FATE THERAPEUTICS INC
Form 10-K
March 17, 2014

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# UNITED STATES
# SECURITIES AND EXCHANGE COMMISSION

**Washington, D.C. 20549**

# FORM 10-K

**(Mark One)**

ý **ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

**For the fiscal year ended December 31, 2013**

o **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

**For the transition period from to .**
**Commission file number 001-36067**

# FATE THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

| **Delaware** | **65-1311552** |
|---|---|
| (State or other jurisdiction of incorporation or organization) | (I.R.S. Employer Identification No.) |
| **3535 General Atomics Court, Suite 200, San Diego, CA** | **92121** |
| (Address of principal executive offices) | (Zip Code) |

Registrant's telephone number, including area code:
**(858)-875-1800**

Securities registered pursuant to Section 12(b) of the Act:

| **Title of each class** | **Name of each exchange on which registered** |
|---|---|
| Common Stock, $0.001 par value | NASDAQ Global Market |

Securities registered pursuant to Section 12(g) of the Act: **None**

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes o or No ý

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. Yes o or No ý

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ý or No o

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Website, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§229.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes ý No o

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K is not contained herein, and will not be contained, to the best of registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K. ý

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, or a smaller reporting company. See the definitions of "large accelerated filer," "accelerated filer" and "smaller reporting company" in Rule 12b-2 of the Exchange Act. (Check one):

| Large accelerated filer o | Accelerated filer o | Non-accelerated filer o (Do not check if a smaller reporting company) | Smaller reporting company ý |
|---|---|---|---|

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Act). Yes o No ý

The registrant did not have a public float on the last business day of its most recently completed second fiscal quarter because there was no public market for the registrant's common equity as of such date.

The number of outstanding shares of the registrant's common stock, par value $0.001 per share, as of March 13, 2014 was 20,435,676.

**INCORPORATION BY REFERENCE**

Portions of the registrant's definitive proxy statement to be filed with the Securities and Exchange Commission, or SEC, pursuant to Regulation 14A in connection with the registrant's 2014 Annual Meeting of Stockholders, to be filed subsequent to the date hereof, are incorporated by reference into Part III of this annual report on Form 10-K. Such proxy statement will be filed with the SEC not later than 120 days after the conclusion of the registrant's fiscal year ended December 31, 2013.

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**FATE THERAPEUTICS, INC.**

**Annual Report on Form 10-K**

**For the Fiscal Year Ended December 31, 2013**

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**FORWARD-LOOKING STATEMENTS**

This Annual Report on Form 10-K contains "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933, as amended (the "Securities Act"), and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). Such forward-looking statements, which represent our intent, belief or current expectations, involve risks and uncertainties and other factors that could cause actual results and the timing of certain events to differ materially from future results expressed or implied by such forward-looking statements. In some cases, you can identify forward-looking statements by terms such as "may," "will," "expect," "anticipate," "estimate," "intend," "plan," "predict," "potential," "believe," "should" and similar expressions. Forward-looking statements in this Annual Report on Form 10-K include, but are not limited to, statements about:

- our projected timing of initiation, rate of enrollment and duration of our clinical trials for our product candidates;
- our plans to resume enrollment in our Phase 2 clinical trial, or to commence other clinical trials, of ProHema;
- our ability and our timing to incorporate the use of, and our ability to continue to use once incorporated, our nutrient-rich media, or 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;
- any review comments, or additional requirements, by FDA based upon our submission of ProHema manufacturing and product data generated using our NRM formulation with materials intended for clinical use;
- our expectations of safety and improved potency and efficacy of ProHema, arising from the use of our NRM formulation in the product's manufacture, in our Phase 2 clinical trial of ProHema in adults undergoing double UCBT, and in any subsequent clinical trials of ProHema;
- our plans to complete the preclinical development of, and to submit an Investigational New Drug, or IND, application for, and to conduct and generate data from the first clinical trials of, our Wnt7a analogs, and the timing of these activities;
- 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 preclinical studies and clinical trials and to manufacture our product candidates and 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 potential price and degree of market acceptance of stem cell-based therapeutics in general and our product candidates in particular;

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

regulatory developments and approval pathways in the United States and foreign countries for stem cell-based therapeutics in general and our product candidates in particular;

our ability to obtain, maintain, defend and enforce intellectual property rights protecting our product candidates, and our ability to develop and commercialize our product candidates without infringing the proprietary rights of third parties;

the accuracy of our estimates regarding revenues, expenses and capital requirements; and

the additional risks and other factors described under the caption "Risk Factors" under Item 1A of this Annual Report on Form 10-K.

