival rates in the inactive cohort and in the historical control were not available for analysis in the ProHema-01 trial.

Safety Assessment

The trial met all established safety criteria and demonstrated that ProHema was well tolerated. Adverse events attributed to ProHema consisted of mild to moderate infusion-related events consisting of rash, nausea, chills, flushing, abdominal pain, and cough, all of which are considered common transplant-related side effects. One subject with known coronary artery disease experienced transient myocardial ischemia that resolved promptly after completion of the infusion.

ProHema-01 Trial Conclusion

We believe the results of our ProHema-01 trial demonstrate human proof-of-concept that the *ex vivo* pharmacologic modulation of HSCs has the potential to improve the key clinical measures of time to, and durability of, neutrophil engraftment. These improvements were demonstrated in allogeneic HSCT using a RIC regimen that is less toxic to patients and an HSC source that increases HLA compatibility and reduces the risk of GvHD.

In an End-of-Phase 1 meeting with the FDA in the first quarter of 2012, we received guidance from the FDA on potential Phase 3 clinical trial endpoints. This guidance suggested that time to engraftment of neutrophils, platelets, or both may be a sufficient primary endpoint to support approval, and that a single Phase 3 trial, enrolling both adult and pediatric subjects, may be sufficient for approval in both age groups, depending on the results.

The ProHema-01 trial was designed with safety as the primary endpoint and not efficacy. To support marketing approval, we will need to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that ProHema is safe and effective, and otherwise meets the appropriate standards required for approval for each targeted indication, in subsequent well-designed and conducted clinical trials, including our Phase 2 clinical trial and a Phase 3 registrational trial that we intend to initiate if our Phase 2 trial is successful. We may not be able to achieve or replicate the results of our Phase 1b clinical trial in our Phase 2 clinical trial or other subsequent trials for a variety of reasons. For example, the anticipated use of our NRM formulation referred to below in our Phase 2 clinical trial may not produce the efficacy or safety benefits that we currently expect; the increase in the number of patients enrolled in later-stage trials may not produce the same or similar results as earlier trials with fewer patients; and the expansion in the number of participating clinical centers in later-stage trials may present operational and manufacturing challenges.

Improved Nutrient-Rich Media Formulation to Enhance the Potency of ProHema

In our ProHema-01 trial, ProHema was manufactured using standard processing media, which is commonly used throughout the clinical setting today for the thawing and washing of umbilical cord blood units. During the second quarter of 2013, we completed additional *in vitro* and animal studies demonstrating that the potency and efficacy profile of ProHema can be significantly improved by using our new nutrient-rich media formulation, which we refer to as our NRM formulation.

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The manufacture of ProHema using our improved NRM formulation, as compared to the use of standard processing media, results in increased expression of PGE2-related genes and improved performance in *ex vivo* homing assays. In addition, the new manufacturing conditions also improved the viability, as measured by HSC recovery. The homing potential of HSCs, as measured by an *in vitro* transwell migration assay, was also improved. The results of our studies using *in vitro* assays are summarized below:

Biologic Measure of Activity	Prior Media	NRM
Expression of relevant genes	2-6 fold	9-126 fold
Homing potential	7%	34%
Viable HSC Recovery	88%	107%
Increase in HSC population	62%	131%

These enhanced modulation effects translated into significantly improved homing and a more than two-fold improvement in engraftment in mouse models, as shown in the graphs below:

Based on the data described above, we believe that the use of our NRM formulation will improve ProHema s potency and efficacy profile in the clinical setting. We intend to incorporate our improved NRM formulation into our clinical development program for ProHema.

Phase 2 Clinical Development in Adult Patients with Hematologic Malignancies

In December 2012, we initiated our ProHema-03 trial, a randomized, controlled, Phase 2 multi-center clinical trial of ProHema in adult patients undergoing double UCBT for hematologic malignancies using both MAC and RIC regimens. Our ProHema-03 trial is currently active at eight major allogeneic HSCT centers in the United States but not recruiting. We recently notified the FDA that we have elected to pause enrollment to enable the manufacture of ProHema incorporating our NRM formulation and to generate data to qualify the optimized manufacturing process that incorporates the NRM formulation. On August 1, 2013, we submitted an IND amendment to the FDA, which contains preclinical and product development data to support the incorporation of our NRM formulation for the manufacture of ProHema and support that the use of the NRM formulation should not result in additional safety risks. In addition, we submitted an amended protocol for our ProHema-03 trial that defines how we will resume enrollment with ProHema as manufactured using our NRM formulation. Specifically, we stated that we will enroll the full 60 subjects under the revised ProHema manufacturing process and that the previously enrolled subjects will be followed and analyzed separately. Based on our recent communications with the FDA regarding the IND amendment, we expect to resume enrollment of our ProHema-03 trial in the first half of 2014, with the goal of generating full data from this trial in mid-2015. However, the FDA may require us to generate additional preclinical or clinical data to support the use of our NRM formulation

in our ProHema-03 trial or may impose additional requirements on our clinical development activities for ProHema, which may cause delays in enrollment and in the availability of full data from our ProHema-03 trial.

Prior to our election to pause enrollment of our ProHema-03 trial, 11 patients conditioned using a MAC regimen had either consented to enrollment or been enrolled into the study. Eight of these patients were randomized to receive ProHema plus an unmanipulated cord blood unit, and three were randomized into the control arm to receive two unmanipulated cord blood units. No patients conditioned using a RIC regimen were enrolled. The three subjects in the control arm engrafted at Days 30, 31 and 40, yielding a control median of 31 days. Five of the eight subjects in the ProHema arm engrafted prior to the control median, at Days 14, 19, 24, 28 and 30. Two of the eight subjects in the ProHema arm engrafted post the control median at Days 40 and 48, and one of the eight subjects in the ProHema arm failed to engraft. No patients to date have experienced secondary graft failure. To date, of the eight subjects in the ProHema arm, four subjects survived to Day 100, two subjects died before Day 100 and two subjects engrafted, remain alive and have not yet reached Day 100. The three subjects in the control arm survived to Day 100. With a median follow-up period of 156 days, five of eight subjects in the ProHema arm remain alive and have engrafted, as compared to one of three subjects in the control arm. One subject in the ProHema arm experienced Grade IV acute GvHD, and one subject in the control arm experienced Grade III acute GvHD. Adverse events attributed to ProHema were primarily limited to common infusion-related side effects. We believe these results are consistent with expected outcomes in adult patients undergoing HSCT using umbilical cord blood after a MAC regimen without ProHema.

Upon resuming enrollment, the trial is expected to enroll 60 additional adult patients across both MAC and RIC regimens using our NRM formulation. Patients in this trial will be randomized, at a ratio of 2:1, with approximately 40 patients receiving ProHema plus an unmanipulated cord blood unit and approximately 20 patients receiving two unmanipulated cord blood units. Prior to randomization, patients will be stratified based upon whether a RIC or MAC regimen will be employed. The primary endpoint of the trial is the cumulative incidence of neutrophil engraftment by a pre-specified control median, which will be adjusted based upon the median times calculated for subjects enrolled to the control arm. The study is designed to demonstrate with statistical significance that 70% of the subjects in the ProHema arm achieve neutrophil engraftment before the control median engraftment time. Secondary endpoints include additional measures of engraftment, including time to neutrophil engraftment, cumulative incidence of neutrophil engraftment by Day 42, time to platelet engraftment, cumulative incidence of platelet engraftment by Day 180, as well as rates of graft failure and of GvHD and event-free and overall survival.

If our ProHema-03 trial is successful, we plan to seek additional regulatory guidance with the goal of initiating a Phase 3 registrational trial of ProHema, which may include both adult and pediatric patients, undergoing UCBT for hematologic malignancies. Based on the regulatory guidance obtained to date, and preliminary statistical power calculations, we believe the Phase 3 program could consist of a single trial enrolling approximately 200 patients, with time to engraftment of neutrophils, platelets, or both as an endpoint to support approval.

Preclinical Development and Clinical Development Plans in Pediatric Patients with Hematologic Malignancies

For pediatric patients, the standard of care in UCBT for the treatment of hematologic malignancies utilizes a single cord blood unit. While the cell dose received by a pediatric patient from a single cord blood unit can be sufficient, data suggests that pediatric patients undergoing single UCBT still suffer from delayed engraftment, high rates of graft failure and high rates of transplant-related morbidity and mortality.

To explore the potential of ProHema in a pediatric patient population, we have initiated a Phase 1 clinical trial to determine safety in the setting of single UCBT in adults with hematologic malignancies, which we refer to as our ProHema-02 trial. Qualifying patients receive the same RIC regimen that was used in our ProHema-01 trial. After conditioning, patients receive a single ProHema cord blood unit. The primary endpoint of the trial is safety. We are also analyzing a range of engraftment measures, as well as rates of GvHD, relapse and survival.

The trial has enrolled eight subjects. Of the eight subjects, six subjects are evaluable, age 19-64 years (median 55.9 years), with the following diagnoses: acute myelogenous leukemia (four subjects), myelodysplastic syndrome (one subject) and multiple myeloma (one subject). Four of the six evaluable subjects engrafted at

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Days 17, 19, 22 and 37, and two experienced primary graft failure. No patients experienced secondary graft failure. Survival at 100 days was 100%. No acute or chronic GvHD has been observed to date. Adverse events attributed to ProHema were limited to common transplant-related side effects.

Based on these results, we engaged in a preliminary review of the ProHema-02 data with the FDA and discussed our intent to conduct a Phase 1b trial in children and adolescents with hematologic malignancies. The FDA indicated that it was open to our conducting such a pediatric trial, but requested a written summary of the ProHema-02 trial as well as a synopsis of our proposed Phase 1b trial in pediatric patients with our justifications for the trial design. Subject to our submission of the requested information and FDA approval of the final study protocol, we plan to initiate a Phase 1b clinical trial in children and adolescents with hematologic malignancies, in which patients would receive a single ProHema unit. The primary endpoint of the trial is expected to be safety as defined by neutrophil engraftment. Secondary endpoints are expected to include additional measures of engraftment, including time to neutrophil engraftment, cumulative incidence of neutrophil engraftment by Day 42, time to platelet engraftment, cumulative incidence of platelet engraftment by Day 180, as well as rates of graft failure and of GvHD and event-free and overall survival. We anticipate commencing enrollment in our planned Phase 1b clinical trial in pediatric patients during 2014 and conducting the trial at one to three clinical centers in the United States. We expect to use our NRM formulation in this trial. In addition, we believe we can conduct our planned Phase 1b clinical trial in pediatric patients under our current IND for ProHema, and thus we may be able to amend our existing IND in order to commence this planned clinical trial. Although we currently believe that amending our existing IND will suffice, we will need to submit clinical development plans to the FDA before we can commence this trial. The FDA may disagree with our plans and require us to file a new IND before we can commence clinical trials of ProHema for the treatment of hematologic malignancies in pediatric patients.

Our Opportunity in Rare Genetic Disorders

Overview

The steady growth in the number of HSCT procedures to treat patients with hematologic malignancies has been paralleled by an increase in the use of HSCT for rare genetic disorders. The treatment of rare genetic disorders requires allogeneic HSCT, as it provides HSCs from a healthy donor, which carry a normal version of the defective gene. It is estimated that over 50 rare, genetic disorders, many of which are life-threatening and lack alternative therapeutic options, have been treated with allogeneic HSCT to date, including:

- LSDs, including Hurler syndrome, Krabbe disease and metachromatic leukodystrophy;
- peroxisomal storage disorders, including adrenoleukodystrophy;
- hemoglobinopathies, such as sickle cell disease and certain thalassemias;
- inherited bone marrow failure syndromes, such as Fanconi anemia and Diamond-Blackfan anemia; and
- inherited immune deficiencies, such as Wiskott-Aldrich syndrome.

The transformative effect of allogeneic HSCT, and UCBT in particular, across these rare genetic disorders has been demonstrated and published in numerous clinical studies, case series and retrospective analyses of multi-national patient registries. For instance, long-term follow up of children with LSDs and peroxisomal storage disorders who underwent allogeneic HSCT has shown that the progressive worsening of many clinical manifestations can be prevented or substantially reduced through early allogeneic HSCT intervention. These effects have been attributed to the ability of HSCs to home to and engraft within the CNS, where they give rise to microglia cells that become a permanent source of enzyme supply through a process called cross-correction.

It is well-recognized that umbilical cord blood has several important advantages over bone marrow and mobilized peripheral blood as a source of HSCs in the setting of allogeneic HSCT for LSDs. First, compared to the hematologic malignancy setting, even more patients lack a suitable related or matched unrelated donor. Second, cord blood can be readily accessed and can reduce time from diagnosis to transplant, a critical factor for patient outcomes, especially in patients with early-onset and rapidly progressing disorders, such as infantile Hurler syndrome or

Krabbe disease. Furthermore, there is growing evidence that the proportion of patients

achieving normal enzyme levels is higher following allogeneic HSCT with cord blood than with traditional HSC sources, which may improve the chances of reversing or halting the progressive manifestations of the disorder.

Unmet Medical Need

The key factors that determine HSCT patient outcomes in the hematologic malignancy setting are also highly relevant for rare genetic disorders and include:

Reconstitution. Timely and durable reconstitution of donor-derived HSCs is a critical success factor following allogeneic HSCT in patients with rare genetic disorders. Additionally, in patients with demyelinating LSDs, the homing of donor-derived HSCs across the blood-brain barrier is critical to arresting the degenerative effects of demyelination.

HLA matching. The degree of HLA matching is an important determinant of outcome following allogeneic HSCT in rare genetic disorders. Specifically, for certain LSDs, the rapid and irreversible progression of the disease requires urgent intervention and the immediate need to find an HLA-matched HSC source. We believe our ability to use pharmacologically optimized cord blood will reduce the time to transplant and improve patient outcomes.

Patient conditioning. Allogeneic HSCT procedures for rare genetic disorders are routinely performed using MAC regimens, because attempts to utilize RIC regimens have resulted in unacceptably high graft failure rates. The use of these highly toxic MAC regimens in infants and young children with rare genetic disorders is of significant concern. We believe the enhanced engraftment potential of our pharmacologically optimized HSCs will enable the broader adoption of RIC regimens.

Potential of Our HSC Modulation Platform in Rare Genetic Disorders

Given our preclinical findings of enhanced homing and engraftment, as well as the clinical proof-of-concept that we have achieved for our HSC modulation platform in the hematologic malignancy setting, we believe that pharmacologically-modulated HSCs have considerable potential to improve outcomes following allogeneic HSCT for rare genetic disorders. We are initially planning to study an *ex vivo* pharmacologically-modulated HSC therapeutic in pediatric patients with demyelinating LSDs. We plan to evaluate this potential both in an initial clinical trial of ProHema, as well as through a focused research program to identify other product candidates.

Preclinical Data. We have demonstrated in a preclinical model that *ex vivo* modulated cord blood increases the number of donor cells that home and migrate across the blood-brain barrier into the CNS. We treated human cord blood-derived HSCs with FT1050 or vehicle control for two hours at 37°C and injected into sub-lethally irradiated NSG mice. Twenty hours following injections, genomic DNA was isolated from the brain tissue of the mice and the number of human cells in each sample was determined. The figure below shows that homing properties of HSCs derived from human cord blood to the CNS were significantly improved by *ex vivo* modulation with FT1050:

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Clinical Plan. We plan to initiate a first clinical trial of ProHema in pediatric patients with demyelinating LSDs in 2014 after filing an IND amendment, with the goal of generating data from this trial in 2015. We believe we can conduct our planned initial clinical trial of ProHema in this patient population under our current IND for ProHema, and thus we may be able to amend our existing IND in order to commence this planned clinical trial. Although we currently believe that amending our existing IND will suffice, we will need to submit clinical development plans to the FDA before we can commence this trial. The FDA may disagree with our plans and require us to file a new IND before we can commence clinical trials of ProHema for the treatment of LSDs in pediatric patients. The primary objective of the trial will be to evaluate the potential of *ex vivo* enhanced HSCs to enable robust engraftment under RIC regimens, where previous studies have shown that unmodulated cord blood units did not perform well. This trial is expected to enroll patients between the ages of one to 21 years. After conditioning, patients will receive a ProHema unit in combination with an unmodulated unit. The first cohort of subjects will receive a conditioning regimen using a combination of high-dose chemotherapy agents that comprise a standard myeloablative regimen used for such transplants but in which one agent has been dose-reduced by 25%. Subsequent cohorts will receive conditioning regimens that are successively dose-reduced. The primary endpoint of the study will be neutrophil engraftment, such that a reduced intensity dosing regimen can be identified that results in consistent and prompt engraftment. Subjects will also be followed for other measures of engraftment and safety. In addition, subjects will undergo regular cognitive and functional evaluations to measure the impact of the HSCT procedure on developmental milestones. We expect the trial will be conducted at one to three centers that specialize in pediatric cord blood transplantation for rare genetic disorders.

Next-Generation HSC Modulators. We are using our HSC modulation platform to develop second generation therapeutics specifically designed to enhance the homing of HSCs to the CNS to improve delivery of essential enzymes that are deficient in patients with LSDs.

Our SSC Modulation Platform

Therapeutic Potential of SSCs in Muscle Regeneration

Skeletal muscle has a potent natural regenerative capacity. Muscle SSCs are regenerative precursor cells that play a key physiological role in the biological processes that drive skeletal muscle growth, maintenance and repair throughout a person s lifespan. In response to natural molecular triggers from exercise, injury or disease, SSCs become activated, proliferate, and either differentiate into *de novo* muscle fibers or fuse with, and augment, existing muscle fibers. The regenerative capacity of muscle is exhausted both in the natural aging process and in degenerative conditions such as muscular dystrophies, where there is a constant cycle of muscle damage and compensatory repair. We are applying our knowledge of stem cell modulation to develop novel biologic therapeutics based on the natural signals that stimulate SSCs *in vivo* to drive muscle regeneration in muscular dystrophies and other neuromuscular diseases and conditions.

Unmet Medical Need in Muscle Dystrophies

Muscular dystrophies encompass a group of rare diseases with diverse genetic bases and pathophysiological manifestations. The most prevalent and well-characterized forms are the X chromosome-linked Duchenne and Becker muscular dystrophies, or DBMDs, in which a loss or deleterious modification to the dystrophin protein results in significant and progressive muscle degeneration. There are many other distinct types of muscular dystrophies resulting from specific genetic mutations or deletions to over 30 distinct genes, including facioscapulohumeral muscular dystrophy, limb-girdle dystrophies and myotonic dystrophy. It is estimated that in the United States, DBMD occurs in one out of 3,500 live births, resulting in approximately 10,000 males living with these diseases. According to a 2007 study, over 80% of patients suffering from DBMD were wheelchair-bound by 14 years of age. In addition, DBMD patients usually do not live to the age of 30. There are no therapeutics specifically approved for the treatment of muscular dystrophies.

A core pathophysiologic phenomenon seen in muscular dystrophies is a cycle of muscle degeneration leading to continuous compensatory SSC activation and differentiation to affect a regenerative response. It is believed that the eventual exhaustion of this regenerative capacity results in accelerated tissue degeneration and, ultimately,

significant loss of muscle function. Several promising therapeutics aimed at preventing further muscular degeneration through the reestablishment of dystrophin function are currently in clinical development. These include oligonucleotide exon-skipping of specific mutations in a subset of DBMD patients, stop-codon override approaches and utrophin up-regulation. To our knowledge, there are no clinical-stage programs focused on driving the natural regenerative process to reestablish muscular strength. We believe that restoring the balance between muscle degeneration and regeneration to induce tissue repair represents a promising approach for the treatment of all muscular dystrophies irrespective of the causative genetic mutation.

We have used our knowledge and systematic interrogation of SSC biology to identify specific natural signaling molecules that drive the muscle regenerative response. Further, we have applied our expertise in protein engineering to design protein analogs with therapeutic potential and preferred pharmaceutical development properties.

Our Proprietary Wnt7a Analogs

We have identified Wnt7a, a naturally-occurring secreted protein, as a key regulator of skeletal muscle regeneration. We have demonstrated that a single administration of a Wnt7a analog resulted in a significant expansion of the SSC population and an increase in muscle hypertrophy. We have engineered analogs of Wnt7a and are developing them for regeneration in muscular dystrophies.

The role of Wnt7a as a potent stimulator of SSC population expansion and muscle hypertrophy was first identified by one of our scientific founders, Michael Rudnicki, Ph.D. This activity was shown to be dependent on a receptor known as Fzd7, which is predominantly expressed in skeletal muscle. Based on these findings, we believe that Wnt7a offers a highly-specific means to effect a regenerative response in skeletal muscle in order to treat neuromuscular diseases, irrespective of etiology. We own or have exclusively licensed worldwide rights to the use of Wnt7a in muscle regeneration.

Wnt7a is a member of a wider family of 19 secreted Wnt proteins known to play a central role in the processes of embryonic development, stem cell fate determination, tissue repair and homeostasis. Despite their widely-recognized importance throughout human physiology, to our knowledge, there are no Wnt proteins currently undergoing clinical development. This is primarily due to specific molecular characteristics that prevent their effective development as biologic therapeutics. We have systematically applied structural prediction, rational design and protein engineering techniques to overcome these challenges. We believe we are the first company to produce an analog of a Wnt protein that is amenable to manufacture, formulation and administration for *in vivo* therapeutic use. Our approach to the development of Wnt protein analogs encompasses the following advantages:

We have overcome manufacturing challenges. Natural Wnt proteins are expressed at very low levels in typical biologic manufacturing systems and are extremely difficult to purify while retaining activity. We have engineered Wnt compositions which enable effective, high level expression in commonly used host cells, thus enabling scaled recombinant manufacturing. We believe our proprietary Wnt compositions also allow scaled protein purification using methods commonly implemented by commercial biologic manufacturing organizations.

We have enabled therapeutic formulations. Natural Wnt proteins have limited solubility in preferred therapeutic excipients. Using structural biology, systematic engineering and signaling activity assessments, we have designed and produced Wnt proteins that retain activity and enable therapeutic formulation to allow *in vivo* administration.

Our product candidates can be readily administered. Natural Wnt proteins are characterized as locally acting signaling molecules, potentially limiting their therapeutic range on administration. We have demonstrated that our Wnt7a analogs induce significant regenerative effects across a whole muscle on a single administration of protein.

Our product candidates retain a high degree of specificity. There are 19 human Wnt proteins and over 15 different receptors and co-receptors that drive a number of diverse signaling pathways and biological mechanisms in a tissue-specific manner. We have engineered Wnt7a analogs that retain

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specificity for the signaling pathway implicated in muscle regeneration but are inactive in other characterized Wnt signaling pathways, thereby potentially avoiding off-target activity or toxicities.

We believe that our knowledge of the role of Wnts in stem cell biology, our proprietary approaches for engineering Wnt-based analogs and their methods of formulation and manufacture represent foundational expertise that can be leveraged beyond Wnt7a. We intend to assess other Wnt-based biologic modulators for use in broader regenerative medicine applications. We own or have exclusively licensed worldwide rights to intellectual property pertaining to the design, composition and methods of manufacture and use of our Wnt analog proteins.

Preclinical Proof-of-Concept for Our Proprietary Wnt7a Analogs

We have demonstrated the therapeutic potential of our proprietary Wnt7a analogs in various preclinical models. They have been shown to expand the population of SSCs, drive muscle hypertrophy, decrease disease-related muscle damage and increase muscle strength with similar potency as naturally-occurring Wnt7a in both wild-type rodents and rodent models of muscular dystrophy, or mdx. Additionally, in *in vitro* cultures of differentiated muscle cells, or myotubes, derived from healthy human subjects and from human subjects with various forms of muscular dystrophies, our proprietary Wnt7a analogs have been shown to drive muscle cell hypertrophy.