*In this Annual Report on Form 10-K, unless the context requires otherwise, "Fate Therapeutics," "Company," "we," "our," and "us" means Fate Therapeutics, Inc. and its subsidiaries.*

## PART I

### ITEM 1. Business

**General Development of Our Business**

Fate Therapeutics, Inc., incorporated under the laws of the State of Delaware in April 2007, is a clinical-stage biopharmaceutical company engaged in the discovery and development of pharmacologic modulators of adult stem cells. Based on our understanding of key biological mechanisms that guide the fate of adult stem cells, we have built two platforms that optimize the activity and enhance the therapeutic potential of adult stem cells: our hematopoietic stem cell, or HSC, modulation platform and our muscle satellite stem cell, or Satellite Cell, modulation platform.

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 are pursuing the development of pharmacologically optimized HSC therapeutics for the treatment of hematologic malignancies and certain lysosomal storage disorders, or LSDs. In addition, we are pursuing the pharmacologic activation of muscle satellite stem cells using Wnt7a-based protein analogs, and we are initially focused on developing Wnt7a-based protein analogs for the treatment of muscular dystrophies. 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 |
| ***Satellite Cell 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.

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We plan to continue the validation of our two platforms by demonstrating the clinical benefit of our initial product candidates over the next two years in three orphan disease settings: hematologic malignancies, LSDs and muscular dystrophy. Our lead product candidate from our HSC modulation platform, ProHema, is presently undergoing Phase 2 clinical development for the treatment of adult patients with hematologic malignancies. We expect to generate full data on the primary and major secondary endpoints from this trial in mid-2015. We are also pursuing the development of ProHema for the treatment of pediatric patients with hematologic malignancies and certain demyelinating LSDs, and we plan to initiate our first clinical trials of ProHema in these clinical settings in 2014 with the goal of generating data from these trials in 2015. Our most advanced product candidates from our Satellite Cell modulation platform are Wnt7a protein analogs, which are presently undergoing IND-enabling development. We plan to initiate a Phase 1 clinical trial of an injectable analog of a Wnt7a-based recombinant human protein in 2015 with the goal of generating data from this clinical trial in 2015.

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.

**Description of Our Business**

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, or LSDs, 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, 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

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certain LSDs, where HSCs have demonstrated the ability to home, or migrate, to and engraft within the central nervous system, or CNS.

Our Satellite Cell modulation platform focuses on the *in vivo* pharmacologic activation of muscle satellite stem cells, which are adult stem cells that regenerate muscle throughout a person's lifespan. The regenerative capacity of satellite cells 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 satellite cells 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 satellite cell 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. Subject to the completion of IND-enabling studies and the filing of an IND application, we plan to initiate a Phase 1 clinical trial of an injectable analog of a Wnt7a-based recombinant human protein in 2015 with the goal of generating data from this clinical trial in 2015.

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 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.

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**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* Muscle Satellite Stem Cell Modulation**

We are applying our knowledge of stem cell modulation to develop novel biologic therapeutics based on the natural signals that stimulate muscle satellite stem cells *in vivo*. Our Satellite Cell 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 satellite cells can be pharmacologically modulated *in vivo* to improve muscle regeneration.

Our Satellite Cell 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 satellite cells and represent a novel and promising approach for the treatment of muscular dystrophies. The advantages of our approach include:

**Our means of satellite cell 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 Satellite Cell 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 satellite cell 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 satellite cell 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

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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.

**Our Satellite Cell 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 two 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 hematopoietic and muscle satellite stem cell modulation.

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

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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 annually 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% and 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,

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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.

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 human 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 sub-lethally irradiated NSG mouse model of HSCT:

**Homing** **Engraftment**

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.

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**Survival**

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* pharmacologically-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.

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***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 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 same 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;
- 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.

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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**

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**Rate and Incidence of Platelet Engraftment**

We also evaluated the incidence of GvHD and observed a low incidence of acute GvHD in the twelve patients 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 of patients either experienced graft failure or died prior to Day 100. At 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 75.0% 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 patient with known coronary artery disease experienced transient myocardial ischemia that resolved promptly after completion of the infusion.