The Unique Dual Mechanism of Action of Wnt7a

In preclinical studies, we have demonstrated that a single injection of either Wnt7a or a Wnt7a analog to the *tibialis anterior* muscle of either wild type or mdx mice induces muscle hypertrophy and a significant expansion of the SSC population in a dose dependent manner. These effects are seen at three weeks following a single intramuscular injection of low microgram amounts of protein. In an example of these effects, we compared the hypertrophic activity of Wnt7a and a Wnt7a analog in treated muscle to both an injection of relevant formulation control and the equivalent untreated muscle on the opposite side of the body in the relevant animal model, which we refer to as the contralateral control. We demonstrated a statistically significant hypertrophic effect of Wnt7a and a Wnt7a analog relative to the contralateral control in the wild-type mouse represented by an approximately 20% increase in the median muscle fiber minimum cross-sectional diameter. We also demonstrated a statistically significant increase in the number of muscle SSCs, represented by an approximately three-fold increase in the number of Pax7 positive cell nuclei, a marker for SSCs, in the treated muscle relative to the contralateral control. The figures below show our preclinical results demonstrating an increase in muscle hypertrophy and SSC population expansion:

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Wnt7a Induced Regeneration Reduces Inflammation and Muscle Damage

Muscle fiber necrosis and inflammation are common abnormalities associated with muscular dystrophies that contribute to tissue fibrosis and a reduction in strength and regenerative capacity. Inducing muscle regeneration in the mdx mouse through a single administration of Wnt7a or a Wnt7a analog results in increased muscle fiber integrity and reduced inflammatory cell infiltration of the tissue. In preclinical studies, we demonstrated a statistically significant reduction in disease-specific muscle fiber necrosis measured as the mean IgG-positive fibers per unit area of muscle and the reduction in positive staining of a cellular biomarker of inflammation, CD11b, within the muscles of mdx mice. The figures below show these results comparing Wnt7a or a Wnt7a analog to formulation control:

Improvement in Muscular Strength

The mdx rodent model of muscular dystrophy is significantly weaker than a wild-type rodent, as measured by specific force. Specific force is the normalization of force per cross-sectional area of muscle and represents a standard and accurate measure of muscular strength. In preclinical studies, we demonstrated that a single administration of Wnt7a or a Wnt7a analog protein induced a statistically significant increase of approximately 17-19% in the specific force or strength generated by the mdx rodent *tibialis anterior* muscle. The figures below show these results comparing Wnt7a or a Wnt7a analog to formulation control:

Activity on Human Dystrophic Muscle Cells

There are more than 30 distinct forms of muscular dystrophies. Each type can differ based on the muscles affected, the age of onset and genetic cause. For example, Duchenne and Becker muscular dystrophies are caused by a deficiency of the dystrophin protein due to a range of mutations in the dystrophin gene. In contrast, facioscapulohumeral dystrophies are thought to be caused by a defect in the expression of the DUX4 gene and

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are characterized by muscle weakness in the face, shoulders and upper arms. In *in vitro* cultures of myotubes derived from healthy human subjects and from human subjects with Duchenne, Becker and facioscapulohumeral dystrophies, our proprietary Wnt7a analogs have been shown to drive muscle cell hypertrophy. We believe these studies support the potential for our proprietary Wnt7a analogs to regenerate human skeletal muscle and to drive muscle hypertrophy across different types of muscular dystrophies irrespective of the underlying genetic cause.

Wnt7a Analog Development Strategy for Muscular Dystrophies

We are currently expanding these preclinical assessments to include dose and regimen optimization in rodent models. We also plan to initiate efficacy and pharmacokinetic assessments in a well characterized canine model of muscular dystrophy to assess the effects in larger muscle groups, allowing for a more predictable transition of dose and administration regimen to human trials.

We have identified potential Wnt7a-specific pharmacodynamic biomarkers, which can be attained through a pre- and post-treatment punch biopsy, to accelerate our clinical development process. These include both cellular effects, such as muscle hypertrophy and SSC population expansion, and molecular signatures based on whole genome expression analysis of Wnt7a-treated muscle. We have identified specific molecular signatures that represent potential biomarkers that may be measured in clinical trials.

We are currently conducting preliminary, non-GLP toxicology assessments with dose escalation, which will inform future IND-enabling toxicology studies. Our initial clinical focus for the Wnt7a analog program is to gain safety information and demonstrate human proof-of-concept in X chromosome-linked dystrophy patients with local administration of therapeutic protein to targeted muscle groups.

Subject to the completion of IND-enabling studies, we plan to file an IND in 2014 to initiate a Phase 1 clinical trial to provide an initial safety assessment in healthy volunteers, with the goal of generating data from our first clinical trials in 2015. In addition, we plan to assess biological activity using histological and gene expression pharmacodynamic markers and measures of muscle strength by electromyography. These biomarkers will enable us to optimize dose and treatment regimens quickly.

Based on the results of our Phase 1 clinical trial, we plan to initiate a study in an X chromosome-linked muscular dystrophy patient population. We believe that the combination of a Phase 1 clinical trial in healthy volunteers, a dose escalation trial in an X chromosome-linked muscular dystrophy population and the establishment of effective pharmacodynamic biomarkers will allow us to efficiently assess both safety and efficacy for our Wnt7a analogs. We also believe these studies will provide a strong foundation for further discussions with the FDA regarding the path to approval in muscular dystrophies.

Indication Expansion Opportunities

We have demonstrated that Wnt7a is both a potent and a specific regulator of SSC population expansion and muscle hypertrophy and integrity. We have identified several Wnt7a analogs that we believe have therapeutic potential. While we are pursuing the development of a lead Wnt7a analog for the treatment of muscular dystrophies, we believe that this analog, as well as certain other Wnt7a analogs, may have potential in treating a wider range of neuromuscular degenerative conditions including cachexia, atrophy, trauma, and sarcopenia. We are currently exploring therapeutic efficacy in additional preclinical models. We believe that the clinical assessment of safety and efficacy of our first Wnt7a analog in healthy volunteers and in muscular dystrophy patients can provide a basis for exploring the therapeutic benefit of Wnt7a in a wider array of neuromuscular disorders.

Additional Research and Discovery Activities

In addition to our two stem cell modulation platforms, we are advancing proprietary technologies for the industrial-scale generation, expansion and maintenance of induced pluripotent stem cells, or iPSCs. The ability to generate iPSCs is recognized to be one of the most important discoveries of the last decade. iPSCs are generated in a process by which fully-differentiated mature cells, such as skin cells or blood cells, are reprogrammed to a

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less-differentiated, embryonic stem cell-like state through the expression of certain pluripotency genes. Over the past five years, iPSCs have been used to produce cardiomyocytes and hepatocytes for the purposes of conducting drug toxicology testing and to produce other cell types for modeling human diseases, such as Parkinson s disease, Huntington s disease and Duchenne s muscular dystrophy. We are currently deploying our iPSC technology in the development of our stem cell modulators.

Our technology is built upon the discoveries and inventions of two of our scientific founders, Drs. Rudolf Jaenisch and Sheng Ding, both of whom are considered pioneers in the field of iPSC technology. We believe that our proprietary iPSC technology enables both the efficient, high throughput generation of stable, well-qualified iPSCs and the large-scale expansion and maintenance of iPSCs. We have exclusively licensed patents and patent applications, and developed proprietary technologies, that we believe are foundational to the practice of iPSC technology for commercial purposes. The key proprietary features and benefits of our iPSC technology include:

Patent-protected cellular compositions of reprogramming. One of the key pluripotency genes typically relied on for the generation of iPSCs is Oct4. The cellular composition comprising a somatic cell having an exogenous nucleic acid that encodes an Oct4 protein is a patent-protected composition of matter in the United States which we have exclusively licensed for commercial purposes.

Patent-protected small molecule combination for reprogramming. We incorporate a patent-protected small molecule in our culture systems of reprogramming. The use of these systems results in a 50-fold increase in reprogramming efficiency.

Proprietary methods for industrial-scale iPSC generation. We have developed an automated method for high-throughput iPSC generation which directly selects high-quality iPSC cells through proprietary combinations of cell surface antibodies. This method significantly enhances the throughput and quality of cellular reprogramming and enables industrial applications, such as disease modeling and toxicology screening from multiple genetic backgrounds.

Proprietary culture systems for iPSC expansion and maintenance. We have developed a proprietary small molecule-enhanced culture system which enables large-scale iPSC culture expansion while maintaining high quality, homogeneous cells. We believe this culture system enables commercial applications of iPSC technology, such as drug screening and, ultimately, iPSC-based cell therapies.

In September 2010, we entered into a collaboration and license agreement with Becton, Dickinson and Company, or BD. The goal of the collaboration is to provide life science researchers and the pharmaceutical community with reliable access to certain advanced iPSC tools and technologies for use in human disease research, drug discovery and development, and the manufacture of cell-based therapies. Under the collaboration and license agreement, we agreed to co-develop certain stem cell reagent products with BD for a period of three years ending in September 2013, and BD has the right to commercialize any co-developed stem cell reagent products on a worldwide basis.

In June 2012, BD commercially launched the first stem cell product co-developed under the collaboration, BD SMC4, which is a patent-protected, pre-formulated cocktail of small molecules for improving cellular reprogramming efficiencies.

Our Intellectual Property

Overview

We strive to protect our product candidates and our stem cell modulation platforms through a variety of methods, including seeking and maintaining patents intended to cover our products and compositions, their methods of use and processes for their manufacture, our platform technologies and any other inventions that are commercially important to the development of our business. We have entered into exclusive license agreements with various academic and research institutions to obtain the rights to use certain patents for the development and commercialization of our product candidates. We also rely on know-how, continuing technological innovation and in-licensing opportunities to develop and maintain our proprietary position. We seek to obtain domestic and international patent protection and endeavor to promptly file patent applications for new commercially valuable inventions to expand our intellectual property portfolio.

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Our intellectual property portfolio is currently composed of 46 issued patents and 174 patent applications that we license from academic and research institutions and 40 patent applications that we own, and these patent and patent applications generally provide us with the rights to develop our product candidates in the United States and worldwide. This portfolio covers (i) our HSC modulation platform, including ProHema; (ii) our SSC modulation platform, including our Wnt7a analogs and (iii) our other technologies, such as our iPSC technology. We believe that we have a significant intellectual property position and substantial know-how relating to the modulation of adult stem cells, including HSCs and SSCs.

We continually assess and refine our intellectual property strategy in order to fortify our position in our target market. To that end, we are prepared to file additional patent applications in any of the above fields if our intellectual property strategy requires such filings, or where we seek to adapt to competition or seize business opportunities. Further, we are prepared to file patent applications relating to new technologies we develop soon after the experimental data necessary for a strong application become available and our cost-benefit analyses justify filing such applications.

In addition to filing and prosecuting patent applications in the United States, we typically file counterpart patent applications in additional countries where we believe such foreign filing is likely to be beneficial, including Europe, Japan, Canada, Australia and China.

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 we may own or license in the future, nor can we be sure that any of our existing patents or any patents we may own or license in the future will be useful in protecting our technology. Please see Risk Factors Risks Related to Our Intellectual Property for additional information on the risks associated with our intellectual property strategy and portfolio.

Intellectual Property Relating to Our HSC Modulation Platform and ProHema

We own six families of pending U.S. and foreign patent applications covering our HSC modulation platform. This portfolio includes 14 pending applications relating to ProHema and other therapeutic compositions of stem cells that have been pharmacologically modulated to enhance their therapeutic properties, and methods of manufacturing the cellular compositions. Applications in this portfolio include claims covering (i) a therapeutic composition of human HSCs that have been modulated *ex vivo* with an agent, such as a prostaglandin agonist, resulting in increased expression of genes associated with the beneficial biological properties of the cells and (ii) methods of improving HSCT and methods of treating patients requiring hematopoietic reconstitution, such as patients undergoing chemotherapy or radiation therapy for cancer, including hematologic malignancies, and patients with non-malignant blood disorders, as well as disclosures of methods for preparing cell populations for transplant, as well as a cell culture media, including NRM, for improved processing and modulating populations of cells *ex vivo* and methods describing a cell potency assay for determining or validating the therapeutic potential in cell populations. Any U.S. patents issued from these applications will have statutory expiration dates between 2030 and 2034.

We have an exclusive license to a portfolio consisting of two families of issued patents and pending patent applications co-owned by the Children s Medical Center Corporation and The General Hospital Corporation. We currently have exclusive rights to nine issued patents and 27 pending patent applications in the United States and worldwide relating to methods for promoting tissue growth or regeneration (including of the hematopoietic system) using modulators that up-regulate the prostaglandin signaling pathway or its downstream mediators. These patent rights consist of an issued U.S. patent (U.S. Patent 8,168,428) claiming a method for promoting HSC engraftment through the *ex vivo* modulation of HSCs using FT1050, including HSCs obtained from cryopreserved cord blood, bone marrow and mobilized peripheral blood. Pending applications in the United States and foreign jurisdictions are directed to therapeutic compositions of HSCs derived from cord blood, wherein the cells have been modulated by increasing prostaglandin activity, methods of preparing these compositions, and methods of promoting hematopoietic reconstitution, expansion and self-renewal using modulators that increase prostaglandin signaling activity. Any patents within this portfolio that have issued or may yet issue will have a statutory expiration date in 2027.

We license exclusive rights to two families of patent applications from the Indiana University Research and Technology Corporation claiming methods of enhancing HSCT procedures by altering prostaglandin activity in HSCs and progenitor cells and methods for enhancing gene transduction efficacy in stem cell gene therapy. These applications describe methods of increasing mobilization of stem cells from a stem cell donor, and methods for increasing HSC homing and engraftment in a stem cell transplant recipient. One family of applications is directed to preferentially modulating certain receptors present on HSCs to increase the therapeutic potential of such cells for homing and engraftment. Claims in these applications specifically cover the modulation of umbilical cord blood by altering prostaglandin activity and methods for increasing gene transduction efficacy for gene therapy. These applications are currently pending in the United States and in certain foreign jurisdictions, and U.S. patents, if issued, from the applications could have terms expiring in 2029 or 2030.

We also license from the University of Rochester on exclusive terms a family of patent applications pending in the United States, Japan and the European Patent Office covering methods of expanding HSC populations *in vivo* or *ex vivo* using compositions comprising prostaglandin or a prostaglandin receptor agonist, including methods of selectively expanding highly proliferative short term HSCs to decrease recovery time in patients undergoing HSCT. Any U.S. patents that may issue from these applications would have a statutory expiration date in 2027.

To supplement our rights to develop and commercialize ProHema, we also have exclusive rights under additional license agreements with academic institutions to patents and patent applications that cover various methods for enhancing HSCT and modulating HSCs, including methods for increasing HSC numbers, promoting engraftment and increasing stem cell mobilization.

Intellectual Property Relating to Our SSC Modulation Platform and Wnt Analogs

In support of our program for the modulation of SSCs using Wnt analogs, we own pending patent applications in the United States and internationally pursuant to the Patent Cooperation Treaty covering compositions of matter, including Wnt polypeptide analogs having production and formulation advantages, as well as formulations containing such Wnt analogs suitable for local and systemic administration, and methods of preparing such Wnt proteins and formulations. These applications specifically disclose and claim our proprietary Wnt7a analogs and formulations containing these Wnt7a analogs that have enhanced production characteristics. Our applications also describe methods of using our novel Wnt analogs for the regeneration of injured or diseased muscle tissue, and include claims to methods of treating a spectrum of diseases and conditions affecting muscle and muscle degenerative diseases, such as muscular dystrophies. Any U.S. patents that may issue from these applications will have a statutory expiration date in 2032 or 2033.

We also license exclusive rights from the Board of Trustees of the Leland Stanford Junior University, or Stanford, to a PCT application directed to novel Wnt proteins that provide enhanced characteristics for producing therapeutic formulations of Wnt proteins, formulations of such proteins, and methods of manufacturing such proteins. Patent protection, to the extent it issues, would be expected to extend to 2032.

We also obtained rights, as the successor in interest to Verio Therapeutics, Inc., or Verio, to a portfolio of U.S. and international patents and patent applications owned by the Ottawa Hospital Research Institute, or OHRI, that supports our program for the treatment of muscle degeneration. These applications were licensed exclusively to Verio under a restated license agreement between Verio and OHRI effective April 2010. This portfolio includes patent applications directed to a novel population of SSCs, enhanced Wnt protein analogs, and the modulation of SSCs to promote muscle regeneration. These issued patents and applications include claims to compositions of novel stem cell populations and methods of treating muscle degenerative disorders by driving SSC population expansion and using small molecules or proteins to promote muscle tissue formation and muscle hypertrophy. These issued patents and any patents that may issue from these pending patent applications will expire on dates ranging from 2022 to 2033.

iPSC Intellectual Property

We own an international patent application that covers our proprietary small molecule-enhanced cell culture system which enables large-scale iPSC culture expansion while maintaining high quality, homogeneous cells. This application also covers a method for industrial-scale iPSC generation. Any patents issued from this application will expire in 2031.

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We have an exclusive license in commercial fields, including for drug discovery and therapeutic purposes, to a portfolio of four patent families including issued patents and pending applications broadly applicable to the reprogramming of somatic cells. This portfolio covers the generation of human pluripotent cells from somatic cells, and includes two issued patents (U.S. Patents 8,071,369 and 7,682,828) claiming compositions employed in reprogramming mammalian somatic cells to a less differentiated state (including to a pluripotent state). These issued patents and any patents that may issue from these pending patent applications will expire on dates ranging from 2024 to 2029.

We also have an exclusive license to a portfolio of seven patent families relating to compositions and methods for reprogramming mammalian somatic cells, which covers non-genetic and viral-free reprogramming mechanisms, including the use of various small molecule classes and compounds and the introduction of cell-penetrating proteins to reprogram mammalian somatic cells. This portfolio includes an issued patent (U.S. Patent 8,044,201) that provides composition of matter protection for a small molecule, thiazovivin, that improves the efficiency of induction of reprogramming in somatic cells, and compositions and methods of using the small molecule. Any issued patents and any patents that may issue from pending patent applications in this portfolio will have statutory expiration dates ranging from 2026 to 2032.

Our Material Technology License Agreements

Children s Medical Center Corporation

In May 2009, we entered into a license agreement with Children s Medical Center Corporation, or CMCC, for rights relating to therapeutic compositions of modulated HSCs and methods for promoting reconstitution of the hematopoietic system using modulators of the prostaglandin pathway, as described in more detail above under Intellectual Property Relating to Our HSC Modulation Platform and ProHema. Under our agreement with CMCC, we acquired an exclusive royalty-bearing, sublicensable, worldwide license to make, use and sell products covered by the licensed patent rights, and to perform licensed processes, in each case, in all fields. CMCC retains a non-exclusive right to practice and use the patent rights for research, educational, clinical or charitable purposes, and also to license other academic and nonprofit organizations to practice the patent rights for research, educational, and charitable purposes (but excluding any clinical use and commercialization of the patent rights to the extent granted to us under the license agreement). Our license is also subject to pre-existing rights of the U.S. government and rights retained by the Howard Hughes Medical Institute and the General Hospital Corporation to use the patent rights for research purposes. Additionally, if we make any discovery or invention that is described in a patent application and is not within the scope of the licensed patent rights but would not have been made but for the licensed patent rights, we are required to disclose the invention to CMCC and enter into a non-exclusive license agreement with CMCC, for no more than a nominal fee, for CMCC to practice the invention solely for internal research purposes or clinical purposes and not for commercial purposes.

Under the terms of the license agreement, we are required to pay to CMCC a yearly license maintenance fee during the term of the agreement. We also are required to make payments to CMCC of up to $5.0 million per product in development, regulatory and sales milestones. If commercial sales of a licensed product commence, we will pay CMCC royalties at percentage rates ranging in the low to mid single digits on net sales of licensed products in countries where such product is protected by patent rights. Our obligation to pay royalties continues on a country by country basis until the expiration of all licensed patent rights covering licensed products in such country, and our royalty payments will be reduced by other payments we are required to make to third parties until a minimum royalty has been reached. In the event that we sublicense the patent rights, CMCC is also entitled to receive a percentage of the sublicensing income received by us.

Under the license with CMCC, we are obligated to use commercially reasonable efforts to bring a licensed product to market as soon as practicable, and also to use good faith and diligent efforts to manufacture and distribute a licensed product, and make licensed products reasonably available to the public during the term of the agreement. We are also required to use good faith and diligent efforts to meet the milestones set forth in development plans as part of the agreement, subject to any revisions to the development plans that may be permitted under certain circumstances. Additionally, if a third party expresses interest in an area under the license that we are not pursuing, under the terms of our agreement with CMCC, we may be required to sublicense rights in that area to the third party.

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The agreement will continue until the last to expire of the patent rights. We may terminate the agreement by providing prior written notice to CMCC, and CMCC has the right to terminate the agreement if we fail to pay royalties or otherwise materially breach the agreement and fail to cure such breach within a specified grace period. CMCC may also terminate the agreement should we cease operations or in the event of our bankruptcy or insolvency.

The Board of Trustees of the Leland Stanford Junior University

In May 2013, we entered into an exclusive license agreement with Stanford for rights relating to novel Wnt analogs. Under our agreement, Stanford granted us an exclusive worldwide license to make, use and sell Wnt proteins and compositions of such proteins that are covered by the licensed patent rights for the treatment, prevention, and palliation of diseases, conditions, syndromes and maladies of humans and animals. The rights exclusively licensed to us under the license are described in more detail above under Intellectual Property Related to Our SSC Modulation Platform and Wnt Analogs.

Stanford retains the right, on behalf of itself and all other non-profit academic research institutions, to practice under the patent rights for any non-profit purpose, including sponsored research and collaborations. We may grant sublicenses to third parties so long as we are actively pursuing the development or commercialization of products covered by the patent rights. We may also be required to sublicense our rights under the agreement at Stanford s request under certain conditions, including if we are unwilling or unable to serve a potential market or territory and there is a third party willing to be a sublicensee in such market or territory.

We are obligated to pay to Stanford a yearly license maintenance fee during the term of the agreement, but we may offset the maintenance fee against earned royalty payments due on net sales occurring in that year. Stanford is entitled to receive a royalty as a percentage of net sales of licensed products, ranging from the low to mid single digits. Our agreement contains provisions for royalty offsets to the extent we need to obtain any rights from third parties to make, use, or sell the licensed products, subject to a minimum floor in the single digits. We have agreed to pay Stanford a percentage of non-royalty revenue we receive from our sublicensees, with the amount owed decreasing if we enter into the applicable sublicense agreement after meeting certain clinical milestones and, should we sublicense rights under the agreement with other patent rights, with the amount owed being apportioned between the patent rights under the agreement and any other rights sublicensed with the patent rights. In addition, we are obligated to pay Stanford up to approximately $900,000 upon the achievement of specific intellectual property, clinical and regulatory milestone events.

Under the license with Stanford, we are obligated to use commercially reasonable efforts to develop, manufacture, and commercialize at least one licensed product; to develop markets for such licensed products; and to meet certain development milestones as agreed upon between us and Stanford.

The agreement terminates on a country-by-country basis upon the last to expire of the patent rights in such country. We may terminate the agreement by providing prior written notice to Stanford, and Stanford has the right to terminate the agreement if we fail to achieve certain milestones or make payments under the agreement, or are not actively pursuing development of a licensed product, or if we otherwise materially breach the agreement and fail to cure such breach within a specified grace period.

Ottawa Hospital Research Institute

We acquired Verio in April 2010, and as the successor to Verio we acquired rights to various patents and patent applications pursuant to a restated license agreement between OHRI and Verio, which we refer to as the OHRI License. The licensed patents and patent applications under the OHRI License include issued patents and patent applications relating to the use of Wnt7a and analogs for the treatment of muscle degeneration, as described in more detail above under Intellectual Property Relating to Our SSC Modulation Platform and Wnt Analogs.

Through the OHRI License, we obtained an exclusive, worldwide, royalty-bearing license, with the right to sublicense, to develop, make, use and sell products covered by the licensed patent rights in all fields. OHRI retains the right under the OHRI License to practice the licensed technology and patent rights for non-commercial, research and academic purposes. We are obligated to pay OHRI an annual license maintenance fee,

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which is creditable towards any royalties owed under the OHRI License. We are also required to make payments to OHRI of up to CDN$1.4 million per product in connection with development, regulatory and commercial milestones. OHRI is entitled to receive a royalty in the low single digit range on net sales of licensed products, and we may offset any payments made to third parties to obtain rights needed for the commercialization of a licensed product against royalties payable to OHRI, provided that such expenses in a given year may not be credited against more than a specified percentage of the royalties payable to OHRI in such year. We have the right to sublicense our rights under OHRI License, and we are obligated to pay OHRI a percentage of any sublicense income.

Under the OHRI License, we are required to use commercially reasonable efforts to exploit the licensed patent rights in countries where it is commercially reasonable to develop licensed products, and to commercialize licensed products. We must also use commercially reasonable efforts to achieve development benchmarks described in the agreement in accordance with the specified time periods. If we fail to achieve a development benchmark in accordance with its applicable timeline, and OHRI determines that we have not used commercially reasonable efforts to develop the applicable product, OHRI may convert our license to the related patent rights to a non-exclusive license or may terminate the agreement, subject to our right to cure such deficiency or extend the timeline for achieving such benchmark once upon the payment of a fee.

We may terminate the OHRI License by providing ninety days written notice to OHRI. OHRI may terminate the OHRI License if we materially breach the license agreement and fail to cure the breach within a grace period, or if we become insolvent or bankrupt. The OHRI License otherwise expires upon the expiration of the last to expire of the licensed patents.

Manufacturing

We do not own or operate, and currently have no plans to establish, any of our own manufacturing facilities. Other than small amounts of compounds and proteins that we may synthesize ourselves for preclinical testing, we currently rely, and expect to continue to rely, on third party contract manufacturing organizations, or CMOs, for the manufacture of our required raw materials and proteins, including FT1050, the small molecule HSC modulator used in manufacturing ProHema.

ProHema Manufacturing

ProHema (formally referred to as ProHema-CB Suspension for Infusion), is a composition of pharmacologically-modulated human cord blood cells. ProHema is produced by treating qualified human umbilical cord units with FT1050 in a multistep process that is performed on the day of transplantation in relative close proximity to the recipient, such that it may be administered within minutes to one or two hours after release. The cord blood units, or CBUs, therefore never leave the vicinity of the clinical center, eliminating the risk that shipment to a distant offsite manufacturing facility may result in delivery delays.

ProHema is manufactured on the same day as product administration, corresponding to Day 0 of the transplant regimen. A cryopreserved CBU that meets clinical protocol criteria for the manufacturing process is used as the starting cellular source material. These CBUs are identified through online search facilities that are able to identify potentially suitable CBUs from cord blood banks around the world, based upon a patient s HLA type and cell dose requirements.

The manufacturing process consists of treating the physician-selected CBU with FT1050 in our proprietary two-hour modulation process. After the cells are modulated, an automated wash is performed to reduce residual FT1050 prior to administration of ProHema. After in-lab filtration and final packaging and labeling, the final product consists of *ex vivo* modulated human cord blood cells. ProHema is then tested in a variety of ways prior to release.

ProHema is manufactured at clinical cell processing facilities operated by or affiliated with our clinical sites. Although some of these facilities may be certified GMP cell manufacturing environments, the ProHema manufacturing process consists largely of closed production, which we believe minimizes the requirement for full GMP environmental monitoring and control. One objective of our product development program is to close

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the ProHema manufacturing process to the point that it may be conducted by the majority of clinical cell processing facilities that are otherwise capable of handling standard HSC products for allogeneic HSCT.

In addition to FT1050, we use other components in the manufacturing of ProHema, including components used in our NRM formulation, as well as disposable materials such as bags and tubing sets. To date, we have obtained the FT1050 starting material for ProHema in our preclinical studies and clinical trials from one third-party manufacturer. We obtain our supply of FT1050 for our clinical trials from this manufacturer on a purchase order basis under a clinical supply manufacturing agreement, and do not have any current contractual relationships for the commercial manufacture and supply of bulk FT1050 substance for manufacturing ProHema. If our current third-party manufacturer of FT1050 should become unavailable to us for any reason, we believe that there are several potential replacements, although we may incur some delay in identifying and qualifying such replacements. We intend to source other components used in the manufacturing of ProHema, including those that comprise our NRM formulation, from other third-party suppliers.

Wnt7a Protein Manufacturing

Our Wnt7a analogs are recombinant proteins generated from a stably-transfected mammalian cell expression system. Our initial supply of Wnt7a analogs used in our preclinical efficacy and pharmacokinetic studies was synthesized within our laboratories by our scientists. Other than small amounts of proteins and compounds that we may synthesize ourselves for preclinical testing, we expect to rely on third parties for the manufacture of the Wnt7a analog any other Wnt-based product candidates that we may develop. We are currently selecting the contract manufacture organization for master cell banking, process development and ultimate cGMP manufacture of our Wnt7a analog therapeutic.

Competition

The biotechnology and pharmaceutical industries are characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products. While we believe that our technology, development experience and scientific knowledge provide us with competitive advantages, we face potential competition from many different sources, including major pharmaceutical, specialty pharmaceutical and biotechnology companies, academic institutions and governmental agencies and public and private research institutions. Any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future.

Many of our competitors will have substantially greater financial, technical and human resources. Accordingly, our competitors may be more successful in developing or marketing products and technologies that are more effective, safer or less costly. Additionally, our competitors may obtain regulatory approval for their products more rapidly and may achieve more widespread market acceptance.

There are several clinical-stage development programs that seek to improve human UCBT through the use of *ex vivo* expansion technologies to increase the quantity of HSCs for use in HSCT or the use of *ex vivo* differentiation technologies to increase the quantity of hematopoietic progenitor cells for use in HSCT. Companies active in this area include, but are not limited to, Gamida Cell Ltd., Biotest Pharmaceuticals Corporation, Aldagen, Inc., a wholly-owned subsidiary of Cytomedix, Inc., Novartis Pharmaceuticals Corporation and Celerant Technology Corp.

Currently, there are no approved pharmaceutical products specifically developed for the treatment of muscular dystrophies. We are aware of several other companies developing therapies that are in various stages of development for the treatment of muscular dystrophies, including Prosensa Holding B.V., Sarepta Therapeutics Inc., PTC Therapeutics, Inc., Summit Corporation plc, Halo Therapeutics LLC, and Tivorsan Pharmaceuticals, Inc.

Government Regulation

In the United States, the FDA regulates biological products under the Federal Food, Drug, and Cosmetic Act, or FDCA, and the Public Health Service Act, or PHS Act, and related regulations. Biological products are also subject to other federal, state, local, and foreign statutes and regulations. The FDA and comparable

regulatory agencies in state and local jurisdictions and in foreign countries impose substantial requirements upon the clinical development, manufacture and marketing of biological products. These agencies and other federal, state, local, and foreign entities regulate research and development activities and the testing, manufacture, quality control, safety, effectiveness, packaging, labeling, storage, distribution, record keeping, reporting, approval, advertising and promotion of our products. Failure to comply with the applicable U.S. regulatory requirements at any time during the product development process, including clinical testing, approval process or after approval may subject an applicant to administrative or judicial sanctions.

Government regulation may delay or prevent marketing of product candidates for a considerable period of time and impose costly procedures upon our activities. The testing and approval process requires substantial time, effort, and financial resources, and we cannot be certain that the FDA or any other regulatory agency will grant approvals for ProHema or any future product candidates on a timely basis, if at all. The FDA s policies may change and additional government regulations may be enacted that could prevent or delay regulatory approval of ProHema or any future product candidates or approval of new disease indications or label changes. We cannot predict the likelihood, nature or extent of adverse governmental regulation that might arise from future legislative, judicial, or administrative action, either in the United States or abroad.

Marketing Approval

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

- completion of nonclinical laboratory and animal tests according to good laboratory practices, or GLPs, and applicable requirements for the humane use of laboratory animals or other applicable regulations;
- submission to the FDA of an IND application which must become effective before human clinical trials may begin;
- performance of adequate and well-controlled human clinical trials according to the FDA s regulations commonly referred to as good clinical practices, or GCPs, and any additional requirements for the protection of human research subjects and their health information, to establish the safety and efficacy of the proposed biological product for its intended use or uses;
- submission to the FDA of a Biologics License Application, or BLA, for marketing approval that includes substantive evidence of safety, purity, and potency from results of nonclinical testing and clinical trials;
- satisfactory completion of an FDA pre-approval inspection of manufacturing facilities where the biological product is produced to assess compliance with good manufacturing practices, or GMPs, to assure that the facilities, methods and controls are adequate to preserve the biological product s identity, strength, quality and purity and, if applicable, the FDA s current good tissue practices, or GTPs, for the use of human cellular and tissue products to prevent the introduction, transmission or spread of communicable diseases;
- potential FDA audit of the nonclinical study sites and clinical trial sites that generated the data in support of the BLA; and
- FDA review and approval, or licensure, of the BLA which must occur before a biological product can be marketed or sold.

U.S. Biological Products Development Process

Before testing any biological product candidate in humans, the product candidate enters the nonclinical testing stage. Nonclinical tests include laboratory evaluations of product chemistry, toxicity and formulation, as well as animal studies to assess the potential safety and activity of the product candidate. The conduct of the nonclinical tests must comply with federal regulations and requirements including GLPs.

Prior to commencing the first clinical trial, the clinical trial sponsor must submit the results of the nonclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and a

proposed clinical protocol, to the FDA as part of an initial IND application. Some nonclinical testing may continue even after the IND application is submitted. The IND automatically becomes effective 30 days after receipt by the FDA unless the FDA, within the 30-day time period, raises concerns or questions about the conduct of the clinical trial and places the clinical trial on a clinical hold. In such case, the IND sponsor must resolve any outstanding concerns with the FDA before the clinical trial may begin. Further, an independent institutional review board, or IRB, for each site proposing to conduct the clinical trial must review and approve the plan for any clinical trial before it commences at that site. An IRB is charged with protecting the welfare and rights of study subjects and considers such items as whether the risks to individuals participating in the clinical trials are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the form and content of the informed consent that must be signed by each clinical trial subject or his or her legal representative and must monitor the clinical trial until completed. The FDA or IRB may impose clinical holds on a biological product candidate at any time before or during clinical trials due to safety concerns or non-compliance. If the FDA imposes a clinical hold, trials may not recommence without FDA or IRB authorization and then only under terms authorized by the FDA and IRB. Accordingly, we cannot be sure that submission of an IND application will result in the FDA allowing clinical trials to begin, or that, once begun, issues will not arise that will result in the suspension or termination of such trials.

Clinical trials involve the administration of the biological product candidate to healthy volunteers or patients under the supervision of qualified investigators, generally physicians not employed by or under the trial sponsor s control. Clinical trials are conducted under protocols detailing, among other things, the objectives of the clinical trial, dosing procedures, subject selection and exclusion criteria, and the parameters to be used to monitor subject safety, including stopping rules that assure a clinical trial will be stopped if certain adverse events should occur. Each protocol and any amendments to the protocol must be submitted to the FDA as part of the IND and to the IRB.

For purposes of BLA approval, human clinical trials are typically conducted in three sequential phases that may overlap:

Phase 1 The biological product is initially introduced into healthy human subjects and tested for safety. In the case of some products for severe or life-threatening diseases, especially when the product may be too inherently toxic to ethically administer to healthy volunteers, the initial human testing is often conducted in patients. These trials may also provide early evidence on effectiveness.

Phase 2 These trials are conducted in a limited number of patients in the target population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance and optimal dosage. Multiple Phase 2 clinical trials may be conducted by the sponsor to obtain information prior to beginning larger and more expensive Phase 3 clinical trials.

Phase 3 Phase 3 trials are undertaken to provide statistically significant evidence of clinical efficacy and to further evaluate dosage, potency, and safety in an expanded patient population at multiple clinical trial sites. They are performed after preliminary evidence suggesting effectiveness of the product has been obtained, and are intended to establish the overall benefit-risk relationship of the investigational product, and to provide an adequate basis for product approval and labeling.

Post-approval clinical trials, sometimes referred to as Phase 4 clinical trials, may be conducted after initial marketing approval. These trials may be required by the FDA as a condition of approval and are used to gain additional experience from the treatment of patients in the intended therapeutic indication, particularly for long-term safety follow-up. The FDA now has express statutory authority to require post-market clinical trials to address safety issues. All of these trials must be conducted in accordance with GCP requirements in order for the data to be considered reliable for regulatory purposes.

During all phases of clinical development, regulatory agencies require extensive monitoring and auditing of all clinical activities, clinical data, and clinical trial investigators. Annual progress reports detailing the results of the clinical trials must be submitted to the FDA. Written IND safety reports must be promptly submitted to the FDA and the investigators for serious and unexpected adverse events; any findings from other studies, tests in laboratory animals or in vitro testing that suggest a significant risk for human subjects; or any clinically important increase in

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the rate of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. The sponsor must submit an IND safety report within 15 calendar days after the sponsor determines that the information qualifies for reporting. The sponsor also must notify the FDA of any unexpected fatal or life-threatening suspected adverse reaction within seven calendar days after the sponsor s initial receipt of the information.

Phase 1, Phase 2, and Phase 3 clinical trials may not be completed successfully within any specified period, if at all. Regulatory authorities, a data safety monitoring board or the sponsor may suspend a clinical trial at any time on various grounds, including a finding that the participants are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB s requirements or if the biological product has been associated with unexpected serious harm to patients.

Our ongoing and planned clinical trials for our product candidates may not begin or be completed on schedule, if at all. Clinical trials can be delayed for a variety of reasons, including delays in:

- obtaining regulatory approval to commence a trial;
- reaching agreement with third-party clinical trial sites and their subsequent performance in conducting accurate and reliable trials on a timely basis;
- obtaining IRB approval to conduct a trial at a prospective site;
- recruiting patients to participate in a trial; and
- supply of the biological product.

Typically, if a biological product is intended to treat a chronic disease, as is the case with ProHema, safety and efficacy data must be gathered over an extended period of time, which can range from six months to three years or more. Success in early stage clinical trials does not ensure success in later stage clinical trials. Data obtained from clinical activities are not always conclusive and may be susceptible to varying interpretations, which could delay, limit or prevent regulatory approval.

Concurrent with clinical trials, companies usually complete additional animal studies and must also develop additional information about the physical characteristics of the biological product as well as finalize a process for manufacturing the product in commercial quantities in accordance with GMP requirements. To help reduce the risk of the introduction of adventitious agents with the use of biological products, the PHS Act emphasizes the importance of manufacturing control for products whose attributes cannot be precisely defined. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, the sponsor must develop methods for testing the identity, strength, quality, potency, and purity of the final biological product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the biological product candidate does not undergo unacceptable deterioration over its shelf life.

U.S. Review and Approval Processes

In order to obtain approval to market a biological product in the United States, a BLA must be submitted to the FDA that provides data establishing to the FDA s satisfaction the safety, purity and potency of the investigational biological product for the proposed indication. The application includes all data available from nonclinical studies and clinical trials, including negative or ambiguous results as well as positive findings, together with detailed information relating to the product s manufacture and composition, and proposed labeling, among other things. The testing and approval processes require substantial time and effort and there can be no assurance that the FDA will accept the BLA for filing and, even if filed, that any approval will be granted on a timely basis, if at all.

Under the Prescription Drug User Fee Act, or PDUFA, as amended, each BLA must be accompanied by a user fee. The FDA adjusts the PDUFA user fees on an annual basis. According to the FDA s fee schedule, effective beginning on October 1, 2013 and in effect through September 30,

2014, the user fee for an application requiring clinical data, such as a BLA, will be $2,169,100 for fiscal year 2014. PDUFA also imposes an annual

product fee for biologics ($104,060 for fiscal year 2014), and an annual establishment fee ($554,600 for fiscal year 2014) on facilities used to manufacture prescription biologics. Fee waivers or reductions are available in certain circumstances, including a waiver of the application fee for the first application filed by a small business. Additionally, no user fees are assessed on BLAs for products designated as orphan drugs, unless the product also includes a non-orphan indication.

The FDA has 60 days from its receipt of a BLA to determine whether the application will be accepted for filing based on the agency s threshold determination that the application is sufficiently complete to permit substantive review. The FDA may refuse to file any BLA that it deems incomplete or not properly reviewable at the time of submission and may request additional information. In this event, the BLA must be resubmitted with the additional information. The resubmitted application also is subject to review before the FDA accepts it for filing. After the BLA submission is accepted for filing, the FDA reviews the BLA to determine, among other things, whether the proposed product is safe and potent, or effective, for its intended use, and has an acceptable purity profile, and whether the product is being manufactured in accordance with GMPs to assure and preserve the product s identity, safety, strength, quality, potency, and purity, and biological product standards. The FDA may refer applications for novel biological products or biological products that present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and, if so, under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

Before approving a BLA, the FDA will inspect the facilities at which the product is manufactured. The FDA will not approve the product unless it determines that the manufacturing processes and facilities are in compliance with GMP requirements and adequate to assure consistent production of the product within required specifications. For a human cellular or tissue product, the FDA also will not approve the product if the manufacturer is not in compliance with the GTPs. These are FDA regulations that govern the methods used in, and the facilities and controls used for, the manufacture of human cells, tissues, and cellular and tissue based products, or HCT/Ps, which are human cells or tissue intended for implantation, transplant, infusion, or transfer into a human recipient. The primary intent of the GTP requirements is to ensure that cell and tissue based products are manufactured in a manner designed to prevent the introduction, transmission and spread of communicable disease. FDA regulations also require tissue establishments to register and list their HCT/Ps with the FDA and, when applicable, to evaluate donors through screening and testing. Additionally, before approving a BLA, the FDA may inspect one or more clinical sites to assure that the clinical trials were conducted in compliance with IND study requirements and GCPs. To assure GMP, GTP and GCP compliance, an applicant must incur significant expenditure of time, money and effort. If the FDA determines the manufacturing process or manufacturing facilities are not acceptable, it typically will outline the deficiencies and often will require the facility to take corrective action and provide documentation evidencing the implementation of such corrective action. This may significantly delay further review of the application. If the FDA finds that a clinical site did not conduct the clinical trial in accordance with GCPs, the FDA may determine the data generated by the clinical site should be excluded from the primary efficacy analyses provided in the BLA, and request additional testing or data. Additionally, notwithstanding the submission of any requested additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

The FDA also has authority to require a Risk Evaluation and Mitigation Strategy, or REMS, from manufacturers to ensure that the benefits of a biological product outweigh its risks. A sponsor may also voluntarily propose a REMS as part of the BLA submission. The need for a REMS is determined as part of the review of the BLA. Based on statutory standards, elements of a REMS may include dear doctor letters, a medication guide, more elaborate targeted educational programs, and in some cases restrictions on distribution. These elements are negotiated as part of the BLA approval, and in some cases may delay the approval date. Once adopted, REMS are subject to periodic assessment and modification.

After the FDA completes its initial review of a BLA, it will communicate to the sponsor that the biological product will either be approved, or it will issue a complete response letter to communicate that the BLA will not be approved in its current form. The complete response letter usually describes all of the specific deficiencies in the BLA identified by the FDA. The deficiencies identified may be minor, for example, requiring labeling

changes, or major, for example, requiring additional clinical trials. Additionally, the complete response letter may include recommended actions that the applicant might take to place the applicant in a condition for approval. If a complete response letter is issued, the applicant may either resubmit the BLA, addressing all of the deficiencies identified in the letter, or withdraw the application.

The FDA may not grant approval on a timely basis, or at all. We may encounter difficulties or unanticipated costs in our efforts to secure necessary governmental approvals, which could delay or preclude us from marketing our products. The testing and approval process for a biological product usually takes several years to complete.

One of the performance goals agreed to by the FDA under PDUFA is to review 90% of standard BLAs in 10 months and 90% of priority BLAs in six months, whereupon a review decision is to be made. The FDA does not always meet its PDUFA goal dates for standard and priority BLAs and its review goals are subject to change from time to time. The review process and the PDUFA goal data may be extended by three months if the FDA requests or the BLA applicant otherwise provides additional information or clarification regarding information already provided in the submission within the last three months before the PDUFA goal date.

Even if a product candidate receives regulatory approval, the approval may be limited to specific disease states, patient populations and dosages, or the indications for use may otherwise be limited, which could restrict the commercial value of the product. Further, the FDA may require that certain contraindications, warnings, or precautions be included in the product labeling. The FDA may impose restrictions and conditions on product distribution, prescribing, or dispensing in the form of a risk management plan, or otherwise limit the scope of any approval. In addition, the FDA may require Phase 4 post-marketing clinical trials, designed to further assess a biological product s safety and effectiveness, and testing and surveillance programs to monitor the safety of approved products that have been commercialized. Further, even after regulatory approval is obtained, later discovery of previously unknown problems with a product may result in the imposition of new restrictions on the product or even complete withdrawal of the product from the market. Delay in obtaining, or failure to obtain and maintain, regulatory approval for ProHema, or obtaining approval but for significantly limited use, would harm our business.

Expedited Development and Review Programs

The FDA has a Fast Track program that is intended to facilitate the development and expedite the review of new drugs and biological products that meet certain criteria. Specifically, new drugs and biological products are eligible for Fast Track designation if they are intended to treat a serious or life-threatening condition or disease and demonstrate the potential to address unmet medical needs for the condition. Fast Track designation applies to the combination of the product and the specific indication for which it is being studied. The sponsor of a new drug or biological may request the FDA to designate the drug or biologic as a Fast Track product at any time during the clinical development of the product. Unique to a Fast Track product, the FDA may consider for review sections of the marketing application on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the application, the FDA agrees to accept sections of the application and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the application.

Any product submitted to the FDA for marketing, including under a Fast Track program, may be eligible for other types of FDA programs intended to expedite development and review, such as priority review and accelerated approval. Any product is eligible for priority review if it has the potential to provide safe and effective therapy where no satisfactory alternative therapy exists or a significant improvement in the treatment, diagnosis or prevention of a disease compared to marketed products. The FDA will attempt to direct additional resources to the evaluation of an application for a new drug or biological product designated for priority review in an effort to facilitate the review. Additionally, a product may be eligible for accelerated approval. Drug or biological products studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit over existing treatments may receive accelerated approval, which means that they may be approved on the basis of adequate and well-controlled clinical trials establishing that the product has an effect on a surrogate endpoint that is reasonably likely to predict a clinical benefit, or on the basis of an effect 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. As a condition of approval, the FDA may require that a sponsor of a drug or biological product receiving accelerated approval perform adequate and well-controlled post-marketing clinical trials. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product. Fast Track designation, priority review and accelerated approval do not change the standards for approval but may expedite the development or approval process.

The Food and Drug Administration Safety and Innovation Act of 2012 also amended the FDCA to require FDA to expedite the development and review of a breakthrough therapy. A drug or biological product can be designated as a breakthrough therapy if it is intended to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that it may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. A sponsor may request that a drug or biological product be designated as a breakthrough therapy at any time during the clinical development of the product. If so designated, FDA shall act to expedite the development and review of the product s marketing application, including by meeting with the sponsor throughout the product s development, providing timely advice to the sponsor to ensure that the development program to gather nonclinical and clinical data is as efficient as practicable, involving senior managers and experienced review staff in a cross-disciplinary review, assigning a cross-disciplinary project lead for the FDA review team to facilitate an efficient review of the development program and to serve as a scientific liaison between the review team and the sponsor, and taking steps to ensure that the design of the clinical trials is as efficient as practicable.

U.S. Patent Term Restoration and Marketing Exclusivity

Depending upon the timing, duration, and specifics of the FDA approval of the use of our product candidates, some of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, commonly referred to as the Hatch-Waxman Amendments. Patent term restoration can compensate for time lost during product development and the regulatory review process by returning up to five years of patent life for a patent that covers a new product or its use. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the product s approval date. The period of patent term restoration is generally one-half the time between the effective date of an IND (falling after issuance of the patent) and the submission date of a BLA, plus the time between the submission date of the BLA and the approval of that application, provided the sponsor acted with diligence. Only one patent applicable to an approved biological product is eligible for the extension and the application for the extension must be submitted prior to the expiration of the patent. The application for patent term extension is subject to approval by the U.S. Patent and Trademark Office, or USPTO, in consultation with the FDA.

A patent term extension is only available when the FDA approves a biological product for the first time. We believe ProHema and the manner in which it modulates HSCs have not been previously approved by the FDA. However, we cannot be certain that the USPTO and the FDA will agree with our analysis or the USPTO will grant a patent term extension.