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*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 in our Phase 2 clinical trial may not produce the efficacy or safety benefits that we currently expect; later-stage trials that enroll a larger number of patients 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 clinical potency and efficacy profile of ProHema may be significantly improved by using our new nutrient-rich media formulation, which we refer to as our NRM formulation, for clinical manufacture.

The manufacture of ProHema using our improved NRM formulation, as compared to the use of standard processing media, resulted in increased expression of PGE2-related genes and improved performance in *ex vivo* homing assays. In addition, the new manufacturing conditions also improved cell 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:

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

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These enhanced modulation effects using our improved NRM formulation, as compared to standard processing media, translated into significantly improved homing and a more than two-fold improvement in engraftment in mouse models, as shown in the graphs below:

**Homing** **Engraftment**

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 March 2014, we initiated enrollment of a randomized, controlled, Phase 2 multi-center clinical trial of ProHema using our NRM formulation in adult patients undergoing double UCBT for hematologic malignancies using both MAC and RIC regimens, which we refer to as our ProHema-03 trial. Our ProHema-03 trial using our NRM formulation is currently active, and has been approved for conduct at ten major allogeneic HSCT centers in the United States. The trial is expected to enroll 60 additional adult patients across both MAC and RIC regimens. 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. We expect to generate full data on the primary and major secondary endpoints from this trial in mid-2015.

In December 2012, we originally initiated the ProHema-03 trial using standard processing media for the manufacture of ProHema. In May 2013, we notified the FDA of our election to pause enrollment and our intent to generate data qualifying the optimized manufacturing process of ProHema using our NRM formulation. On August 1, 2013, we submitted to the FDA an amendment to our IND application, which contained preclinical and product development data supporting the use of our NRM formulation in the manufacture of ProHema and that its use should not result in additional safety risks. In addition, we submitted an amended protocol defining how we planned to resume the ProHema-03

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trial using our NRM formulation. Specifically, we stated that we planned to enroll the full 60 patients using our NRM formulation for the manufacture of ProHema, and that patients enrolled using standard processing media for the manufacture of ProHema prior to our election to pause enrollment would be followed and analyzed separately. In March 2014, we submitted to the FDA manufacturing and product data incorporating our NRM formulation for the manufacture of ProHema, where such data was generated using the NRM formulation intended for clinical use. Any FDA review or comments on this submission may lead the FDA to 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 in May 2013 to pause enrollment of our ProHema-03 trial to qualify the optimized manufacturing process of ProHema using our NRM formulation, 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 patients in the control arm engrafted at Days 30, 31 and 40, yielding a control median of 31 days. Five of the eight patients in the ProHema arm engrafted prior to the control median, at Days 14, 19, 24, 28 and 30. Two of the eight patients in the ProHema arm engrafted post the control median at Days 40 and 48, and one of the eight patients in the ProHema arm failed to engraft. Of the eight patients in the ProHema arm, six patients survived to Day 100 and two patients died before Day 100. The three patients in the control arm survived to Day 100. With a median overall follow-up for overall survival of 11.0 months, four of eight patients in the ProHema arm remain alive with a median survival that has not been reached but is greater than 9.0 months, as compared to one of three patients in the control arm who remain alive with a median survival of 6.0 months. No patients have experienced secondary graft failure. One patient in the ProHema arm experienced Grade IV acute GvHD, and one patient each in the ProHema and control arms experienced Grade III acute GvHD. Adverse events attributed to ProHema were primarily limited to common infusion-related side effects.

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 conducted 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 received the same RIC regimen that was used in our ProHema-01 trial. After conditioning, patients received a single ProHema cord blood unit. The primary endpoint of the trial was safety. We analyzed a range of engraftment measures as well as rates of GvHD, relapse and survival.