A biological product can obtain pediatric market exclusivity in the United States. Pediatric exclusivity, if granted, adds six months to existing exclusivity periods and patent terms. This six-month exclusivity, which runs from the end of other exclusivity protection or patent term, may be granted based on the voluntary completion of a pediatric study in accordance with an FDA-issued Written Request for such a study.

An abbreviated approval pathway for biological products shown to be similar to, or interchangeable with, an FDA-licensed reference biological product was created by the Biologics Price Competition and Innovation Act of 2009, which was part of the Patient Protection and Affordable Care Act, or PPACA, signed into law on March 23, 2010. This amendment to the PHS Act attempts to minimize duplicative testing. Biosimilarity, which requires that there be no clinically meaningful differences between the biological product and the reference product in terms of safety, purity, and potency, can be shown through analytical studies, animal studies, and a clinical trial or trials. Interchangeability requires that a biological product is biosimilar to the reference biological product and the product must demonstrate that it can be expected to produce the same clinical results as the reference product and, for products administered multiple times, the product and the reference product may be

switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biological product. However, complexities associated with the larger, and often more complex, structure of biological products, as well as the process by which such products are manufactured, pose significant hurdles to implementation that are still being worked out by the FDA.

A reference biological product is granted twelve years of exclusivity from the time of first licensure of the reference product. On April 10, 2013, President Obama released his proposed budget for fiscal year 2014 and proposed to cut this twelve year period of exclusivity down to seven years. He also proposed to prohibit additional periods of exclusivity for brand biological products due to minor changes in product formulation, a practice often referred to as evergreening. The first biological product submitted under the abbreviated approval pathway that is determined to be interchangeable with the reference product has exclusivity against other biologics submitting under the abbreviated approval pathway for the lesser of (i) one year after the first commercial marketing, (ii) 18 months after approval if there is no legal challenge, (iii) 18 months after the resolution in the applicant s favor of a lawsuit challenging the biologic s patents if an application has been submitted, or (iv) 42 months after the application has been approved if a lawsuit is ongoing within the 42-month period.

FDA Post-Approval Requirements

Maintaining substantial compliance with applicable federal, state, local, and foreign statutes and regulations requires the expenditure of substantial time and financial resources. Rigorous and extensive FDA regulation of biological products continues after approval, particularly with respect to GMP. We will rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of any products that we may commercialize. Manufacturers of our products are required to comply with applicable requirements in the GMP regulations, including quality control and quality assurance and maintenance of records and documentation. We cannot be certain that we or our present or future suppliers will be able to comply with the GMP and other FDA regulatory requirements. Other post-approval requirements applicable to biological products include reporting of GMP deviations that may affect the identity, potency, purity and overall safety of a distributed product, record-keeping requirements, reporting of adverse effects, reporting updated safety and efficacy information, and complying with electronic record and signature requirements. After a BLA is approved, the product also may be subject to official lot release. As part of the manufacturing process, the manufacturer is required to perform certain tests on each lot of the product before it is released for distribution. If the product is subject to official release by the FDA, the manufacturer submits samples of each lot of product to the FDA together with a release protocol showing a summary of the history of manufacture of the lot and the results of all of the manufacturer s tests performed on the lot. The FDA also may perform certain confirmatory tests on lots of some products, such as viral vaccines, before releasing the lots for distribution by the manufacturer. In addition, the FDA conducts laboratory research related to the regulatory standards on the safety, purity, potency, and effectiveness of biological products.

Discovery of previously unknown problems or the failure to comply with the applicable regulatory requirements, by us or our suppliers, may result in restrictions on the marketing of a product or withdrawal of the product from the market as well as possible civil or criminal sanctions and adverse publicity. FDA sanctions could include refusal to approve pending applications, suspension or revocation of an approval, clinical hold, warning or untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, mandated corrective advertising or communications with doctors, debarment, restitution, disgorgement of profits, or civil or criminal penalties. Any agency or judicial enforcement action could have a material adverse effect on us.

Biological product manufacturers and other entities involved in the manufacture and distribution of approved biological products are required to register their facilities with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with GMPs and other laws. In addition, changes to the manufacturing process or facility generally require prior FDA approval before being implemented and other types of changes to the approved product, such as adding new indications and additional labeling claims, are also subject to further FDA review and approval.

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Labeling, Marketing and Promotion

The FDA closely regulates the labeling, marketing and promotion of biological products, including direct-to-consumer advertising, promotional activities involving the internet, and industry-sponsored scientific and educational activities. While doctors are free to prescribe any product approved by the FDA for any use, a company can only make claims relating to safety and efficacy of a biological product that are consistent with FDA approval, and the company is allowed to market a biological product only for the particular use and treatment approved by the FDA. In addition, any claims we make for our products in advertising or promotion must be appropriately balanced with important safety and risk information and otherwise be adequately substantiated. Failure to comply with these requirements can result in adverse publicity, warning letters, corrective advertising, injunctions, potential civil and criminal penalties and exclusion from government healthcare programs.

Orphan Designation

We have been granted orphan designation in the United States for human allogeneic HSCs *ex vivo* modulated with FT1050 for the enhancement of stem cell engraftment and in the European Union for ProHema for the treatment of acute myelogenous leukemia through the *ex vivo* modulation of allogeneic umbilical cord blood cells. Under the Orphan Drug Act, the FDA may grant orphan designation to biological products 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 biological product in the United States for this type of disease or condition will be recovered from sales of the product. Orphan designation must be requested before submitting a BLA. After the FDA grants orphan designation, the identity of the applicant, as well as the name of the therapeutic agent and its designated orphan use, are disclosed publicly by the FDA. Orphan designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

If a biological product that receives orphan designation is the first such product approved by FDA for the orphan indication, it receives orphan product exclusivity, which for seven years prohibits the FDA from approving another application to market the same product for the same indication. Orphan product exclusivity will not bar approval of another product under certain circumstances, including if the new product is shown to be clinically superior to the approved product on the basis of greater efficacy or safety, or if the company with the orphan product exclusivity is unable to meet market demand. More than one product may also be approved by the FDA for the same orphan indication or disease as long as the products are different. As a result, even in any indication for which any of our products has been granted orphan designation, the FDA can still approve different products for use in treating the same indication or disease covered by our product, which could create a more competitive market for us. Additionally, competitors may obtain approval for the same product but for a different indication for which the orphan product has exclusivity. Orphan product exclusivity also could block the approval of one of our products for seven years if a competitor obtains approval of the same biological product as defined by the FDA first or if our product candidate is determined to be contained within the competitor s product for the same indication or disease. If a biological product designated as an orphan product receives marketing approval for an indication broader than what is designated, it may not be entitled to orphan product exclusivity. Orphan drug status in the European Union has similar, but not identical, benefits.

Pediatric Research Equity Act

Under the Pediatric Research Equity Act, or PREA, a BLA or BLA supplement must contain data to assess the safety and effectiveness of the biological product for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The intent of PREA is to compel sponsors whose products have pediatric applicability to study those products in pediatric populations, rather than ignoring pediatric indications for adult indications that could be more economically desirable. The FDA may grant deferrals for submission of data or full or partial waivers. By its terms, PREA does not apply to any biological product for an indication for which orphan designation has been granted, unless the FDA issues regulations saying otherwise. Because the FDA has not issued any such regulations, submission of a pediatric assessment is not required for an application to market a product for an orphan-designated indication.

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Anti-Kickback and False Claims Laws

In the United States, the research, manufacturing, distribution, sale and promotion of biological products are potentially subject to regulation by various federal, state and local authorities in addition to the FDA, including the Centers for Medicare & Medicaid Services, other divisions of the U.S. Department of Health and Human Services (e.g., the Office of Inspector General), the U.S. Department of Justice, state Attorneys General, and other federal, state and local government agencies. For example, sales, marketing and scientific/educational grant programs must comply with the Anti-Kickback Statute, as amended, the federal False Claims Act, as amended, (the False Claims Act) the privacy regulations promulgated under the Health Insurance Portability and Accountability Act, or HIPAA, and similar state laws. Pricing and rebate programs must comply with the Medicaid Drug Rebate Program requirements of the Omnibus Budget Reconciliation Act of 1990, as amended, and the Veterans Health Care Act of 1992, as amended. If products are made available to authorized users of the Federal Supply Schedule of the General Services Administration, additional laws and requirements apply. All of these activities are also potentially subject to federal and state consumer protection and unfair competition laws.

As noted above, in the United States, we are subject to complex laws and regulations pertaining to healthcare fraud and abuse, including, but not limited to, the Anti-Kickback Statute, the False Claims Act, and other state and federal laws and regulations. The Anti-Kickback Statute makes it illegal for any person, including a biological product manufacturer (or a party acting on its behalf) to knowingly and willfully solicit, receive, offer, or pay any remuneration that is intended to induce the referral of business, including the purchase or order of an item for which payment may be made under a federal healthcare program, such as Medicare or Medicaid. Violations of this law are punishable by up to five years in prison, criminal fines, administrative civil money penalties, and exclusion from participation in federal healthcare programs. In addition, many states have adopted laws similar to the Anti-Kickback Statute. Some of these state prohibitions apply to the referral of patients for healthcare services reimbursed by any insurer, not just federal healthcare programs such as Medicare and Medicaid. Due to the breadth of these federal and state anti-kickback laws and the potential for additional legal or regulatory change in this area, it is possible that our future sales and marketing practices or our future relationships with physicians might be challenged under anti-kickback laws, which could harm us. Because we intend to commercialize products that could be reimbursed under a federal healthcare program and other governmental healthcare programs, we plan to develop a comprehensive compliance program that establishes internal controls to facilitate adherence to the rules and program requirements to which we will or may become subject.

The False Claims Act prohibits anyone from, among other things, knowingly presenting, or causing to be presented, for payment to federal programs (including Medicare and Medicaid) claims for items or services, including biological products, that are false or fraudulent. Although we likely would not submit claims directly to payers, manufacturers can be held liable under these laws if they are deemed to cause the submission of false or fraudulent claims by, for example, providing inaccurate billing or coding information to customers or promoting a product off-label. In addition, our future activities relating to the reporting of wholesaler or estimated retail prices for our products, the reporting of prices used to calculate Medicaid rebate information and other information affecting federal, state, and third-party coverage and reimbursement for our products, and the sale and marketing of our products, are subject to scrutiny under this law. For example, pharmaceutical companies have been prosecuted under the False Claims Act in connection with their off-label promotion of drugs. Penalties for a False Claims Act violation include three times the actual damages sustained by the government, plus mandatory civil penalties of between $5,500 and $11,000 for each separate false claim, the potential for exclusion from participation in federal healthcare programs, and, although the federal False Claims Act is a civil statute, conduct that results in a False Claims Act violation may also implicate various federal criminal statutes. If the government were to allege that we were, or convict us of, violating these false claims laws, we could be subject to a substantial fine and may suffer a decline in our stock price. In addition, private individuals have the ability to bring actions under the False Claims Act and certain states have enacted laws modeled after the False Claims Act.

There are also an increasing number of state laws that require manufacturers to make reports to states on pricing and marketing information. Many of these laws contain ambiguities as to what is required to comply with the laws. In addition, as discussed below, beginning in August 2013, a similar federal requirement will require manufacturers to track and report to the federal government certain payments made to physicians and teaching

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hospitals made in the previous calendar year. These laws may affect our sales, marketing, and other promotional activities by imposing administrative and compliance burdens on us. 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 soon federal, authorities.

Other Regulations

We are also subject to numerous federal, state and local laws relating to such matters as safe working conditions, manufacturing practices, environmental protection, fire hazard control, and disposal of hazardous or potentially hazardous substances. We may incur significant costs to comply with such laws and regulations now or in the future.

Facilities

We occupy approximately 23,684 square feet of office and laboratory space in San Diego, California under a lease that expires in 2016. We believe that our facilities are adequate for our current needs.

Employees

As of June 30, 2013, we employed 33 full-time employees, including 17 in research and development, ten in clinical development and six in general and administrative. We have never had a work stoppage, and none of our employees is represented by a labor organization or under any collective bargaining arrangements. We consider our employee relations to be good.

Legal Proceedings

We are not a party to any material legal proceedings at this time. From time to time, we may be subject to various legal proceedings and claims that arise in the ordinary course of our business activities. Although the results of litigation and claims cannot be predicted with certainty, as of the date of this prospectus, we do not believe we are party to any claim or litigation the outcome of which, if determined adversely to us, would individually or in the aggregate be reasonably expected to have a material adverse effect on our business. Regardless of the outcome, litigation can have an adverse impact on us because of defense and settlement costs, diversion of management resources and other factors.

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MANAGEMENT

Executive Officers and Directors

The following table sets forth information regarding our executive officers and directors, including their ages as of August 15, 2013:

Name	Age	Position
Executive Officers:		
Christian Weyer, M.D., M.A.S.	44	President, Chief Executive Officer and Director
J. Scott Wolchko	43	Chief Financial Officer and Chief Operating Officer
Pratik S. Multani, M.D., M.S.	46	Chief Medical Officer
Daniel D. Shoemaker, Ph.D.	45	Chief Technology Officer
Peter Flynn, Ph.D.	39	Senior Vice President, Early Program Development
Non-Management Directors:		
William H. Rastetter, Ph.D.[(1)(3)].	65	Chairman of the Board
John D. Mendlein, Ph.D., J.D.	53	Vice Chairman of the Board
Timothy P. Coughlin[(1)(2)]	46	Director
Mark J. Enyedy[(1)(2)]	49	Director
Amir Nashat, Sc.D.[(3)]	40	Director
Robert T. Nelsen[(2)]	50	Director

(1) Member of the audit committee.

(2) Member of the compensation committee.

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

Christian Weyer, M.D., M.A.S. has served as our President and Chief Executive Officer and a director since October 2012. Dr. Weyer joined us after a 12-year tenure with Amylin Pharmaceuticals, Inc., a biopharmaceutical company, where he most recently served as Senior Vice President of Research and Development until the completion of Amylin s acquisition by Bristol-Myers Squibb in August 2012. During his tenure with Amylin, Dr. Weyer also served as Vice President of Medical Development and Vice President of Corporate Development. Prior to joining Amylin, he spent three years, from 1997 to 2000, with the National Institutes of Health, NIDDK, in Phoenix, Arizona, where he conducted clinical research on the pathogenesis of obesity and type 2 diabetes. Dr. Weyer holds an M.D. from the University of Düsseldorf, Germany, and a postdoctoral master s degree in clinical research from the University of California, San Diego. We believe Dr. Weyer s extensive leadership, executive, managerial, business and pharmaceutical company experience qualifies him to serve as a member of our board of directors. In addition, Dr. Weyer s day-to-day management and intimate knowledge of our business and operations provide our board with an in-depth understanding of the Company.

J. Scott Wolchko has served as our Chief Financial Officer since the commencement of our operations in September 2007 and as our Chief Operating Officer since February 2013. Mr. Wolchko began his career in 1994 as an investment banker with Morgan Stanley & Co., serving in the firm s New York City and Menlo Park, California offices. As a member of the firm s Investment Banking Health Care Group, he assisted emerging growth companies in the life sciences sector complete capital-raising and M&A transactions. Prior to joining us, from July 2001 to September 2007, Mr. Wolchko served as the Chief Financial Officer of Bocada, Inc., an enterprise software company that specializes in data protection management. Mr. Wolchko holds an M.S. in biochemical engineering from the University of Virginia, and a B.S. in biomedical engineering from the University of Vermont.

Pratik S. Multani, M.D., M.S. has served as our Chief Medical Officer since May 2013 and was previously our Senior Vice President of Clinical Development from May 2011 to May 2013, and Vice President of Clinical Development from April 2009 to May 2011. Prior to that, Dr. Multani was Vice President of Clinical Development

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at Kalypsys, Inc., a pharmaceutical company, from August 2007 to March 2009, where he advanced the development of multiple compounds in the therapeutic areas of pain and inflammation and metabolic diseases. From 2005 to 2007, he served as Senior Vice President of Clinical Development and then Chief Medical Officer at Kanisa Pharmaceuticals, an oncology-focused pharmaceutical company. From 1999 to 2004, advancing from Associate Director of Oncology and Hematology to Senior Director of Medical Research at Biogen-Idec. Dr. Multani holds an M.S. in epidemiology from Harvard School of Public Health, an M.D. from Harvard Medical School and a B.S. in chemistry and biology from Yale University. He completed his Internal Medicine residency at the Massachusetts General Hospital followed by a medical oncology fellowship at the Dana Farber/Partners joint program, after which he was a member of the transplant unit at Massachusetts General Hospital.

Daniel D. Shoemaker, Ph.D. has served as our Chief Technology Officer since February 2009 and leads our drug discovery efforts. From 2003 to 2009, Dr. Shoemaker was previously Chief Scientific Officer of ICxBiosystems, a biotechnology firm that develops advanced detection technologies for use in biodefense, cancer and prenatal diagnostics. From 2003 to 2005, he was Chief Scientific Officer of GHC Technologies, a biotechnology company. From 1998 to 2003, Dr. Shoemaker held several positions at Merck Research Laboratories, including Director of Target Discovery, Senior Director at Rosetta Inpharmatics and research fellow in the Department of Molecular Neurosciences, where his main focus was on target identification and biomarker discovery. Dr. Shoemaker received his Ph.D. in biochemistry from Stanford University and his B.S. in biochemistry from the University of California, Santa Barbara.

Peter Flynn, Ph.D. has served as our Senior Vice President, Early Program Development since February 2013 and was previously our Vice President of Biologic Therapeutics and iPSC Technology from May 2011 to February 2013. From May 2009 to May 2011, he served as our Senior Director of Protein Discovery. Prior to joining us, from January 2007 to May 2009, he was Vice President of Research for Ren Pharmaceuticals, a renal and cario-renal therapeutics company. Prior to Ren, from March 2001 to January 2007, Dr. Flynn was Director of Biochemistry Research at KaloBios Pharmaceuticals, an antibody therapeutics company. Prior to the formation of KaloBios, Dr. Flynn was a researcher at UCSF Comprehensive Cancer Center. He holds a Ph.D. from the ICRF London (Cancer Research UK) and a B.Sc. in molecular biology from University College London.

William H. Rastetter, Ph.D. has served as Chairman of the Board and a director since November 2011. From February 2012 to October 2012, he also served as our interim Chief Executive Officer. He is a Co-Founder of Receptos, Inc., a biopharmaceutical company, where he has been a director and Chairman of the Board since May 2009 and was Acting Chief Executive Officer from May 2009 to November 2010. Dr. Rastetter served as a Partner at the venture capital firm of Venrock from 2006 to February 2013. Prior to that, Dr. Rastetter was Executive Chairman of Biogen Idec, from the merger of the two companies (Biogen and Idec Pharmaceuticals) in 2003 through the end of 2005. He joined Idec Pharmaceuticals at its founding in 1986 and served as Chairman and Chief Executive Officer. Prior to Idec, he was Director of Corporate Ventures at Genentech, Inc. and also served in a scientific capacity at Genentech. Dr. Rastetter also serves as the Chairman of Illumina, Inc. and Neurocrine Biosciences, Inc. and as a director of Regulus Therapeutics, Inc. Dr. Rastetter held various faculty positions at the Massachusetts Institute of Technology and Harvard University and was an Alfred P. Sloan Fellow. Dr. Rastetter holds a Ph.D. and M.A. in chemistry from Harvard University and an S.B. in chemistry from the Massachusetts Institute of Technology. We believe Dr. Rastetter is qualified to serve on our board of directors due to his extensive experience in the biotechnology industry, his broad leadership experience with Idec Pharmaceuticals and on several boards of pharmaceutical companies, and his experience with financial matters.

John D. Mendlein, Ph.D., J.D. has served as our Vice Chairman of the Board since November 2011 and a director since April 2008. He also previously served as our Chief Executive Officer, as well as the founding Chairman of the Board and Chief Science Officer. Dr. Mendlein also serves as Executive Chairman and Chief Executive Officer of aTyr Pharma, Inc., a biopharmaceutical company, a position he has held since September 2011. He also holds board positions with Moderna Therapeutics and BIO (Biotechnology Industry Organization) including its emerging companies board. Dr. Mendlein previously served as the Chief Executive Officer of Adnexus Therapeutics, a biopharmaceutical company, from 2005 to 2008, which was purchased by Bristol-Myers Squibb (BMY) in 2008. Before that, he served as Chairman and Chief Executive Officer of Affinium Pharmaceuticals, Inc. from 2000 to 2005, and board member, General Counsel and Chief Knowledge Officer at

Aurora Bioscience Corporation from August 1996 to September 2001. Dr. Mendlein holds a Ph.D. in physiology and biophysics from the University of California, Los Angeles, a J.D. from the University of California, Hastings College of the Law, and a B.S. in biology from the University of Miami. We believe that Dr. Mendlein s extensive business and leadership experience in the biotechnology industry qualifies him to serve as a member of our board of directors.

Timothy P. Coughlin has been a director since August 2013. He has served as Vice President and Chief Financial Officer of Neurocrine Biosciences, Inc., a biopharmaceutical company, since September 2006, where he previously served as Vice President, Controller. Prior to joining Neurocrine in 2002, he was with Catholic Health Initiatives, a nationwide integrated healthcare delivery system, where he served as Vice President, Financial Services. Mr. Coughlin also served as a Senior Manager in the Health Sciences practice of Ernst & Young LLP and its predecessors from 1989 to 1999. Mr. Coughlin holds a Master s degree in international business from San Diego State University and a B.B.A. in accounting from Temple University. Mr. Coughlin is a certified public accountant in both California and Pennsylvania. We believe Mr. Coughlin is qualified to serve on our board of directors due to his extensive background in financial and accounting matters for public companies and his leadership experience in the biotechnology industry, including his position at Neurocrine.

Mark J. Enyedy has served as a director since July 2012. Mr. Enyedy is Senior Vice President and Head of the Internal Medicine business unit for Shire plc, a biopharmaceutical company, a position he has held since August 2013. Prior to Shire, from September 2011 to July 2013, Mr. Enyedy served as Chief Executive Officer and director of Proteostasis Therapeutics, Inc., a biopharmaceutical company. Prior to Proteostasis, he served 15 years with Genzyme Corporation, a biotechnology company, most recently as President of the Transplant, Oncology and Multiple Sclerosis divisions. Before joining Genzyme, Mr. Enyedy was an associate in the business law department at Palmer & Dodge. Mr. Enyedy holds a J.D. from Harvard Law School and a B.S. in criminal justice from Northeastern University. We believe that Mr. Enyedy s extensive strategic, operational and business experience with life sciences companies qualifies him to serve as a member of our board of directors.

Amir Nashat, Sc.D. has served as a director since September 2007. He is also a Managing General Partner at Polaris Venture Partners. He joined Polaris in April 2002 and focuses on investments in healthcare, consumer products and energy. Dr. Nashat currently represents Polaris as a director of Receptos, Inc., as well as several private companies. Additionally, Dr. Nashat has served as a director of Adnexus Therapeutics (acquired by Bristol Myers Squibb) and other private companies. Dr. Nashat holds a Sc.D. in chemical engineering from the Massachusetts Institute of Technology with a minor in biology, and an M.S. and B.S. in materials science and mechanical engineering from the University of California, Berkeley. We believe that Dr. Nashat is qualified to serve on our board of directors due his extensive experience within the field of drug discovery and development, his broad leadership experience on various boards, and his financial expertise with life sciences companies.

Robert T. Nelsen has served as a member of our board of directors since September 2007. Mr. Nelsen was a co-founder of ARCH Venture Partners, a venture capital firm, and has served in various capacities for ARCH and affiliated entities since July 1986. He is currently a managing director of ARCH Venture Corporation. Mr. Nelsen is a director of Agios, Inc., Ikaria, Inc., Kythera Biopharmaceuticals, Inc., Sapphire Energy, Inc., Ensemble Therapeutics Corporation, NeurogesX, Inc., Syros Pharmaceuticals Inc., among others, and serves as chairman of the board of Hua Medicine. Mr. Nelsen also serves as a Trustee of the Fred Hutchinson Cancer Research Institute, the Institute for Systems Biology, and is a director of the National Venture Capital Association. Mr. Nelsen previously served on the boards of Illumina, Inc., Caliper Life Sciences, Inc., Adolor Corporation, Receptos, Inc., and entities affiliated with deCode Genetics, Inc., among others. Mr. Nelsen holds an M.B.A. from the University of Chicago and a B.S. with majors in biology and economics from the University of Puget Sound. We believe Mr. Nelsen is qualified to sit on our board of directors due to his extensive experience as an investor in, and director of, early stage biopharmaceutical and life sciences companies.

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Our Scientific Founders

Our leadership position in the pharmacologic modulation of adult stem cells has its foundation in our longstanding relationship with our scientific founders, who are renowned pioneers in the field of developmental and stem cell biology:

Philip Beachy, Ph.D., Ernest and Amelia Gallo Professor at Stanford University School of Medicine, Department of Biochemistry, Institute for Stem Cell Biology and Regenerative Medicine, and Investigator of the Howard Hughes Medical Institute, or HHMI, studies the normal functions of secreted protein signals of the Hedgehog and Wnt pathways and the pathological roles of such signaling pathways in developmental disorders and cancer growth.

Sheng Ding, Ph.D., Senior Investigator, Gladstone Institute of Cardiovascular Disease, works in the field of developing and applying innovative chemical approaches to stem cell biology and regeneration, with a focus on discovering and characterizing novel small molecules that can control various cell fate and function, including stem cell maintenance, activation, differentiation and reprogramming in various developmental stages and tissues.

Rudolf Jaenisch, M.D., Founding Member of the Whitehead Institute, Professor of Biology at the Massachusetts Institute of Technology, and Member of the National Academy of the Sciences, is recognized as the first scientist to generate a transgenic mouse and one of the first scientists to reprogram fully mature adult cells and generate iPSCs and successfully demonstrate the application of iPSC technology to disease correction in rodent systems.

Randall Moon, Ph.D., William and Marilyn Connor Chair and Founding Director of the Institute for Stem Cell and Regenerative Medicine at University of Washington and HHMI Investigator, studies the Wnt signal transduction pathways with an emphasis on their normal roles in vertebrates, their mechanisms of action, their linkage to various disease processes, and the development of therapeutics targeting these pathways.

Michael Rudnicki, Ph.D., Director of the Regenerative Medicine Program and the Sprott Centre for Stem Cell Research at the Ottawa Hospital Research Institute and International HHMI Investigator, has made numerous discoveries in the understanding of tissue regeneration, including the pivotal role of Wnt7a in stimulating muscle stem cell growth and Pax7 as a transcription factor required for the specification of satellite cells.

David Scadden, M.D., Gerald and Darlene Jordan Professor at Harvard Medical School, Co-director of Harvard Stem Cell Institute, and Director of Massachusetts General Hospital Center for Regenerative Medicine, is a practicing hematologist and oncologist and is focused on translating stem cell science to improve the lives of people with chronic disease.

Leonard Zon, M.D., Grousbeck Professor of Pediatric Medicine at Harvard Medical School, Director of the Stem Cell Program at Children s Hospital Boston, and HHMI Investigator, is internationally recognized for his research in the emerging fields of stem cell biology and cancer genetics.

Composition of Our Board of Directors

Our board of directors currently consists of seven members, all of whom were elected pursuant to the board composition provisions of a voting agreement, which will terminate immediately prior to the completion of this offering. Upon the termination of these provisions, there will be no further contractual obligations regarding the election of our directors. Our nominating and corporate governance committee and board of directors may therefore consider a broad range of factors relating to the qualifications and background of director nominees, which may include diversity and is not limited to race, gender or national origin. We have no formal policy regarding board diversity. Our nominating and corporate governance committee s and board of directors priority in selecting board members is identification of persons who will further the interests of our stockholders through his or her established record of professional accomplishment, the ability to contribute positively to the collaborative culture among board members, knowledge of our business, understanding of the competitive landscape and professional and personal experiences and expertise relevant to our business strategy. Our directors hold office until their successors have been elected and qualified or until the earlier of their resignation or removal.

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Director Independence. Our board of directors has determined that all of our directors, except for Dr. Mendlein and Dr. Weyer, are independent, as determined in accordance with the rules of The NASDAQ Stock Market and the SEC. In making such independence determination, the board of directors considered the relationships that each non-employee director has with us and all other facts and circumstances that the board of directors deemed relevant in determining their independence, including the beneficial ownership of our capital stock by each non-employee director, and, where applicable, the transactions involving them described below under Certain Relationships and Related Party Transactions . In considering the independence of the directors listed above, our board of directors considered the association of our directors with the holders of more than 5% of our common stock. Upon the completion of this offering, we expect that the composition and functioning of our board of directors and each of our committees will comply with all applicable requirements of The NASDAQ Stock Market and the rules and regulations of the SEC. There are no family relationships among any of our directors or executive officers.

Staggered Board. In accordance with the terms of our amended and restated certificate of incorporation and amended and restated bylaws that will become effective upon the completion of this offering, our board of directors will be divided into three classes, Class I, Class II and Class III, with each class serving staggered three-year terms. Upon the expiration of the term of a class of directors, directors in that class will be eligible to be elected for a new three-year term at the annual meeting of stockholders in the year in which their term expires.

Our Class I directors will be Dr. Mendlein and Mr. Nelsen;

Our Class II directors will be Mr. Enyedy, Dr. Nashat and Dr. Rastetter; and

Our Class III directors will be Mr. Coughlin and Dr. Weyer.

Our amended and restated certificate of incorporation and amended and restated bylaws that will become effective upon the completion of this offering provide that the authorized number of directors may be changed only by resolution of the board of directors. Any additional directorships resulting from an increase in the number of directors will be distributed among the three classes.

The division of our board of directors into three classes with staggered three-year terms may delay or prevent stockholder efforts to effect a change of our management or a change in control.

Leadership Structure of the Board

The positions of our chairman of the board and chief executive officer are presently separated at Fate. Separating these provisions allows our chief executive officer to focus on our day-to-day business, while allowing the chairman of the board to lead our board of directors in its fundamental role of providing advice to and independent oversight of management. Our board of directors recognizes the time, effort and energy that the chief executive officer must devote to his position in the current business environment, as well as the commitment required to serve as our chairman of the board, particularly as our board of directors oversight responsibilities continue to grow. Our board of directors also believes that this structure ensures a greater role for the independent directors in the oversight of our company and active participation of the independent directors in setting agendas and establishing priorities and procedures for the work of our board of directors. Although our amended and restated bylaws that will be in effect upon the completion of this offering will not require our chairman of the board and chief executive officer positions to be separate, our board of directors believes that having separate positions is the appropriate leadership structure for us at this time and demonstrates our commitment to good corporate governance.

Board s Role in Risk Oversight

Risk is inherent with every business, and how well a business manages risk can ultimately determine its success. We face a number of risks, including risks relating to our financial condition, development and commercialization activities, operations and intellectual property as more fully discussed under Risk Factors in this prospectus. Management is responsible for the day-to-day management of risks we face, while our board of directors, as a whole and through its committees, has responsibility for the oversight of risk management. In its risk oversight role, our board of directors has the responsibility to satisfy itself that the risk management processes designed and implemented by management are adequate and functioning as designed.

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The role of our board of directors in overseeing the management of our risks is conducted primarily through committees of the board of directors, as disclosed in the descriptions of each of the committees below and in the charters of each of the committees. The full board of directors (or the appropriate board committee in the case of risks that are under the purview of a particular committee) discusses with management our major risk exposures, their potential impact on our company, and the steps we take to manage them. When a board committee is responsible for evaluating and overseeing the management of a particular risk or risks, the chairman of the relevant committee reports on the discussion to the full board of directors during the committee reports portion of the next board meeting. This enables our board of directors and its committees to coordinate the risk oversight role, particularly with respect to risk interrelationships.

Board Committees

Our board of directors has established an audit committee, a compensation committee and a nominating and governance committee. Each committee will operate under a charter approved by our board. Following this offering, copies of each committee s charter will be posted on the Corporate Governance section of our website, at www.fatetherapeutics.com.

Audit Committee

Mr. Coughlin, Mr. Enyedy and Dr. Rastetter currently serve on the audit committee, which is chaired by Mr. Coughlin. Our board of directors has determined that each member of the audit committee is an independent director under the NASDAQ Marketplace Rules and Rule 10A-3 of the Exchange Act. Our board of directors has designated Mr. Coughlin as an audit committee financial expert, as defined under the applicable rules of the SEC. The audit committee s responsibilities include:

- appointing, approving the compensation of, and assessing the independence of our independent registered public accounting firm;
- approving auditing and permissible non-audit services, and the terms of such services, to be provided by our independent registered public accounting firm;
- reviewing the internal audit plan with the independent registered public accounting firm and members of management responsible for preparing our financial statements;
- reviewing and discussing with management and the independent registered public accounting firm our annual and quarterly financial statements and related disclosures as well as critical accounting policies and practices used by us;
- reviewing the adequacy of our internal control over financial reporting;
- establishing policies and procedures for the receipt and retention of accounting-related complaints and concerns;
- recommending, based upon the audit committee s review and discussions with management and the independent registered public accounting firm, whether our audited financial statements shall be included in our Annual Report on Form 10-K;
- monitoring the integrity of our financial statements and our compliance with legal and regulatory requirements as they relate to our financial statements and accounting matters;
- preparing the audit committee report required by SEC rules to be included in our annual proxy statement;

reviewing all related party transactions for potential conflict of interest situations and approving all such transactions; and

reviewing quarterly earnings releases.

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Compensation Committee

Mr. Coughlin , Mr. Enyedy and Mr. Nelsen currently serve on the compensation committee, which is chaired by Mr. Enyedy. Our board of directors has determined that each member of the compensation committee is independent as that term is defined in the applicable NASDAQ Stock Market and SEC rules. The compensation committee s responsibilities include:

- annually reviewing and recommending to the board for approval corporate goals and objectives relevant to the compensation of our Chief Executive Officer;
- evaluating the performance of our Chief Executive Officer in light of such corporate goals and objectives and determining the compensation of our Chief Executive Officer;
- reviewing and approving the compensation of our other executive officers;
- reviewing and establishing our overall management compensation, philosophy and policy;
- overseeing and administering our compensation and similar plans;
- evaluating and assessing potential and current compensation advisors in accordance with the independence standards identified in the applicable NASDAQ Stock Market and SEC rules;
- retaining and approving the compensation of any compensation advisors;
- reviewing and approving our policies and procedures for the grant of equity-based awards;
- reviewing and making recommendations to the board of directors with respect to director compensation;
- preparing the compensation committee report required by SEC rules to be included in our annual proxy statement; and
- reviewing and discussing with management the compensation discussion and analysis to be included in our annual proxy statement or Annual Report on Form 10-K.

Nominating and Corporate Governance Committee

Dr. Nashat and Dr. Rastetter currently serve on the nominating and corporate governance committee, which is chaired by Dr. Rastetter. Our board of directors has determined that each member of the nominating and corporate governance committee is independent as that term is defined in the applicable NASDAQ Stock Market rules. The nominating and corporate governance committee s responsibilities include:

- developing and recommending to the board of directors criteria for board and committee membership;

establishing procedures for identifying and evaluating board of director candidates, including nominees recommended by stockholders;

identifying individuals qualified to become members of the board of directors;

recommending to the board of directors the persons to be nominated for election as directors and to each of the board s committees;

developing and recommending to the board of directors a set of corporate governance guidelines; and

overseeing the evaluation of the board of directors and management.

Our board of directors may establish other committees from time to time.

Compensation Committee Interlocks and Insider Participation

None of the members of our compensation committee has at any time during the prior three years been one of our officers or employees. None of our executive officers currently serves, or in the past fiscal year has served, as a member of the board of directors or compensation committee of any entity that has one or more executive officers serving on our board of directors or compensation committee.

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Corporate Governance

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 persons performing similar functions. Following the completion of this offering, a current copy of the code will be posted on the Corporate Governance section of our website, which is located at www.fatetherapeutics.com. If we make any substantive amendments to, or grant any waivers from, the code of business conduct and ethics for any officer or director, we will disclose the nature of such amendment or waiver on our website or in a current report on Form 8-K.

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EXECUTIVE AND DIRECTOR COMPENSATION

Summary Compensation Table

The following table presents information regarding the total compensation earned by each individual who served as our chief executive officer at any time during the fiscal year ended December 31, 2012 and our two other most highly compensated executive officers who were serving as executive officers as of December 31, 2012. We refer to these officers as our named executive officers.

Name and Principal Position	Year	Salary ($)	Option Awards ($)[1]	Total ($)
Christian Weyer, M.D., M.A.S. [2]				
President and Chief Executive Officer	2012	$ 74,038	$ 677,486	$ 751,524
William H. Rastetter, Ph.D.				
Former Interim President and Chief Executive Officer [3]	2012			
J. Scott Wolchko				
Chief Financial Officer and Chief Operating Officer	2012	$ 241,000	$ 96,092	$ 337,092
Pratik S. Multani, M.D., M.S.				
Chief Medical Officer	2012	$ 313,000	$ 102,334	$ 415,334

(1) In accordance with SEC rules, this column reflects the aggregate grant date fair value of the option awards granted during 2012 computed in accordance with Financial Accounting Standard Board ASC Topic 718 for stock-based compensation transactions, or ASC 718. Assumptions used in the calculation of these amounts are included in Note 5 to our consolidated financial statements included elsewhere in 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 options, the exercise of the stock options or the sale of the common stock underlying such stock options.

(2) Dr. Weyer joined our company in October 2012. Amount shown represents the compensation earned by Dr. Weyer during 2012 from and after his October 8, 2012 start date.

(3) Dr. Rastetter served as our interim President and Chief Executive Officer from February 2012 to October 2012. He did not receive any cash compensation for his service in this capacity. Dr. Rastetter purchased 118,360 shares of restricted common stock issued under our 2007 Equity Incentive Plan, or our 2007 Plan, pursuant to his March 16, 2012 restricted stock purchase agreement. Such shares were purchased for $1.63 per share, which reflects the fair value of the common stock as of such date, for a total aggregate purchase price of $192,336.50.

Employment Arrangements with Our Named Executive Officers

Each of our named executive officers is party to a written employment agreement with us and is employed at-will, except that we did not enter into a written contract with Dr. Rastetter in connection with his service as our interim president and chief executive officer.

Christian Weyer, M.D., M.A.S.

Dr. Weyer entered into an at-will employment agreement with us on October 2, 2012 and commenced employment with us on October 8, 2012. His initial annual base salary is $350,000, subject to periodic review and adjustments at the discretion of the board of directors or the compensation committee. Beginning with the calendar year 2013, Dr. Weyer will be considered annually for a bonus target of up to 50% of his then-current base salary, as determined by the board of directors or the compensation committee. Any bonus awarded to Dr. Weyer for calendar year 2013 will also take into account his employment for the portion of calendar year 2012 during which he was employed with us. In connection with the commencement of his employment, we granted the following stock options to Dr. Weyer under our 2007 Plan:

an option to purchase 218,097 shares of common stock (which we refer to as the performance-based grant) of which (i) 25% of the shares underlying such option (which we refer to as the transaction-based

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shares) vests over two years in equal monthly installments commencing on the earlier of (x) the date one month after achievement of the specified transaction milestone (as defined in the agreement) and (y) the date one month after the closing of a change of control (as defined in the agreement); and (ii) the remaining shares underlying such option are subject to performance-based vesting, where 25% of the shares underlying the option will vest based upon the per share common stock price received upon (x) the completion of this offering, or (y) a change of control (which we refer to as an exit value) of at least $19.50, an additional 25% of the shares will vest upon the achievement of an exit value of at least $32.50 and an additional 25% of the shares will vest upon the achievement of an exit value of at least $45.50;

an option to purchase 293,040 shares of common stock (which we refer to as the standard time-based grant), of which 25% of the shares underlying such option vests on October 8, 2013, and the remaining 75% vests in equal monthly installments thereafter through October 8, 2016, subject to Dr. Weyer s continued service to our company through each such vesting date; and

an option to purchase 143,154 shares of common stock (which we refer to as the early exercise time-based grant and together with the standard time-based grant, the time-based grants), which is subject to the same vesting schedule as the standard time-based grant and was subject to early exercise upon grant.

Payments Provided upon Termination for Good Reason or Without Cause

Dr. Weyer s employment is at will. In the event of termination for good reason or without cause, Dr. Weyer will be entitled to receive (i) the amount of his accrued but unpaid salary, earned but unpaid bonus, and any accrued but unused vacation as of the date of termination, (ii) reimbursement of any expenses properly incurred on behalf of the Company prior to any such termination and not yet reimbursed, (iii) continuation of his base salary for a period of twelve months after the effective date of termination, provided that such payments will be reduced dollar-for-dollar by any amounts received from employment or self-employment during the severance period if such termination follows a change in control, and (iv) continuation of group health plan benefits, with the cost of such benefits shared in the same relative proportion by the Company and Dr. Weyer until the earlier of (x) twelve months after termination and (y) the date Dr. Weyer becomes eligible for benefits through another employer or otherwise ineligible for COBRA, in the case of each of (iii) and (iv), subject to the execution and non-revocation of a release agreement, resignation from any and all positions and return of all Company property.

In addition, in the event Dr. Weyer is terminated without cause or for good reason following a change in control, (i) all of the then-unvested shares subject to the time-based grants shall immediately vest, and (ii) all of the then-unvested transaction-based shares shall immediately vest.

Payments Provided upon a Change of Control

In the event of a change of control, 50% of the then-unvested shares subject to Dr. Weyer s time-based grants shall vest immediately prior to such change in control. In addition, any portion of Dr. Weyer s unvested time-based grants or performance-based grant that is (i) unvested but eligible for continued or accelerated vesting and (ii) not assumed or substituted on substantially the same terms by the acquirer in connection with such change in control, will be converted into the right to receive the consideration payable to holders of common stock of the Company in connection with such change of control. Upon the closing of a change of control, Dr. Weyer s performance-based grant will terminate with respect to the number of performance-based option shares for which each applicable exit value is not achievable.

Under Dr. Weyer s employment agreement, the terms below are generally defined as follows:

cause means: (i) embezzlement, misappropriation of material assets or property of the Company; (ii) the conviction of, or plea of guilty or no contest to a felony or a crime involving moral turpitude, theft or securities laws violations; (iii) ongoing and repeated failure to perform the lawful duties and responsibilities of the position after receiving notice; or (iv) the employee s uncured breach of the employment agreement or related agreements with the Company;

change of control means (i) the liquidation, dissolution or winding up of the Company; (ii) the acquisition of the Company by means of any transaction or series of related transactions in which the Company s stockholders immediately prior to such transaction hold less than fifty percent (50%) of the voting power of the surviving or acquiring entity; or (iii) the sale, conveyance or other disposal of all or substantially all of the property or business of the Company; provided that a change of control will not include (x) a merger or consolidation with a wholly-owned subsidiary of the Company, (y) a merger effected exclusively for the purpose of changing the domicile of the Company or (z) any transaction or series of related transactions principally for bona fide equity financing purposes in which the Company is the surviving corporation; and

good reason means that the employee has complied with the appropriate notice procedures following the occurrence of any of the following: (i) the material diminution in the employee s responsibilities, authority and function; (ii) a material reduction in the employee s base salary that is not pursuant to a salary reduction program affecting substantially all senior level employees; or (iii) a change in the employee s workplace location of more than fifty (50) miles.

J. Scott Wolchko

Mr. Wolchko entered into an at-will employment agreement and commenced employment with us on September 17, 2007. The employment agreement was amended on November 11, 2008. His initial annual base salary was $160,000, subject to periodic review and adjustments based upon achievement of performance goals as determined by the board of directors. Pursuant to the terms of his employment agreement, Mr. Wolchko was issued 25,641 shares of restricted common stock on September 17, 2007. All of the shares subject to such restricted stock issuance were fully vested as of September 17, 2011.

Payments Provided upon a Change of Control

In the event that within twelve months of a change of control, Mr. Wolchko is terminated involuntarily without cause or for good reason, Mr. Wolchko shall be entitled to receive a cash severance payment equal to six months of his then-current salary and shall be reimbursed for six months of COBRA benefits, subject to the execution and non-revocation of a release agreement.

Under Mr. Wolchko s employment agreement, the terms below are generally defined as follows:

cause means (i) the occurrence of any of the following, as determined by the Board: (i) conviction of any felony or any crime involving fraud, dishonesty or moral turpitude under the laws of the United States or any state thereof; (ii) attempted fraud against the Company; (iii) material violation of any contract or agreement between the employee and the Company or any statutory duty owed to the Company; or (iv) repeated or habitual drug or alcohol use that materially and adversely interferes with the performance of the employee s services to the Company;

change of control means (i) the consummation of a merger or consolidation of the Company with any other corporation which results in the voting securities of the Company outstanding immediately prior thereto failing to represent more than fifty percent (50%) of the total voting power represented by the voting securities of the surviving entity after such transaction or (ii) the sale or disposition by the Company of all or substantially all of the Company s assets; and

good reason means that the employee has complied with the appropriate notice process following the occurrence of any of the following events: (i) a reduction by fifteen (15) or greater percent of the employee s then-current base salary (unless the base salary of all senior management is similarly reduced); (ii) a material reduction in the employee s kind or level of employee benefits (unless the benefits of all senior management are similarly reduced); or (iii) the employee s relocation to a facility or a location more than fifty (50) miles from the Company s headquarters.

Pratik S. Multani, M.D., M.S.

Dr. Multani entered into an at-will employment agreement with us on March 23, 2009 and commenced employment with us on April 20, 2009. His initial annual base salary was $285,000. Dr. Multani will be

considered annually for a bonus target of up to 30% of his then-current base salary, subject to achievement of reasonably attainable performance goals and milestones as agreed between Dr. Multani and our chief executive officer. No bonus was paid to Dr. Multani for the year ended December 31, 2012. In connection with the commencement of his employment, Dr. Multani was granted an option to purchase 25,961 shares of our common stock, 25% of which vested on April 20, 2010, and the remaining 75% of which fully vested as of April 20, 2013.

Payments Provided upon a Change of Control

In the event that within twelve months following a change of control (as defined in the 2007 Equity Incentive Plan), Dr. Multani is terminated involuntarily without cause or for good reason, Dr. Multani shall be entitled to receive a cash severance payment equal to six months of his then-current salary and reimbursement for six months of COBRA benefits, subject to the execution and non-revocation of a release agreement.

Under Dr. Multani s employment agreement, the terms below are generally defined as follows:

cause means the occurrence of any of the following, as determined by the Board: (i) conviction of any felony or any crime involving fraud, dishonesty or moral turpitude under the laws of the United States or any state thereof; (ii) attempted fraud against the Company; (iii) material violation of any contract or agreement between the employee and the Company or any statutory duty owed to the Company; or (iv) repeated or habitual drug or alcohol use that materially and adversely interferes with the performance of the employee s services to the Company;

change of control means (i) the liquidation, dissolution or winding up of the Company; (ii) the acquisition of the Company where the stockholders immediately prior to the transaction hold less than fifty percent (50%) of the voting power of the surviving or acquiring entity or (iii) the sale, conveyance or other disposal of substantially all the business or property of the Company; and

good reason means that the employee has complied with the appropriate notice process following the occurrence of any of the following events: (i) a reduction by fifteen (15) or greater percent of the employee s then-current base salary (unless the base salary of all senior management is similarly reduced); (ii) a material reduction by the Company or any successor thereof in the employee s kind or level of employee benefits (unless the benefits of all senior management are similarly reduced); or (iii) the employee s relocation to a facility or a location more than fifty (50) miles from the Company s headquarters.

Employee Confidentiality and Assignment Agreements

Each of our named executive officers has entered into a standard form agreement with respect to confidential information and assignment of inventions. Among other things, this agreement obligates each named executive officer to refrain from disclosing any of our proprietary information received during the course of employment and to assign to us any inventions conceived or developed during the course of employment.

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Outstanding Equity Awards at Fiscal Year-End

The following table summarizes, for each of the named executive officers, the number of outstanding equity awards held by each of our named executive officers as of December 31, 2012.