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The trial enrolled eight patients. Of the eight patients, six patients were evaluable, age 19-64 years (median 55.9 years), with the following diagnoses: acute myelogenous leukemia (four patients), myelodysplastic syndrome (one patient) and multiple myeloma (one patient). Four of the six evaluable patients engrafted at Days 17, 19, 22 and 37, and two experienced primary graft failure. Survival at 100 days was 100%. At a median follow-up of 10.2 months, no patients experienced secondary graft failure, and there was no reported acute or chronic GvHD. 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 for the manufacture of ProHema in this trial. In addition, we believe we can conduct our planned Phase 1b clinical trial in pediatric patients under our current IND application for ProHema, and thus we may be able to amend our existing IND application in order to commence this planned clinical trial. Although we currently believe that amending our existing IND application 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 application 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 that carries 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

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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 to and migrate across the blood-brain barrier into the CNS. We

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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:

**CNS Homing**

In additional follow-up experiments, 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. Eight weeks following injections, the number of human cells which had engrafted in the CNS were determined using qPCR against human specific sequences and the number of human iduronidase transcripts was measured using RT-qPCR. The figures below show that *ex vivo* modulation increases the number of donor-derived human cells engrafted in the CNS and results in higher numbers of iduronidase transcripts, which is the gene that is defective in patients with Hurler's syndrome:

**CNS Engraftment**

**Enzyme mRNA in CNS**

*Clinical Plan*

We plan to file an IND application in mid-2014 to initiate a first clinical trial of ProHema using our NRM formulation in pediatric patients with demyelinating LSDs later in 2014, with the goal of generating data from this trial in 2015. The primary objective of this trial is expected to evaluate the potential of *ex vivo* enhanced HSCs to enable robust engraftment under MAC and RIC regimens, where previous studies have shown that unmodulated cord blood units demonstrate higher rates of

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graft failure and longer times to engraftment as compared to their use in the treatment of hematologic malignancies. This trial is expected to enroll patients between the ages of one and 21 years. After conditioning, patients would receive a ProHema unit in combination with an unmodulated cord blood unit. The first cohort of patients would 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 would receive conditioning regimens that are successively dose-reduced. The primary endpoint of the study is expected to be neutrophil engraftment, such that a reduced intensity dosing regimen could be identified that results in consistent and prompt engraftment. Patients would also be followed for other measures of engraftment and safety. In addition, patients would 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 biological mechanisms that are critical to improving and enabling the curative potential of HSCT in patients with orphan hematologic malignancies and rare genetic disorders, including homing of HSCs to the CNS to improve delivery of essential enzymes which are deficient in patients with LSDs.

**Our Satellite Cell Modulation Platform**

***Therapeutic Potential of Muscle Satellite Stem Cells in Muscle Regeneration***

Skeletal muscle has a potent natural regenerative capacity. Muscle satellite stem cells, or satellite cells, 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, satellite cells 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 satellite cells *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 satellite cell activation and differentiation to affect a

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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 satellite cell 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 in preclinical studies that a single administration of a Wnt7a analog resulted in a significant expansion of the satellite cell 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 satellite cell 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.

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**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 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 additional therapeutic 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 satellite cells, 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 satellite cell 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 satellite cells, represented by an approximately three-fold increase in the number of Pax7 positive cell nuclei, a marker for satellite cells, in the treated

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muscle relative to the contralateral control. The figures below show our preclinical results demonstrating an increase in muscle hypertrophy and satellite cell population expansion:

**Muscle Hypertrophy**

**Satellite Cell Population Expansion**

*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 has been shown to increase muscle fiber integrity and reduce 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

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rodent *tibialis anterior* muscle. The figures below show these results comparing Wnt7a or a Wnt7a analog to formulation control:

**Specific Force Measurements**

*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 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 our preclinical assessments to include dose and regimen optimization in rodent models, and are currently conducting preliminary, non-GLP toxicology assessments with dose escalation, which are intended to inform future IND-enabling toxicology studies. We also plan to initiate efficacy and pharmacokinetic assessments in a well-characterized large animal model of muscular dystrophy to assess the effects in larger muscle groups. We believe these studies of larger muscle groups may allow 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, in an effort to accelerate our clinical development process. These include both cellular effects, such as muscle hypertrophy and satellite cell 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.

Subject to the completion of IND-enabling studies and the filing of an IND application, we plan to initiate a Phase 1 clinical trial of an injectable analog of a Wnt7a-based recombinant human protein candidate in healthy volunteers in 2015, with the goal of generating data from this clinical trial in 2015. The primary objective of this trial is expected to evaluate safety and dose of a product candidate locally-administered to a targeted muscle group. We also plan to assess biological activity in this trial using histological and gene expression pharmacodynamic markers and measures of muscle strength by electromyography. Based on the results of our Phase 1 clinical trial in healthy volunteers, we expect to conduct a clinical trial to assess safety and dose, and to demonstrate human proof-of-concept, of a

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product candidate administered locally to a targeted muscle group in X chromosome-linked dystrophy patients. We believe that the combination of a Phase 1 clinical trial in healthy volunteers, a dose escalation clinical trial in an X chromosome-linked muscular dystrophy population and the establishment of effective pharmacodynamic biomarkers would allow us to efficiently assess both safety and efficacy for an injectable analog of a Wnt7a-based recombinant human protein candidate. We also believe these studies would 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 satellite cell 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 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 currently 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

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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, to develop and 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, which concluded in September 2013, we co-developed certain stem cell reagent products with BD. BD has the right to commercialize these co-developed 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.