	Option Awards				Stock Awards	
Name	Number of Securities underlying Unexercised Options (#) Exercisable	Number of Securities underlying Unexercised Options (#) Unexercisable	Option Exercise Price ($)	Option Expiration Date	Number of Shares that Have Not Vested (#)	Market Value of Shares that Have Not Vested ($)[1]
Christian Weyer, M.D., M.A.S.		218,097[2]	$ 1.37	10/9/2022		
		293,040[3]	$ 1.37	10/9/2022		
	143,154[3][4]		$ 1.37	10/9/2022		
William H. Rastetter, Ph.D.					86,304[5]	$ 117,806
Pratik S. Multani, M.D., M.S.	25,961[4][6]		$ 0.52	5/11/2019		
	17,273[7]	26,364[7]	$ 1.63	2/8/2022		
		22,400[8]	$ 1.63	2/8/2022		
	2,114[9]	18,180[9]	$ 1.37	7/23/2022		
J. Scott Wolchko	13,685[7]	20,888[7]	$ 1.63	2/8/2022		
		26,400[8]	$ 1.63	2/8/2022		
	2,114[9]	18,180[9]	$ 1.37	7/23/2022		

(1) There was no public market for our common stock as of December 31, 2012. The fair value of our common stock as of December 31, 2012 was $1.37 per share.

(2) 25% of the shares underlying this option will vest monthly over two years commencing on the earlier of a change of control or transaction milestone, subject to acceleration if Dr. Weyer is terminated without cause or resigns for good reason at any time after such change of control or transaction milestone. The remaining shares underlying this option are subject to performance-based vesting, where 25% of the shares underlying the option will vest upon the achievement of an exit value (as further defined in the applicable stock option agreement) of at least $19.50, an additional 25% of the shares will vest upon the achievement of an exit value of at least $32.50 and an additional 25% of the shares will vest upon the achievement of an exit value of at least $45.50. For more information on vesting of this grant, see Employment Arrangements with Our Named Executive Officers above.

(3) 25% of the shares underlying this option will vest on October 8, 2013, with the remainder of the shares vesting in equal monthly installments over the following three years through October 8, 2016. The vesting of 50% of the then-unvested shares will accelerate upon a change of control, and the vesting of all remaining unvested shares will accelerate if Dr. Weyer is terminated without cause or resigns for good reason at any time after such change of control.

(4) This option was subject to early exercise upon grant.

(5) Under the terms of Dr. Rastetter s March 16, 2012 restricted common stock purchase agreement issued under our 2007 Plan, our right of repurchase with respect to the shares that have not yet vested will lapse in equal monthly installments through November 9, 2015, and will lapse in full upon a change of control. The shares listed are currently held by Dr. Rastetter s family trust.

(6) 25% of the shares underlying this option vested on April 20, 2010, with the remainder of the shares vesting in equal monthly installments over the following three years through April 20, 2013.

(7) 25% of the shares underlying this option vested on May 30, 2012, with the remainder of the shares vesting in equal monthly installments over the following three years through May 30, 2015. The vesting of 50% of the then-unvested shares will accelerate upon a change of control, and the vesting of all remaining unvested shares will accelerate if the option holder is terminated without cause or resigns for good reason at any time after such change of control.

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(8) The shares underlying this option will vest monthly over two years commencing on the earlier of a change of control or performance milestone, as further set forth in the option holder s applicable stock option agreement, subject to acceleration upon a change of control if such change of control transaction occurs after the achievement of the applicable performance milestone. In August 2013, our board of directors determined that the performance milestone in the applicable option agreements would be achieved upon the completion of this offering.

(9) The shares underlying this option vest in equal monthly installments over four years from July 3, 2012 through July 3, 2016. The vesting of 50% of the then-unvested shares will accelerate upon a change of control, and the vesting of all remaining unvested shares will accelerate if the option holder is terminated without cause or resigns for good reason at any time after such change of control.

On August 12, 2013, our board of directors granted options to purchase 24,615 shares of our common stock to each of Dr. Multani and Mr. Wolchko at an exercise price of $7.87 per share. 6,154 shares underlying each such option will vest monthly over four years from the completion of this offering, and the remaining 18,461 shares underlying each such option are divided into five equal parts, each of which will vest monthly over a two-year period following our achievement of a specified clinical, development or operational milestone.

Director Compensation

The following table presents the total compensation for each person other than our chief executive officer who served as a member of our board of directors during 2012. Other than as set forth in the table and described more fully below, we did not pay any compensation, reimburse any expense of, make any equity awards or non-equity awards to, or pay any other compensation to any of the non-employee members of our board of directors in 2012.

In 2012, we did not maintain any standard fee arrangements for the non-employee members of our board of directors for their service as directors.

Director	Option Award[1] ($)	All Other Compensation ($)	Total[2] ($)
William H. Rastetter, Ph.D.[3]			
John D. Mendlein, Ph.D., J.D.[4]	$ 38,775	$ 75,289	$ 114,064
Timothy P. Coughlin[5]			
Mark J. Enyedy[6]	$ 31,860		$ 31,860
Amir Nashat, Sc.D.			
Robert T. Nelsen			
Bryan E. Roberts, Ph.D.[7]			
Carl Weissman[8]			

(1) In accordance with SEC rules, this column reflects the aggregate grant date fair value of the option awards granted during 2012 computed in accordance with ASC 718. Assumptions used in the calculation of these amounts are included in Note 5 to our consolidated financial statements included elsewhere in 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 options, the exercise of the stock options or the sale of the common stock underlying such stock options.

(2) We did not provide any cash, equity or other compensation to directors Amir Nashat, Robert T. Nelsen, Bryan E. Roberts or Carl Weissman during 2012 other than reimbursement of out-of-pocket expenses incurred in connection with attendance at board meetings.

(3) Dr. Rastetter held 118,360 shares of restricted common stock as of December 31, 2012, which shares were subsequently transferred to his family trust.

(4) Dr. Mendlein held 140,107 shares of restricted common stock and an option to purchase 31,562 shares of common stock as of December 31, 2012. The amounts shown consist of base salary paid to Dr. Mendlein in

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connection with his previous employment agreement in the amount of $71,956 and fees paid in the amount of $3,333 in connection with his current consulting agreement not related to his service as a director. See Certain Relationships and Related Party Transactions for more information on these agreements.

(5) Mr. Coughlin joined our board of directors in August 2013.

(6) Mr. Enyedy held an option to purchase 30,769 shares of common stock as of December 31, 2012.

(7) Dr. Roberts resigned from our board of directors in August 2013.

(8) Mr. Weissman resigned from our board of directors in August 2013.

On February 6, 2013, our board of directors granted options to purchase 28,461 shares of our common stock to each of Dr. Rastetter and Dr. Mendlein at an exercise price of $1.37 per share. Such options will vest in full only upon the achievement of an exit value (as further defined in the applicable stock option agreement) of at least $26.00. On August 12, 2013, our board of directors granted Mr. Coughlin an option to purchase 23,076 shares of our common stock at an exercise price of $7.87 per share. 25% of the shares underlying this option will vest on August 12, 2014, with the remainder of the shares vesting in equal monthly installments over the following three years through August 12, 2017.

In August 2013, our board of directors adopted a non-employee director compensation policy, effective as of the effectiveness of this registration statement, that is designed to provide a total compensation package that enables us to attract and retain, on a long-term basis, high-caliber non-employee directors. Under this policy, all non-employee directors will be paid cash compensation as set forth below, prorated based on days of service during a calendar year.

Board of Directors	Annual Retainer
All non-employee members	$ 35,000
Additional retainer for Chairperson	$ 35,000
Audit Committee:	
Chairperson	$ 15,000
Non-Chairperson members	$ 7,500
Compensation Committee:	
Chairperson	$ 10,000
Non-Chairperson members	$ 5,000
Nominating and Corporate Governance Committee:	
Chairperson	$ 7,000
Non-Chairperson members	$ 5,000

In addition, under the policy, each new non-employee director who is initially appointed or elected to our board of directors after effectiveness of the policy shall receive an option grant to purchase up to 12,000 shares of common stock, which will vest in equal annual installments during the three years following the grant date, subject to the director s continued service on our board of directors. In addition, on the date of the annual meeting of stockholders, each continuing non-employee director will be eligible to receive an annual option grant to purchase up to 6,000 shares of common stock, which will vest in full upon the earlier of the first anniversary of the date of grant or the date of the following annual meeting of stockholders. All of the foregoing options will be granted at fair market value on the date of grant.

We have agreed to reimburse all reasonable out-of-pocket expenses incurred by non-employee directors in attending Board and committee meetings.

Compensation Risk Assessment

We believe that although a portion of the compensation provided to our executive officers and other employees is performance-based, our executive compensation program does not encourage excessive or unnecessary risk taking. This is primarily due to the fact that our compensation programs are designed to encourage our executive

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officers and other employees to remain focused on both short-term and long-term strategic goals, in particular in connection with our pay-for-performance compensation philosophy. As a result, we do not believe that our compensation programs are reasonably likely to have a material adverse effect on our company.

Equity Compensation Plans

2007 Equity Incentive Plan

Our 2007 Equity Incentive Plan was approved by our board of directors and our stockholders in September 2007 and was most recently amended in August 2013. We refer to our 2007 Equity Incentive Plan, as amended, as the 2007 Plan. We have reserved an aggregate of 2,423,072 shares of our common stock for the issuance of equity awards under the 2007 Plan. This number is subject to adjustment in the event of a stock split, stock dividend or other change in our capitalization. Effective upon the completion of this offering, our board of directors has determined not to grant any further awards under our 2007 Plan. The shares we issue under the 2007 Plan may be authorized but unissued shares or shares we reacquire. The shares of common stock underlying any equity awards that are forfeited, canceled, repurchased, expired or are otherwise terminated (other than by exercise) under the 2007 Plan are currently added back to the shares of common stock available for issuance under the 2007 Plan. Upon the completion of this offering, such shares will be added to the shares of common stock available for issuance under the 2013 Plan (as defined below).

The 2007 Plan permits us to make grants of incentive stock options to employees and grants of non-qualified stock options and restricted stock to employees, officers, directors and consultants. Our 2007 Plan is administered by our board of directors. Our board of directors has the authority to select the individuals to whom awards will be granted, to make any combination of awards to participants, to accelerate the exercisability or vesting of any award and to determine the specific terms and conditions of each award, subject to the provisions of the 2007 Plan.

The 2007 Plan permits the granting of (1) options to purchase common stock intended to qualify as incentive stock options under Section 422 of the Internal Revenue Code of 1986, as amended, or the Code, and (2) options that do not so qualify. The option exercise price of each option will be determined by our board of directors but may not be less than 100% of the fair market value of the common stock on the date of grant. The term of each option will be fixed by our board of directors and may not exceed ten years from the date of grant. All stock option awards that are granted pursuant to the 2007 Plan are covered by an option agreement.

The 2007 Plan permits the award of restricted shares of common stock to participants, subject to such terms, conditions and restrictions as our board of directors may determine. All restricted stock awards that are granted pursuant to the 2007 Plan are covered by a restricted stock purchase agreement.

The 2007 Plan provides that upon the occurrence of a change of control, as defined in the 2007 Plan, all outstanding stock options will terminate at the effective time or consummation of such change of control, unless the surviving entity agrees to assume such stock options or substitute similar stock awards for those outstanding under the 2007 Plan. If options under the 2007 Plan terminate, optionees will be provided an opportunity to exercise their vested options prior to the consummation of the change of control.

Our board of directors may amend, alter, suspend or terminate the 2007 Plan at any time, subject to stockholder approval where such approval is required by applicable law. Our board of directors may also amend, modify or terminate any outstanding award, provided that no amendment to an award may materially impair any of the rights of a participant under any awards previously granted without his or her written consent. No awards may be granted under the 2007 Plan after September 26, 2017.

2013 Stock Option and Incentive Plan

Our 2013 Stock Option and Incentive Plan was adopted by our board of directors and approved by our stockholders in August 2013 and will become effective immediately prior to the completion of this offering. We refer to the 2013 Stock Option and Incentive Plan as the 2013 Plan. The 2013 Plan will replace the 2007 Plan.

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The 2013 Plan allows our compensation committee to make equity-based incentive awards to our officers, employees, directors and other key persons (including consultants).

We have initially reserved 1,020,000 shares of our common stock for the issuance of awards under the 2013 Plan. This number is subject to adjustment in the event of a stock split, stock dividend or other change in our capitalization. The 2013 Plan provides that the number of shares reserved and available for issuance will automatically increase each January 1, beginning on January 1, 2014, by (i) 4% of the outstanding number of shares of our common stock on the immediately preceding December 31, or (ii) such lesser number as determined by our compensation committee.

The shares issuable pursuant to awards granted under the 2013 Plan will be authorized but unissued shares or shares that we reacquire. The shares of common stock underlying any awards from the 2013 Plan and the 2007 Plan that are forfeited, cancelled, held back upon exercise or settlement of an award to satisfy the exercise price or tax withholding, reacquired by us prior to vesting, satisfied without any issuance of common stock, expire or are otherwise terminated (other than by exercise) under the 2013 Plan and the 2007 Plan will be added back to the shares of common stock available for issuance under the 2013 Plan.

Under the 2013 Plan, stock options and stock appreciation rights with respect to no more than 1,020,000 shares may be granted to any one individual in any one calendar year. No more than 1,020,000 shares may be issued in the form of incentive stock options, such number to be cumulatively increased on January 1, 2014 and on each January 1 thereafter by the lesser of (i) the amount by which the number of shares of common stock reserved for issuance under the 2013 Plan was increased on January 1 of the applicable calendar year or (ii) 729,000 shares in any one calendar year period.

The 2013 Plan will be administered by the compensation committee of our board of directors. The compensation committee has full power to select, from among the individuals eligible for awards, the individuals to whom awards will be granted, to make any combination of awards to participants, and to determine the specific terms and conditions of each award, subject to the provisions of the 2013 Plan. Persons eligible to participate in the 2013 Plan will be those full or part-time officers, employees, non-employee directors and other key persons (including consultants) as selected from time to time by our compensation committee in its discretion.

The 2013 Plan permits the granting of both options to purchase common stock intended to qualify as incentive stock options under Section 422 of the Code and options that do not so qualify. The exercise price of each stock option will be determined by our compensation committee but may not be less than 100% of the fair market value of our common stock on the date of grant or, in the case of an incentive stock option granted to a 10% owner, less than 110% of the fair market value of our common stock on the date of grant. The term of each stock option will be fixed by the compensation committee and may not exceed ten years from the date of grant. The compensation committee will determine at what time or times each option may be exercised.

The compensation committee may award stock appreciation rights subject to such conditions and restrictions as it may determine. Stock appreciation rights entitle the recipient to shares of common stock, or cash, equal to the value of the appreciation in our stock price over the exercise price. The exercise price may not be less than 100% of the fair market value of the common stock on the date of grant.

The compensation committee may award restricted stock or restricted stock units to participants subject to such conditions and restrictions as it may determine. These conditions and restrictions may include the achievement of certain performance goals or continued employment with us through a specified vesting period. The compensation committee may also grant shares of common stock that are free from any restrictions under the 2013 Plan. Unrestricted stock may be granted to participants in recognition of past services or for other valid consideration and may be issued in lieu of cash compensation due to such participant.

The compensation committee may grant performance share awards to participants which entitle the recipient to receive share awards of common stock upon the achievement of certain performance goals and such other conditions as the compensation committee shall determine.

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The compensation committee may grant cash bonuses under the 2013 Plan to participants, subject to the achievement of certain performance goals.

The compensation committee may grant performance-based awards to participants in the form of restricted stock, restricted stock units, performance shares or cash-based awards upon the achievement of certain performance goals and such other conditions as the compensation committee shall determine. The compensation committee may grant such performance-based awards under the 2013 Plan that are intended to qualify as performance-based compensation under Section 162(m) of the Code. Those awards would only vest or become payable upon the attainment of performance goals that are established by our compensation committee and related to one or more performance criteria. The performance criteria that could be used with respect to any such awards include: total shareholder return, earnings before interest, taxes, depreciation and amortization, net income (loss) (either before or after interest, taxes, depreciation or amortization), changes in the market price of our common stock, economic value-added, funds from operations or similar measures, sales or revenue, development, clinical or regulatory milestones, acquisitions or strategic transactions, operating income (loss), cash flow (including, but not limited to, operating cash flow and free cash flow), return on capital, assets, equity, or investment, stockholder returns, return on sales, gross or net profit levels, productivity, expense, margins, operating efficiency, customer satisfaction, working capital, earnings (loss) per share of our common stock, sales or market shares and number of customers, any of which may be measured either in absolute terms or as compared to any incremental increase or as compared to results of a peer group. From and after the time that we become subject to Section 162(m) of the Code, the maximum award that is intended to qualify as performance-based compensation under Section 162(m) of the Code that may be made to any one employee during any one calendar year period is 1,020,000 shares with respect to a stock-based award and $2,000,000 with respect to a cash-based award.

The 2013 Plan provides that upon the effectiveness of a sale event, as defined in the 2013 Plan, in the event that all awards are not assumed or continued or substituted by the successor entity, all awards granted under the 2013 Plan shall terminate. In addition, in connection with a sale event, we may make or provide for a cash payment to participants holding options and stock appreciation rights equal to the difference between the per share cash consideration payable to stockholders in the sale event and the exercise price of the options or stock appreciation rights.

Our board of directors may amend or discontinue the 2013 Plan and our compensation committee may amend or cancel outstanding awards for purposes of satisfying changes in law or any other lawful purpose, but no such action may adversely affect rights under an award without the holder s consent. Certain amendments to the 2013 Plan require the approval of our stockholders.

No awards may be granted under the 2013 Plan after the date that is ten years from the date of stockholder approval of the 2013 Plan. No awards under the 2013 Plan have been made prior to the date of this prospectus.

2013 Employee Stock Purchase Plan

Our 2013 Employee Stock Purchase Plan, or ESPP, was adopted by our board of directors and approved by our stockholders in September 2013 and will become effective immediately prior to the completion of this offering. The ESPP will be administered by the compensation committee of our board of directors. The ESPP authorizes the initial issuance of up to a total of 729,000 shares of our common stock to participating employees, such number to be cumulatively increased on January 1, 2015 and on each January 1 thereafter by the lesser of (i) 2% of the outstanding number of shares of our common stock on the immediately preceding December 31, (ii) 450,000 shares, or (iii) such lesser number as determined by our compensation committee.

All employees who have been employed by us or our designated subsidiaries for at least six weeks and whose customary employment is for more than 20 hours a week are eligible to participate in our ESPP. Any employee who owns, or would own upon such purchase under our ESPP, 5% or more of the voting power or value of our stock is not eligible to purchase shares under our ESPP.

We may make one or more offerings to our employees to purchase stock under our ESPP. Unless otherwise determined by the administrator of the ESPP, the first offering will begin on January 1st of the year designated

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by our compensation committee and end on the following June 30th. Unless otherwise determined by our compensation committee, subsequent offerings will begin on the first business day occurring on or after each January 1st and July 1st and will end on the last business day occurring on or before the following June 30th and December 31st, respectively, each referred to as offering periods. Our compensation committee may designate different offering periods in its discretion but no offering shall exceed six months in duration or overlap with another offering.

Each employee who is a participant in the ESPP may purchase shares by authorizing payroll deductions of up to 10% of his or her eligible compensation during an offering period. Unless the participating employee has previously withdrawn from the offering, his or her accumulated payroll deductions will be used to purchase common stock on the last business day of the offering period at a price equal to 85% of the fair market value of the common stock on the first or the last business day of the offering period, whichever is lower, provided that no more than a number of shares of common stock determined by dividing $12,500 by the fair market value of our common stock on the first day of the offering period or such other maximum number established by the compensation committee may be purchased by any one employee during each offering period. Under applicable tax rules, an employee may purchase no more than $25,000 worth of common stock, valued at the start of the purchase period, under the ESPP in any calendar year.

The accumulated payroll deductions of any employee who is not a participant on the last day of an offering period will be refunded. An employee s rights under the ESPP terminate upon voluntary withdrawal from the plan or when the employee ceases employment for any reason.

The ESPP will terminate on the tenth anniversary of the date on which it was approved by our board of directors and stockholders and may be terminated or amended by our board of directors at any time prior to that date. Amendments that increase the number of shares of our common stock authorized under the ESPP and certain other amendments require the approval of our stockholders.

401(k) Savings Plan and Other Benefits

We have established a 401(k) plan to allow our employees to save on a tax-favorable basis for their retirement. We do not match any contributions made by any employees, including our named executive officers, pursuant to the plan. We also pay, on behalf of our employees, the premiums for health, life and disability insurance.

Pension Benefits, Non-Qualified Defined Contribution Plans and Other Non-Qualified Defined Compensation Plans

We do not provide a pension plan or nonqualified defined contribution plans for any of our employees, and none of our named executive officers participated in a nonqualified deferred compensation plan during the fiscal year ended December 31, 2012.

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CERTAIN RELATIONSHIPS AND RELATED PARTY TRANSACTIONS

Other than the compensation agreements and other arrangements described under Executive and Director Compensation in this prospectus and the transactions described below, since January 1, 2010, there has not been and there is not currently proposed, any transaction or series of similar transactions to which we were, or will be, a party in which the amount involved exceeded, or will exceed, $120,000 and in which any director, executive officer, holder of five percent or more of any class of our capital stock or any member of the immediate family of, or entities affiliated with, any of the foregoing persons, had, or will have, a direct or indirect material interest.

Private Placements of Securities

Series C Preferred Stock Financing

In May 2012, we entered into a Series C convertible preferred stock purchase agreement, which was subsequently amended in July 2012. Pursuant to the purchase agreement, we issued an aggregate of 16,808,504 shares of our Series C convertible preferred stock at a price of $1.00 per share in three closings in May 2012, July 2012 and October 2012.

The following table summarizes the participation in our Series C preferred stock financing by holders of five percent or more of our voting securities:

Name of Investor	Shares of Series C Convertible Preferred Stock	Aggregate Purchase Price Paid
ARCH Venture Fund VI, L.P.(1)	3,709,314	$ 3,709,314
OVP Venture Partners VII, L.P.(2)	3,217,323	$ 3,217,323
OVP VII Entrepreneurs Fund, L.P.(2)	22,680	$ 22,680
Polaris Venture Partners Entrepreneurs Fund V, L.P.(3)	69,760	$ 69,760
Polaris Venture Partners Founders Fund V, L.P.(3)	24,518	$ 24,518
Polaris Venture Partners Special Founders Fund V, L.P.(3)	35,793	$ 35,793
Polaris Venture Partners V, L.P.(3)	3,579,243	$ 3,579,243
Venrock Associates V, L.P.(4)	3,346,914	$ 3,346,914
Venrock Entrepreneurs Fund V, L.P.(4)	78,638	$ 78,638
Venrock Partners V, L.P.(4)	283,762	$ 283,762

(1) Robert T. Nelsen, a member of our board of directors, is affiliated with ARCH Venture Partners. See footnote 2 to the table in Principal Stockholders for more information.

(2) Carl Weissman, a former member of our board of directors, is affiliated with OVP Venture Partners. See footnote 5 to the table in Principal Stockholders for more information.

(3) Amir Nashat, a member of our board of directors, is affiliated with Polaris Venture Partners. See footnote 3 to the table in Principal Stockholders for more information.

(4) Bryan E. Roberts, a former member of our board of directors, is affiliated with Venrock. See footnote 4 to the table in Principal Stockholders for more information.

Upon the completion of this offering, all shares of Series C convertible preferred stock purchased and held by investors in our Series C preferred stock financing will convert into shares of our common stock on a one-for-one basis. In connection with our Series C preferred stock financing, the stockholders listed above entered into an amended and restated investor rights agreement with us in May 2012, which was further amended

and restated in June and August 2013. The terms of the amended and restated investor rights agreement are described in more detail under Description of Capital Stock Registration Rights.

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2013 Convertible Note Financings

In June and July 2013, we issued convertible promissory notes in an aggregate principal amount of $3.7 million to certain existing stockholders. The terms of these convertible promissory notes are further described under Management s Discussion and Analysis of Financial Condition and Results of Operations Liquidity and Capital Resources 2013 Convertible Note Financings.

The following table summarizes the participation in our June and July 2013 convertible note financing by holders of five percent or more of our voting securities:

Name of Investor	Principal Amount of Notes Purchased	Number of Shares of Common Stock Issuable upon Conversion of Notes[(1)]
ARCH Venture Fund VI, L.P.	$ 766,403.08	128,447
OVP Venture Partners VII, L.P.	$ 695,885.33	116,629
OVP VII Entrepreneurs Fund, L.P.	$ 4,905.48	822
Polaris Venture Partners Entrepreneurs Fund V, L.P.	$ 14,413.20	2,415
Polaris Venture Partners Founders Fund V, L.P.	$ 5,065.87	849
Polaris Venture Partners Special Founders Fund V, L.P.	$ 7,395.55	1,239
Polaris Venture Partners V, L.P.	$ 739,528.46	123,943
Venrock Associates V, L.P.	$ 691,525.58	115,898
Venrock Entrepreneurs Fund V, L.P.	$ 16,247.74	2,723
Venrock Partners V, L.P.	$ 58,629.76	9,826

(1) The number of shares of common stock issuable upon conversion of our 2013 Notes is determined based on the initial public offering price of $6.00 per share.

All of the notes set forth in the table above were purchased in June 2013.

Transactions with our Executive Officers, Directors and Beneficial Owners

Employment Agreements

We have entered into offer letters or employment related agreements with each of Christian Weyer, J. Scott Wolchko and Pratik S. Multani. For more information regarding these arrangements, see Executive and Director Compensation Employment Arrangements with Our Named Executive Officers.

Consulting Agreement

In December 2012, we entered into a consulting agreement with John D. Mendlein, the vice chairman of our board of directors, which terminated his prior employment agreement with us. Pursuant to the consulting agreement, Dr. Mendlein provides consulting services with respect to leadership and performance of strategic projects. As compensation for such services, Dr. Mendlein is entitled to an annual fee in the amount of $20,000, payable in periodic installments. In addition, we have agreed to enter into an agreement with Dr. Mendlein upon the completion of this offering regarding a change of control (as defined in his consulting agreement), at his request, whereby we will make a gross-up payment such that, in the event certain excise taxes and penalties are imposed upon Dr. Mendlein as a result of Section 280G or 4999 of the Code, his net after-tax payments and benefits will be equal to what he would have received absent such penalty tax. The consulting agreement does not have a specified term and is terminable by us or Dr. Mendlein at any time, with or without cause or notice.

Under the terms of Dr. Mendlein s prior employment agreement with us entered into in April 2008, Dr. Mendlein s served as the executive chairman of our board of directors and interim Chief Scientific Officer. His annual base salary for the first eighteen (18) months was $150,000, and $100,000 after October 1, 2009. Dr. Mendlein would be considered annually for a bonus target of up to 40% of his then-current base salary,

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subject to achievement of reasonably attainable performance targets as determined by our board of directors. No bonus was paid to Dr. Mendlein for the year ended December 31, 2012. If Dr. Mendlein s employment was terminated by him for good reason or by the Company without cause, he would have received (i) a cash severance amount equal to six months of his then-current salary and one-half of the full bonus, and (ii) six months of COBRA and other employee benefits, subject to the execution and non-revocation of a release agreement and written acknowledgement of continuing confidentiality obligations.

Contemporaneously with the consulting agreement, we entered into an amended and restated restricted stock purchase agreement with Dr. Mendlein to amend the repurchase restrictions applicable to the remaining 35,026 restricted shares issued pursuant to his April 2008 restricted stock purchase agreement. Under the amended agreement, our repurchase right will lapse upon the achievement of one or more performance milestones. In addition, our right to repurchase (x) 50% of Dr. Mendlein s then-restricted shares will lapse in full immediately prior to a change in control, and (y) 25% of Dr. Mendlein s then-restricted shares will lapse in full immediately prior to the completion of this offering.

Indemnification Agreements

We have entered into indemnification agreements with or have contractual obligations to provide indemnification to each of our directors and intend to enter into such agreements with certain of our executive officers. These agreements require us, among other things, to indemnify these individuals for certain expenses (including attorneys fees), judgments, fines and settlement amounts reasonably incurred by such person in any action or proceeding, including any action by or in our right, on account of any services undertaken by such person on behalf of our company or that person s status as a member of our board of directors to the maximum extent allowed under Delaware law.

Restricted Stock and Stock Option Awards

For information regarding restricted stock and stock option awards granted to our named executive officers and directors, see Executive and Director Compensation.

Registration Rights

We and certain holders of our capital stock and holders of the 2013 Notes have entered into an investor rights agreement pursuant to which these stockholders will have, among other things, registration rights under the Securities Act, with respect to common stock that they will hold following this offering. See Description of Capital Stock Registration Rights for a further description of the terms of these agreements.

Participation in this Offering

Certain holders of five percent or more of our voting securities and stockholders who are affiliated with certain of our directors have agreed to purchase an aggregate of 2,499,999 shares of our common stock in this offering at the initial public offering price as follows:

Beneficial Owner	Shares to be Purchased in Offering
ARCH Venture Fund VI, L.P.	833,333
Entities affiliated with Polaris Venture Partners	833,333
Entities affiliated with Venrock	833,333

The underwriting discount for the shares purchased in this offering by the stockholders listed above will be $0.21 per share. In addition, William Rastetter and John Mendlein, members of our board of directors, have agreed to purchase 83,333 and 16,667 shares of common stock, respectively, in this offering at the initial public offering price. The underwriting discount for the shares purchased in this offering by Drs. Rastetter and Mendlein will be $0.42 per share.

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Policies for Approval of Related Party Transactions

Our board of directors reviews and approves transactions with directors, officers and holders of five percent or more of our voting securities and their affiliates, each a related party. Prior to this offering, the material facts as to the related party s relationship or interest in the transaction are disclosed to our board of directors prior to their consideration of such transaction, and the transaction is not considered approved by our board of directors unless a majority of the directors who are not interested in the transaction approve the transaction. Further, when stockholders are entitled to vote on a transaction with a related party, the material facts of the related party s relationship or interest in the transaction are disclosed to the stockholders, who must approve the transaction in good faith.

In connection with this offering, we have adopted a written related party transactions policy that such transactions must be approved by our audit committee.

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PRINCIPAL STOCKHOLDERS

The following table sets forth certain information known to us regarding beneficial ownership of our capital stock as of August 15, 2013, as adjusted to reflect the sale of common stock offered by us in this offering, for:

- each person, or group of affiliated persons, known by us to be the beneficial owner of more than five percent of our capital stock;
- our named executive officers;
- each of our other directors; and
- all executive officers and directors as a group.

Beneficial ownership is determined in accordance with the rules of the SEC and generally includes voting or investment power with respect to securities. Except as noted by footnote, and subject to community property laws where applicable, we believe based on the information provided to us that the persons and entities named in the table below have sole voting and investment power with respect to all common stock shown as beneficially owned by them.

The percentage of beneficial ownership prior to this offering in the table below is based on 8,998,483 shares of common stock deemed to be outstanding as of August 15, 2013, and gives effect to (i) the conversion of all outstanding shares of our convertible preferred stock into an aggregate of 7,229,590 shares of common stock, (ii) the issuance of an aggregate of 403,841 shares of common stock pursuant to the redemption of exchangeable shares of Fate Canada as described in Description of Capital Stock Exchangeable Shares in Canadian Subsidiary, but does not give effect to the conversion, as described below, of an aggregate principal amount of $23.7 million and all accrued but unpaid interest on our 2013 Notes upon the completion of this offering and (iii) our one-for-6.5 reverse split of our common stock, which became effective on September 12, 2013. The percentage of beneficial ownership after this offering in the table below is based on 19,164,469 shares of common stock to be outstanding after this offering and gives effect to (i) the conversion of all outstanding shares of our convertible preferred stock into an aggregate of 7,229,590 shares of common stock, (ii) the conversion of an aggregate of approximately $21.0 million in principal and accrued interest outstanding on our 2013 Notes upon the completion of this offering into an aggregate of 3,499,319 shares of our common stock, based on the initial public offering price of $6.00 per share, and assuming we repay in cash approximately $2.8 million in outstanding principal and accrued interest under the convertible promissory notes issued in August 2013 in lieu of the issuance of 470,252 shares of common stock upon the conversion of such notes, which, if issued, would cause the holders of such notes, together with their affiliates and related parties, to beneficially own 15% or more of our outstanding shares of common stock immediately following this offering and (iii) the issuance of an aggregate of 403,841 shares of our common stock pursuant to the redemption of exchangeable shares of Fate Canada as described in Description of Capital Stock Exchangeable Shares in Canadian Subsidiary. The information in the table below assumes no exercise of the underwriters option to purchase additional shares. Options to purchase shares of common stock that are exercisable within 60 days of August 15, 2013 are deemed to be beneficially owned by the persons holding these options for the purpose of computing percentage ownership of that person, but are not treated as outstanding for the purpose of computing any other person s ownership percentage. Shares beneficially owned include restricted shares of common stock acquired upon any early exercise of stock options granted under our 2007 Plan.

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Name and Address of Beneficial Owner[1]	Number of Shares Beneficially Owned before Offering	Number of Shares Beneficially Owned after Offering	Percentage of Shares Beneficially Owned before Offering	Percentage of Shares Beneficially Owned after Offering
Five Percent or Greater Stockholders:				
ARCH Venture Fund VI, L.P.[2]	1,511,408	2,473,188	16.8%	12.9%
Entities affiliated with Polaris Venture Partners[3]	1,511,407	2,473,186	16.8%	12.9%
Entities affiliated with Venrock[4]	1,511,407	2,473,187	16.8%	12.9%
Entities affiliated with OVP Venture Partners[5]	1,382,016	1,499,467	15.4%	7.8%
Entities affiliated with Fidelity Investments[6]		2,873,490		15.0%
All 5% Stockholders as a group	5,916,238	11,792,518	65.7%	61.5%
Named Executive Officers and Directors:				
Christian Weyer, M.D., M.A.S.[7].	143,154	143,154	1.6%	*
J. Scott Wolchko[8].	63,353	63,353	*	*
Pratik S. Multani, M.D., M.S. [9]	59,599	59,599	*	*
William H. Rastetter, Ph.D. [10].	118,360	201,693	1.3%	1.1%
John D. Mendlein, Ph.D. [11]	171,669	188,336	1.9%	*
Timothy P. Coughlin[12]				
Mark J. Enyedy[13]	9,615	9,615	*	*
Amir Nashat, Sc.D.[3].	1,511,407	2,473,186	16.8%	12.9%
Robert T. Nelsen[2]	1,511,408	2,473,188	16.8%	12.9%
All executive officers and directors as a group (11 persons)[14]	3,694,094	5,717,653	39.6%	29.3%

* Represents beneficial ownership of less than one percent.

(1) Unless otherwise indicated, the address for each beneficial owner is c/o Fate Therapeutics, Inc., 3535 General Atomics Court, Suite 200, San Diego, CA 92121.

(2) The ownership of ARCH Venture Fund VI, L.P. (ARCH Fund VI) consists of an aggregate of 1,511,408 shares of common stock issuable upon conversion of 4,390,706 shares of Series A convertible preferred stock, 1,500,000 shares of Series B convertible preferred stock and 3,709,314 shares Series C convertible preferred stock. The number and percentage of shares owned with this offering also includes 128,447 shares of common stock issuable to ARCH Fund VI upon conversion of all outstanding principal and accrued interest on the 2013 Note held by ARCH Fund VI upon the closing of this offering, as described above, and 833,333 shares of common stock purchased in the offering at the initial public offering price. The sole general partner of ARCH Fund VI is ARCH Venture Partners VI, L.P. (ARCH Partners VI), which may be deemed to beneficially own certain of the shares held by ARCH Fund VI. ARCH Partners VI disclaims beneficial ownership of all shares held by ARCH Fund VI in which it does not have an actual pecuniary interest. The sole general partner of ARCH Partners VI is ARCH Venture Partners VI, LLC (ARCH VI LLC), which may be deemed to beneficially own certain of the shares held by ARCH Fund VI. ARCH VI LLC disclaims beneficial ownership of all shares held by ARCH Fund VI in which it does not have an actual pecuniary interest. The managing directors of ARCH VI LLC, Robert T. Nelson, Keith Crandell and Clinton Bybee (together the Managing Directors), are deemed to have voting and dispositive power over the shares held by ARCH Fund VI and may be deemed to beneficially own certain of the shares held by ARCH Fund VI. Mr. Nelsen, a member of our board of directors, is one of the Managing Directors. The Managing Directors disclaim beneficial ownership of all shares held by ARCH Fund VI. The mailing address of the beneficial owner is 8725 West Higgins Road, Suite 290, Chicago, IL 60631.

(3) Consists of: (i) an aggregate of 1,458,409 shares of common stock issuable upon conversion of 4,236,741 shares of Series A convertible preferred stock, 1,447,401 shares of Series B convertible preferred stock and 3,579,243 shares of Series C convertible preferred stock held by Polaris Venture Partners V, L.P. (Polaris Ventures), (ii) an aggregate of 28,424 shares of common stock issuable upon conversion of 82,572 shares of Series A convertible preferred stock, 28,209 shares of Series B convertible preferred stock and 69,760 shares of Series C convertible preferred stock held by Polaris Venture Partners Entrepreneurs Fund V, L.P. (Polaris Entrepreneurs Fund), (iii) an aggregate of 9,990 shares of common stock issuable upon conversion of 29,023 shares of Series A convertible preferred stock, 9,915 shares of Series B

convertible

preferred stock and 24,518 shares of Series C convertible preferred stock held by Polaris Venture Partners Founders Fund V, L.P. (Polaris Founders Fund) and (iv) an aggregate of 14,584 shares of common stock issuable upon conversion of 42,370 shares of Series A convertible preferred stock, 14,475 shares of Series B convertible preferred stock and 35,793 shares of Series C convertible preferred stock held by Polaris Venture Partners Special Founders Fund V, L.P. (Polaris Special Founders Fund). The number and percentage of shares owned after this offering also includes (i) 123,943 shares of common stock issuable to Polaris Ventures upon conversion of all outstanding principal and accrued interest on the 2013 Note held by Polaris Ventures, (ii) 2,415 shares of common stock issuable to Polaris Entrepreneurs Fund upon conversion of all outstanding principal and accrued interest on the 2013 Note held by Polaris Entrepreneurs Fund, (iii) 849 shares of common stock issuable to Polaris Founders Fund upon conversion of all outstanding principal and accrued interest on the 2013 Note held by Polaris Founders Fund and (iv) 1,239 shares of common stock issuable to Polaris Special Founders Fund upon conversion of all outstanding principal and accrued interest on the 2013 Note held by Polaris Special Founders Fund, in each case based on the assumptions described above. In addition, the number and percentage of shares owned after this offering include an aggregate of 833,333 shares of common stock purchased in the offering at the initial public offering price. Each of the funds has sole voting and investment power with respect to the shares held by such funds. The general partner of Polaris Ventures, Polaris Entrepreneurs Fund, Polaris Founders Fund and Polaris Special Founders Fund is Polaris Venture Management Co. V, LLC (Polaris Management), and Polaris Management may be deemed to have sole voting and investment power over such shares. Polaris Management disclaims beneficial ownership of all such shares, except to the extent of any pecuniary interest therein. Amir Nashat, a member of our board of directors, is one of six members of Polaris Management. He has shared voting and investment power over such shares and may be deemed the indirect beneficial owner of such shares. Dr. Nashat disclaims beneficial ownership of these shares, except to the extent of any pecuniary interest therein. The members of North Star Venture Management 2010 LLC are also members of Polaris Management, and as members of the general partner, they may be deemed to share voting and investment power over such shares. The mailing address of the beneficial owner is 1000 Winter Street, Suite 3350, Waltham, MA 02451.

(4) Consists of: (i) an aggregate of 1,363,744 shares of common stock issuable upon conversion of 3,961,734 shares of Series A convertible preferred stock, 1,353,450 shares of Series B convertible preferred stock and 3,346,914 shares of Series C convertible preferred stock held by Venrock Associates V, L.P. (Venrock), (ii) an aggregate of 115,622 shares of common stock issuable upon conversion of 335,889 shares of Series A convertible preferred stock, 114,750 shares of Series B convertible preferred stock and 283,762 shares of Series C convertible preferred stock held by Venrock Partners V, L.P. (Venrock Partners) and (iii) an aggregate of 32,041 shares of common stock issuable upon conversion of 93,083 shares of Series A convertible preferred stock, 31,800 shares of Series B convertible preferred stock and 78,638 shares of Series C convertible preferred stock held by Venrock Entrepreneurs Fund V, L.P. (Venrock Entrepreneurs). The number and percentage of shares owned after this offering also includes (i) 115,898 shares of common stock issuable to Venrock upon conversion of all outstanding principal and accrued interest on the 2013 Note held by Venrock, (ii) 9,826 shares of common stock issuable to Venrock Partners upon conversion of all outstanding principal and accrued interest on the 2013 Note held by Venrock Partners and (iii) 2,723 shares of common stock issuable to Venrock Entrepreneurs upon conversion of all outstanding principal and accrued interest on the 2013 Note held by Venrock Entrepreneurs, in each case based on the assumptions described above. In addition, the number and percentage of shares owned after this offering include an aggregate of 833,333 shares of common stock purchased in the offering at the initial public offering price. The sole general partner of Venrock is Venrock Management V, LLC (Venrock Management V). The sole general partner of Venrock Partners is Venrock Partners Management V, LLC (Venrock Partners Management V). The sole general partner of Venrock Entrepreneurs is VEF Management V, LLC (VEF). Venrock Management V, Venrock Partners Management V and VEF disclaim beneficial ownership over all shares held by Venrock Associates, Venrock Partners and Venrock Entrepreneurs, except to the extent of any pecuniary interest therein. Bryan E. Roberts is our former director and is a partner of Venrock, and as such, he may be deemed to have voting and investment power with respect to these shares. Dr. Roberts disclaims beneficial ownership of these shares except to the extent of any pecuniary interest therein. William H. Rastetter, a member of our board of directors,

was formerly a partner of Venrock, but does not have voting or investment control over the shares held by the Venrock entities. The mailing address of the beneficial owner is 3340 Hillview Avenue, Palo Alto, CA 94304.

(5) Consists of: (i) an aggregate of 1,372,342 shares of common stock issuable upon conversion of 1,137,383 shares of Series A convertible preferred stock, 3,972,000 shares of Series B convertible preferred stock and 3,217,323 shares of Series C convertible preferred stock held by OVP Venture Partners VII, L.P. (OVP Venture Partners) and (ii) an aggregate of 9,674 shares of common stock issuable upon conversion of 8,018 shares of Series A convertible preferred stock, 28,000 shares of Series B convertible preferred stock and 22,680 shares of Series C convertible preferred stock held by OVP VII Entrepreneurs Funds, L.P. (OVP Entrepreneurs Fund). The number and percentage of shares owned after this offering also includes (i) 116,629 shares of common stock issuable to OVP Venture Partners upon conversion of all outstanding principal and accrued interest on the 2013 Note held by OVP Venture Partners and (ii) 822 shares of common stock issuable to OVP Entrepreneurs Fund upon conversion of all outstanding principal and accrued interest on the 2013 Note held by OVP Entrepreneurs Fund, in each case based on the assumptions described above. The sole general partner of OVP Venture Partners is OVMC VII, LLC (OVMC). The sole general partner of OVP Entrepreneurs Fund is OVMC VII, LLC (OVMC). OVMC disclaims beneficial ownership of the shares held by OVP Venture Partners and OVP Entrepreneurs Fund, except to the extent of any pecuniary interest therein. Our former director Carl Weissman is an assignee member of OVMC, and as such, he does not have direct voting or investment power with respect to these shares. The mailing address of the beneficial owner is 1616 Eastlake Ave. E., Suite 208, Seattle, WA 98102.

(6) The number and percentage of shares owned after this offering consists of: (i) 1,436,746 shares of common stock issuable to Ball & Co fbo Fidelity Mt. Vernon Street Trust: Fidelity Growth Company (Mt. Vernon Street Trust) upon conversion of a portion of the outstanding principal and accrued interest on the 2013 Note held by Mt. Vernon Street Trust; (ii) 136,921 shares of common stock issuable to Bangle & Co fbo Fidelity Advisor Series VII: Fidelity Advisor Biotechnology Fund (Fidelity Advisor) upon conversion of a portion of the outstanding principal and accrued interest on the 2013 Note held by Fidelity Advisor; and (iii) 1,299,823 shares of common stock issuable to Mag & Co fbo Fidelity Select Portfolios: Biotechnology Portfolio (Fidelity Select) upon conversion of a portion of the outstanding principal and accrued interest on the 2013 Note held by Fidelity Select, in each case based on the assumptions described above. Each of these entities is a registered investment fund (each, a Fund) advised by Fidelity Management & Research Company (FMR Co.), a registered investment adviser under the Investment Advisers Act of 1940, as amended, or its affiliate. The address of FMR Co., a wholly-owned subsidiary of FMR LLC and an investment adviser registered under Section 203 of the Investment Advisers Act of 1940 is 245 Summer Street, Boston, Massachusetts 02210. FMR LLC, through its control of FMR Co., Edward C. Johnson 3d, as Chairman of FMR LLC, and each Fund has power to dispose of the securities owned by such Fund. Neither FMR LLC nor Edward C. Johnson 3d has sole power to vote or direct the voting of the shares owned directly by each Fund, which power resides with each Fund s Board of Trustees.

(7) Consists of options to purchase 143,154 shares of common stock that are exercisable within 60 days of August 15, 2013 held by Dr. Weyer, all shares of which, if exercised, would be subject to our right of repurchase.

(8) Consists of: (i) 35,026 shares of common stock and (ii) options to purchase 28,327 shares of common stock that are exercisable within 60 days of August 15, 2013 held by Mr. Wolchko.

(9) Consists of options to purchase 59,599 shares of common stock that are exercisable within 60 days of August 15, 2013 held by Dr. Multani.

(10) Consists of 118,360 shares of common stock held by The Rastetter Family Trust, dated September 2, 2010, 66,578 shares of which are subject to our right of repurchase as of August 15, 2013. William H. Rastetter and Marisa Gard Rastetter, as co-trustees of The Rastetter Family Trust, share dispositive power over these shares. The number and percentage of shares owned after this offering also includes 83,333 shares purchased in this offering at the initial public offering price. For more information regarding our right of repurchase over the

restricted stock held by The Rastetter Family Trust, see Executive and Director Compensation Outstanding Equity Awards at Fiscal Year-End .

(11) Consists of: (i) 140,107 shares of common stock, 35,026 shares of which are subject to our right of repurchase as of August 15, 2013 as set forth in the amended and restated restricted stock purchase agreement with Dr. Mendlein, and (ii) options to purchase 31,562 shares of common stock that are exercisable within 60 days of August 15, 2013 held by Dr. Mendlein, 19,727 shares of which, if exercised, would be subject to our right of repurchase. The number and percentage of shares owned after this offering also includes 16,667 shares purchased in this offering at the initial public offering price. For more information regarding Dr. Mendlein s restricted stock, see Certain Relationships and Related Party Transactions Consulting Agreement .

(12) Mr. Coughlin joined our board of directors in August 2013.

(13) Consists of options to purchase 9,615 shares of common stock that are exercisable within 60 days of August 15, 2013 held by Mr. Enyedy.

(14) Includes the number of shares beneficially owned by the named executive officers and directors listed in the above table, as well as (i) 10,769 shares of common stock owned of record by Peter Flynn, our Senior Vice President, Early Program Development, (ii) options to purchase 33,801 shares of common stock that are exercisable within 60 days of August 15, 2013 held by Dr. Flynn, (iii) 34,615 shares of common stock owned of record by Daniel D. Shoemaker, our Chief Technology Officer, and (iv) options to purchase 26,344 shares of common stock that are exercisable within 60 days of August 15, 2013 held by Dr. Shoemaker.

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DESCRIPTION OF CAPITAL STOCK

The following descriptions are summaries of the material terms of our amended and restated certificate of incorporation and amended and restated bylaws, which will be effective upon completion of this offering. The descriptions of the common stock and preferred stock give effect to changes to our capital structure that will occur immediately prior to the completion of this offering. We refer in this section to our amended and restated certificate of incorporation as our certificate of incorporation, and we refer to our amended and restated bylaws as our bylaws.

General

Upon completion of this offering, our authorized capital stock will consist of 150,000,000 shares of common stock, par value $0.001 per share, and 5,000,000 shares of preferred stock, par value $0.001 per share, all of which shares of preferred stock will be undesignated.

As of June 30, 2013, 8,586,782 shares of our common stock were outstanding and held by 80 stockholders of record. This amount assumes the conversion of all outstanding shares of our convertible preferred stock into common stock, which will occur immediately prior to the completion of this offering, but excludes (i) the issuance of 403,841 shares of common stock pursuant to the redemption of an aggregate of 900,000 exchangeable shares of Fate Canada as described below, which will occur immediately prior to the completion of this offering and (ii) the issuance of 3,499,319 shares of common stock upon the conversion of approximately $21.0 million in outstanding principal and accrued interest under our 2013 Notes upon the completion of this offering, based on the initial public offering price per share of $6.00, and assuming that we repay in cash an aggregate of approximately $2.8 million in principal and accrued interest under the 2013 Notes issued in August 2013 in lieu of the issuance of 470,252 shares of common stock upon the conversion of such notes, which, if issued, would cause the holders of such notes, together with their affiliates and related entities, to beneficially own 15% or more of our outstanding common stock following the completion of this offering. In addition, as of June 30, 2013, we had outstanding options to purchase 1,518,712 shares of our common stock under our 2007 Equity Incentive Plan, at a weighted average exercise price of $1.46 per share, 377,492 of which were exercisable.

Common Stock

The holders of our common stock are entitled to one vote for each share held on all matters submitted to a vote of the stockholders. The holders of our common stock do not have any cumulative voting rights. Holders of our common stock are entitled to receive ratably any dividends declared by the board of directors out of funds legally available for that purpose, subject to any preferential dividend rights of any outstanding preferred stock. Our common stock has no preemptive rights, conversion rights or other subscription rights or redemption or sinking fund provisions.

In the event of our liquidation, dissolution or winding up, holders of our common stock will be entitled to share ratably in all assets remaining after payment of all debts and other liabilities and any liquidation preference of any outstanding preferred stock. The shares to be issued by us in this offering will be, when issued and paid for, validly issued, fully paid and non-assessable.

Preferred Stock

Immediately prior to the completion of this offering, all outstanding shares of our convertible preferred stock will be converted into shares of our common stock. Immediately prior to the completion of this offering, our amended and restated certificate of incorporation will be amended and restated to delete all references to such shares of convertible preferred stock. Upon the completion of this offering, our board of directors will have the authority, without further action by our stockholders, to issue up to 5,000,000 shares of preferred stock in one or more series and to fix the rights, preferences, privileges and restrictions thereof. These rights, preferences and privileges could include dividend rights, conversion rights, voting rights, terms of redemption, liquidation preferences, sinking fund terms and the number of shares constituting, or the designation of, such series, any or all of which may be greater than the rights of common stock. The issuance of our preferred stock could adversely affect the voting power of holders of common stock and the likelihood that such holders will receive dividend

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payments and payments upon our liquidation. In addition, the issuance of preferred stock could have the effect of delaying, deferring or preventing a change in control of our company or other corporate action. Immediately after completion of this offering, no shares of preferred stock will be outstanding, and we have no present plan to issue any shares of preferred stock.

Convertible Promissory Notes

In June and July 2013, we borrowed $3.7 million through the issuance of certain convertible promissory notes to existing stockholders. These notes accrue interest at 2% per year and are due on June 24, 2014. In August 2013, we borrowed $20.0 million through the issuance of certain convertible promissory notes to new investors. These notes accrue interest at 2% and are due on August 8, 2016. Upon the completion of this offering, subject to certain limitations on the conversion of our 2013 Notes issued in August 2013, approximately $21.0 million in principal and accrued interest under the 2013 Notes will convert into an aggregate of 3,499,319 shares of common stock, based on the initial public offering price per share of $6.00. For more information on the 2013 Notes, see Management s Discussion and Analysis of Financial Condition and Results of Operations Liquidity and Capital Resources 2013 Convertible Note Financings.

Warrants

As of June 30, 2013, we had outstanding warrants to purchase 200,000 shares of Series C convertible preferred stock and 30,000 shares of Series B convertible preferred stock, which are exercisable for an aggregate of 36,074 shares of common stock upon the completion of this offering.

In January 2009, in connection with a loan and security agreement entered into with SVB, we issued to SVB a warrant to purchase $60,000 worth of shares of the class and series of stock issued in the first sale or issuance of shares of convertible preferred stock or other senior equity securities after the issue date of the warrant. Upon completion of the Series B preferred stock financing, this warrant became exercisable for 30,000 shares of Series B convertible preferred stock. The warrant has a net exercise provision and contains provisions for the adjustment of the exercise price and the number of shares issuable upon the exercise of the warrant in the event of certain stock dividends, stock splits, recapitalizations, reclassifications and consolidations. The warrant is exercisable until its expiration on January 5, 2019.

In August 2011, in connection with the second amendment of the loan and security agreement entered into with SVB, we issued to SVB a warrant to purchase $200,000 worth of shares of the class and series of stock issued in the first sale or issuance of shares of convertible preferred stock or other senior equity securities after the issue date of the warrant. Upon completion of the Series C preferred stock financing, this warrant became exercisable for 200,000 shares of Series C convertible preferred stock. The warrant has a net exercise provision and contains provisions for the adjustment of the exercise price and the number of shares issuable upon the exercise of the warrant in the event of certain stock dividends, stock splits, recapitalizations, reclassifications and consolidations. The warrant is exercisable until its expiration on August 25, 2021.

Exchangeable Shares in Canadian Subsidiary

As of June 30, 2013, there were 900,000 exchangeable shares outstanding in the capital of our subsidiary, Fate Canada, which will be redeemed on the date immediately prior to the date of completion of this offering for an aggregate of 403,841 shares of our common stock. In addition, we may be obligated to issue to the holders of exchangeable shares of Fate Canada up to an aggregate of 480,764 shares of our common stock for no additional consideration, subject to (i) the occurrence of certain preclinical, clinical and commercial milestone events or (ii) upon (a) any consolidation or merger, other than one in which our stockholders immediately prior to such transaction continue to hold at least a majority of the voting power of the surviving entity in substantially the same proportions, or any transaction or series of related transactions to which we are a party and in which over 50% of our voting power is transferred in an arm s length transaction, in each case excluding bona fide equity financings and transfers to affiliates, or (b) a sale of all or substantially all of our assets or business, in each case, following the completion of this offering.

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Holders of substantially all of the exchangeable shares of Fate Canada are subject to lock-up agreements with the underwriters that restrict the sale of our securities for 180 days. See Underwriting for a description of these lock-up agreements.

Registration Rights

Upon the completion of this offering, the holders of 11,554,113 shares of our common stock, including shares issuable upon the conversion of our convertible preferred stock and 2013 Notes and shares issuable upon the exercise of outstanding warrants, or their permitted transferees, are entitled to rights with respect to the registration of these securities under the Securities Act, which we refer to as our registrable securities. These rights are provided under the terms of an investor rights agreement between us and certain holders our common stock, Series A convertible preferred stock, Series B convertible preferred stock, Series B-1 convertible preferred stock, Series C convertible preferred stock, warrants and 2013 Notes. The investor rights agreement includes demand registration rights, short-form registration rights and piggyback registration rights. All fees, costs and expenses of underwritten registrations under these agreements will be borne by us and all selling expenses, including underwriting discounts and selling commissions, will be borne by the holders of the shares being registered.

Demand Registration Rights

Beginning 180 days after the completion of this offering, the holders of at least 40% of our registrable securities are entitled to demand registration rights. Under the terms of the investor rights agreement, upon the written request of such holders to sell registrable securities with an anticipated aggregate offering price (net of underwriting discounts and commissions) of at least $5.0 million, we will be required to use our best efforts to file a registration statement covering the offering and sale of such securities and use reasonable, diligent efforts to effect the registration of all or a portion of these securities for public resale. We are required to effect only two registrations pursuant to this provision of the investor rights agreement. In the event we register securities in connection with an underwritten offering, the underwriters will have the right to limit the number of shares included in such offering.

Short-Form Registration Rights

Upon the completion of this offering, the holders of at least 25% of our registrable securities are also entitled to short form registration rights. Pursuant to the investor rights agreement, if we are eligible to file a registration statement on Form S-3, upon the written request of such holders to sell registrable securities at an aggregate offering price (net of underwriting discounts and commissions) of at least $1.0 million, we will be required to use our best efforts to effect a registration of such securities. We are required to effect only two registrations in any twelve month period pursuant to this provision of the investor rights agreement. In the event we register securities in connection with an underwritten offering, the underwriters will have the right to limit the number of shares included in such offering.

Piggyback Registration Rights

Upon the completion of this offering, the holders of our registrable securities are entitled to piggyback registration rights. If we register any of our securities either for our own account or for the account of other security holders, such holders are entitled to include their shares in the registration. In the event we register securities in connection with an underwritten offering, the underwriters will have the right to limit the number of shares included in such offering.

Indemnification

Our investor rights agreement contains customary cross-indemnification provisions, under which we are obligated to indemnify holders of registrable securities in the event of material misstatements or omissions in the registration statement attributable to us, and they are obligated to indemnify us for material misstatements or omissions attributable to them.

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Expiration of Registration Rights

The registration rights granted under the investor rights agreement will terminate after the earlier of (i) the fourth anniversary of the completion of this offering, (ii) with respect to any holder of registrable securities, the date on which all of such holder s shares can be sold during a three-month period without registration in reliance on Rule 144 under the Securities Act, or (iii) termination of the agreement upon consent of the parties.

Registration Rights Related to Holders of Exchangeable Shares

In addition, to the extent that any of our officers, senior management employees or consultants, or any of their respective affiliates, have the right to require registration of, or have the opportunity to include their shares of common stock on a registration statement, we will provide the holders of exchangeable shares of Fate Canada with substantially similar registration rights. Such registration rights shall terminate two years after this offering.

Anti-Takeover Effects of Our Certificate of Incorporation and Bylaws and Delaware Law

Our certificate of incorporation and bylaws include a number of provisions that may have the effect of delaying, deferring or preventing another party from acquiring control of us and encouraging persons considering unsolicited tender offers or other unilateral takeover proposals to negotiate with our board of directors rather than pursue non-negotiated takeover attempts. These provisions include the items described below.

Board Composition and Filling Vacancies

Our certificate of incorporation provides for the division of our board of directors into three classes serving staggered three-year terms, with one class being elected each year. Our certificate of incorporation also provides that directors may be removed only for cause and then only by the affirmative vote of the holders of 75% or more of the shares then entitled to vote at an election of directors. Furthermore, any vacancy on our board of directors, however occurring, including a vacancy resulting from an increase in the size of our board, may only be filled by the affirmative vote of a majority of our directors then in office even if less than a quorum. The classification of directors, together with the limitations on removal of directors and treatment of vacancies, has the effect of making it more difficult for stockholders to change the composition of our board of directors.

No Written Consent of Stockholders

Our certificate of incorporation provides that all stockholder actions are required to be taken by a vote of the stockholders at an annual or special meeting, and that stockholders may not take any action by written consent in lieu of a meeting. This limit may lengthen the amount of time required to take stockholder actions and would prevent the amendment of our bylaws or removal of directors by our stockholders without holding a meeting of stockholders.

Meetings of Stockholders

Our certificate of incorporation and bylaws provide that only a majority of the members of our board of directors then in office may call special meetings of stockholders and only those matters set forth in the notice of the special meeting may be considered or acted upon at a special meeting of stockholders. Our bylaws limit the business that may be conducted at an annual meeting of stockholders to those matters properly brought before the meeting.

Advance Notice Requirements

Our bylaws establish advance notice procedures with regard to stockholder proposals relating to the nomination of candidates for election as directors or new business to be brought before meetings of our stockholders. These procedures provide that notice of stockholder proposals must be timely given in writing to our corporate secretary prior to the meeting at which the action is to be taken. Generally, to be timely, notice must be received at our principal executive offices not less than 90 days nor more than 120 days prior to the first anniversary date of the annual meeting for the preceding year. Our bylaws specify the requirements as to form and content of all stockholders notices. These requirements may preclude stockholders from bringing matters before the stockholders at an annual or special meeting.

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Amendment to Certificate of Incorporation and Bylaws

Any amendment of our certificate of incorporation must first be approved by a majority of our board of directors, and if required by law or our certificate of incorporation, must thereafter be approved by a majority of the outstanding shares entitled to vote on the amendment and a majority of the outstanding shares of each class entitled to vote thereon as a class, except that the amendment of the provisions relating to stockholder action, board composition, limitation of liability and the amendment of our bylaws and certificate of incorporation must be approved by not less than 75% of the outstanding shares entitled to vote on the amendment, and not less than 75% of the outstanding shares of each class entitled to vote thereon as a class. Our bylaws may be amended by the affirmative vote of a majority of the directors then in office, subject to any limitations set forth in the bylaws; and may also be amended by the affirmative vote of at least 75% of the outstanding shares entitled to vote on the amendment, or, if our board of directors recommends that the stockholders approve the amendment, by the affirmative vote of the majority of the outstanding shares entitled to vote on the amendment, in each case voting together as a single class.

Undesignated Preferred Stock

Our certificate of incorporation provides for 5,000,000 authorized shares of preferred stock. The existence of authorized but unissued shares of preferred stock may enable our board of directors to discourage an attempt to obtain control of us by means of a merger, tender offer, proxy contest or otherwise. For example, if in the due exercise of its fiduciary obligations, our board of directors were to determine that a takeover proposal is not in the best interests of our stockholders, our board of directors could cause shares of preferred stock to be issued without stockholder approval in one or more private offerings or other transactions that might dilute the voting or other rights of the proposed acquirer or insurgent stockholder or stockholder group. In this regard, our certificate of incorporation grants our board of directors broad power to establish the rights and preferences of authorized and unissued shares of preferred stock. The issuance of shares of preferred stock could decrease the amount of earnings and assets available for distribution to holders of shares of common stock. The issuance may also adversely affect the rights and powers, including voting rights, of these holders and may have the effect of delaying, deterring or preventing a change in control of us.

Section 203 of the Delaware General Corporation Law

Upon completion of this offering, we will be subject to the provisions of Section 203 of the Delaware General Corporation Law. In general, Section 203 prohibits a publicly held Delaware corporation from engaging in a business combination with an interested stockholder for a three-year period following the time that this stockholder becomes an interested stockholder, unless the business combination is approved in a prescribed manner. Under Section 203, a business combination between a corporation and an interested stockholder is prohibited unless it satisfies one of the following conditions:

- before the stockholder became interested, our board of directors 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, shares owned by persons who are directors and also officers, and employee stock plans, in some instances, but not the outstanding voting stock owned by the interested stockholder; or
- at or after the time the stockholder became interested, the business combination was approved by our board of directors and authorized at an annual or special meeting of the stockholders by the affirmative vote of at least two-thirds 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, lease, pledge or other disposition involving the interested stockholder of 10% or more of the assets of the corporation;

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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.

Exchange Listing

Our common stock has been approved for listing on The NASDAQ Global Market under the trading symbol FATE.

Transfer Agent and Registrar

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

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SHARES ELIGIBLE FOR FUTURE SALE

Prior to this offering, there has been no public market for our shares. Future sales of our common stock in the public market, or the availability of such shares for sale in the public market, could adversely affect market prices prevailing from time to time. As described below, only a limited number of shares will be available for sale shortly after this offering due to contractual and legal restrictions on resale. Nevertheless, sales of our common stock in the public market after such restrictions lapse, or the perception that those sales may occur, could adversely affect the prevailing market price at such time and our ability to raise equity capital in the future.

Based on the number of shares outstanding as of June 30, 2013, upon the completion of this offering, 19,156,609 shares of our common stock will be outstanding, assuming no exercise of the underwriters option to purchase additional shares and no exercise of outstanding options or warrants, and assuming the conversion of approximately $21.0 million in principal and accrued interest on our 2013 Notes upon the completion of this offering, based on the initial public offering price of $6.00 per share, and assuming that we repay in cash an aggregate of approximately $2.8 million in principal and accrued interest under the 2013 Notes issued in August 2013 in lieu of the issuance of 470,252 shares of common stock upon the conversion of such notes, which, if issued, would cause the holders of such notes, together with their affiliates and related entities, to beneficially own 15% or more of our outstanding common stock following the completion of this offering. Of the outstanding shares, all of the shares sold in this 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, may only be sold in compliance with the limitations described below.

Rule 144

In general, a person who has beneficially owned restricted stock for at least six months would be entitled to sell their securities provided that (i) such person is not deemed to have been one of our affiliates at the time of, or at any time during the 90 days preceding, a sale and (ii) we are subject to the Securities Exchange Act of 1934, as amended, periodic reporting requirements for at least 90 days before the sale. Persons who have beneficially owned restricted shares for at least six months but who are our affiliates at the time of, or any time during the 90 days preceding, a sale, would be subject to additional restrictions, by which such person would be entitled to sell within any three-month period only a number of securities that does not exceed the greater of either of the following:

- 1% of the number of shares then outstanding, which will equal approximately 191,566 shares immediately after this offering assuming no exercise of the underwriters option to purchase additional shares, based on the number of shares outstanding as of June 30, 2013; 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;

provided, in each case, that we are subject to the Exchange Act periodic reporting requirements for at least 90 days before the sale. Such sales both by affiliates and by non-affiliates must also comply with the manner of sale, current public information and notice provisions of Rule 144.

Rule 701

Rule 701 under the Securities Act, as in effect on the date of this prospectus, permits resales of shares in reliance upon Rule 144 but without compliance with certain restrictions of Rule 144, including the holding period requirement. Most of our employees, executive officers or directors who purchased shares under a written compensatory plan or contract may be entitled to rely on the resale provisions of Rule 701, but all holders of Rule 701 shares are required to wait until 90 days after the date of this prospectus before selling their shares. However, substantially all Rule 701 shares are subject to lock-up agreements as described below and under Underwriting included elsewhere in this prospectus and will become eligible for sale upon the expiration of the restrictions set forth in those agreements.

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Lock-Up Agreements

All of our directors and executive officers and holders of substantially all of our shares have signed a lock-up agreement which prevents them from selling any of our common stock or any securities convertible into or exercisable or exchangeable for common stock for a period of not less than 180 days from the date of this prospectus without the prior written consent of the representatives. The representatives may, subject to certain requirements, release some or all of the shares subject to lock-up agreements prior to the expiration of the 180-day period.

Registration Rights

Upon completion of this offering, certain holders of our securities will be entitled to various rights with respect to registration of their shares under the Securities Act. Registration of these shares under the Securities Act would result in these shares becoming fully tradable without restriction under the Securities Act immediately upon the effectiveness of the registration. See Description of Capital Stock Registration Rights for additional information.

Equity Incentive Plans

We intend to file one or more registration statements on Form S-8 under the Securities Act to register our shares issued or reserved for issuance under our equity incentive plans. The first such registration statement is expected to be filed soon after the date of this prospectus and will automatically become effective upon filing with the SEC. Accordingly, shares registered under such registration statement will be available for sale in the open market, unless such shares are subject to vesting restrictions with us or the lock-up restrictions described above. As of September 30, 2013, we estimate that such registration statement on Form S-8 will cover approximately 3,469,901 shares.

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MATERIAL UNITED STATES FEDERAL INCOME TAX

CONSIDERATIONS FOR NON-U.S. HOLDERS

The following is a summary of material U.S. federal income tax considerations of the ownership and disposition of our common stock to non-U.S. holders. It is not intended to be a complete analysis of all the U.S. federal income tax considerations that may be relevant to non-U.S. holders. This summary is based upon the provisions of the Internal Revenue Code of 1986, as amended (the Code), Treasury regulations promulgated thereunder, administrative rulings and judicial decisions, all as of the date hereof. These authorities may be changed, possibly with retroactive effect, which may result in U.S. federal income tax consequences different from those set forth below. We have not sought any ruling from the Internal Revenue Service (the IRS) with respect to the statements made and the conclusions reached in the following summary. There can be no assurance that the IRS will agree with such statements and conclusions or that any contrary position taken by the IRS would not be sustained by a court.

This summary also does not address the tax considerations arising under the laws of any foreign, state or local jurisdiction. In addition, this discussion does not address tax considerations applicable to an investor s particular circumstances or to investors that may be subject to special tax rules, including, without limitation:

banks, insurance companies or other financial institutions;

persons subject to the alternative minimum tax;

tax-exempt organizations;

an integral part or controlled entity of a foreign sovereign;

dealers in securities or currencies;

traders in securities that elect to use a mark-to-market method of accounting for their securities holdings;

persons that own, or are deemed to own, more than five percent of our capital stock (except to the extent specifically set forth below);

controlled foreign corporations or passive foreign investment companies

certain former citizens or long-term residents of the United States;

persons who hold our common stock as a position in a hedging transaction, straddle, conversion transaction or other risk reduction transaction;

persons deemed to sell our common stock under the constructive sale provisions of the Code; or

persons who hold our common stock other than as a capital asset (generally, an asset held for investment purposes).

In addition, if a partnership holds our common stock, the tax treatment of a partner generally will depend on the status of the partner and upon the activities of the partnership. Accordingly, partnerships that hold our common stock, and partners in such partnerships, should consult their tax advisors.

YOU ARE URGED TO CONSULT YOUR TAX ADVISOR WITH RESPECT TO THE APPLICATION OF THE UNITED STATES FEDERAL INCOME TAX LAWS TO YOUR PARTICULAR SITUATION, AS WELL AS ANY TAX CONSEQUENCES OF THE PURCHASE, OWNERSHIP AND DISPOSITION OF OUR COMMON STOCK ARISING UNDER THE UNITED STATES FEDERAL ESTATE OR GIFT TAX RULES OR UNDER THE LAWS OF ANY STATE, LOCAL, FOREIGN OR OTHER TAXING JURISDICTION OR UNDER ANY APPLICABLE TAX TREATY.

Non-U.S. Holder Defined

For purposes of this discussion, a non-U.S. holder is a beneficial owner of a share of common stock received that is (i) a foreign corporation, (ii) a nonresident alien individual, or (iii) a foreign estate or trust that in either case is not subject to U.S. federal income tax on a net income basis on income or gain from a note or share of common stock.

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Distributions

We have not made any distributions on our common stock and do not plan to make any distributions for the foreseeable future. However, if we do make distributions on our common stock, those payments will constitute dividends for U.S. tax purposes to the extent paid from our current or accumulated earnings and profits, as determined under U.S. federal income tax principles. To the extent those distributions exceed both our current and our accumulated earnings and profits, they will constitute a return of capital and will first reduce your basis in our common stock, but not below zero, and then will be treated as gain from the sale of stock, which will be subject to tax as described in Gain on Disposition of Common Stock , below.

Any dividend paid to you generally will be subject to U.S. withholding tax either at a rate of 30% of the gross amount of the dividend or such lower rate as may be specified by an applicable income tax treaty. In order to receive a reduced treaty rate, you must provide us with an IRS Form W-8BEN or other appropriate version of IRS Form W-8 or successor form certifying qualification for the reduced rate.

Dividends received by you that are effectively connected with your conduct of a U.S. trade or business are exempt from such withholding tax. In order to obtain this exemption, you must provide us with an IRS Form W-8ECI or successor form properly certifying such exemption. Such effectively connected dividends, although not subject to withholding tax, are taxed at the same graduated rates applicable to U.S. persons, net of certain deductions and credits. In addition, if you are a corporate non-U.S. holder, dividends you receive that are effectively connected with your conduct of a U.S. trade or business may also be subject to a branch profits tax at a rate of 30% or such lower rate as may be specified by an applicable income tax treaty.

If you are eligible for a reduced rate of withholding tax pursuant to a tax treaty, you may obtain a refund of any excess amounts withheld if you file an appropriate claim for refund with the IRS.

Gain on Disposition of Common Stock

You generally will not be required to pay U.S. federal income tax on any gain realized upon the sale or other disposition of our common stock unless:

- the gain is effectively connected with your conduct of a U.S. trade or business;
- you are an individual non-U.S. holder who holds our common stock as a capital asset, who is present in the United States for a period or periods aggregating 183 days or more during the calendar year in which the sale or disposition occurs and certain other conditions are met; or