As of March 12, 2014, our intellectual property portfolio is currently composed of 91 issued patents and 180 patent applications that we license from academic and research institutions and 42 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 satellite cell 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 satellite cells.

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.

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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***

As of March12, 2014, we own six families of pending U.S. and foreign patent applications covering our HSC modulation platform. This portfolio includes 16 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 19 issued patents and 26 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

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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 Satellite Cell Modulation Platform and Wnt Analogs***

In support of our program for the modulation of satellite cells using Wnt analogs, we own patent applications pending in the United States and internationally 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 family of patent applications pending in the US and internationally 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 satellite cells, enhanced Wnt protein analogs, and the modulation of satellite cells 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 satellite cell 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.

***Intellectual Property Relating to iPSC Technology***

We own two patent families with applications pending in the US and internationally directed to our proprietary small molecule-enhanced cell culture system which enables large-scale iPSC culture expansion while maintaining high quality, homogeneous cells. These applications also cover methods for industrial-scale iPSC generation. Any patents issued from these applications will expire in 2031 or 2036.

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

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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 exclusive licenses 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 patent applications pending in the US and internationally 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

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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.

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 Satellite Cell 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.

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***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 Satellite Cell 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, 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.

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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 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 and 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.

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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, and import and export 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;

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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 application 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 sponsor of the IND application 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

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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 application 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 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.

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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 or components required for the manufacture 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.

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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

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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

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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 application (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

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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. 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.

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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.

***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 that are not independent of the influence of the supporting company. 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 is disclosed publicly by the FDA. Orphan designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

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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 Food and Drug Administration Safety and Innovation Act of 2012, or FDASIA, requires manufacturers of biological products that include a new active ingredient, new indication, new dosage form, new dosing regimen or new route of administration to submit a pediatric study plan to the FDA as part of the IND application. The plan must be submitted not later than 60 days after the end-of-Phase 2 meeting with the FDA; or if there is no such meeting, before the initiation of any Phase 3 trials or a combined Phase 2 and Phase 3 trial; or if a Phase 3 trial or combined Phase 2 and Phase 3 trial will not be conducted, no later than 210 days before a marketing application or supplement is submitted. 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.

***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

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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, beginning in August 2013, a similar federal requirement requires manufacturers to track and report to the federal government certain payments made to physicians and teaching 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.

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***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 December 31, 2013, we employed 37 full-time employees, including 19 in research and development, 10 in clinical development and eight 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.

**Corporate Information**

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 Annual Report on Form 10-K, and you should not consider any information on, or that can be accessed through, our website a part of this Annual Report on Form 10-K.

We own various U.S. federal trademark registrations and applications, and unregistered trademarks, including the following marks referred to in this document: Fate Therapeutics®, our corporate logo and ProHema®. All other trademarks or trade names referred to in this document are the property of their respective owners. Solely for convenience, the trademarks and trade names in this document 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.

On October 4, 2013, we completed an initial public offering, or IPO, whereby we sold 7,666,667 shares of our common stock at a public offering price of $6.00 per share. Gross proceeds from the offering were $46.0 million. After giving effect to underwriting discounts, commissions and other cash costs related to the offering, net proceeds were $40.5 million. In addition, each of the following occurred in connection with the completion of our IPO on October 4, 2013:

- the conversion of all outstanding shares of our convertible preferred stock into 7,229,590 shares of our common stock;
- the conversion of $22.1 million of outstanding principal and accrued interest on our convertible notes into 3,679,401 shares of our common stock and the cash repayment of $1.7 million of outstanding principal and accrued interest on our convertible notes;
- the write-off of $0.3 million of unamortized debt discount from the issuance of certain of our convertible notes;
- the issuance of 480,763 shares of our common stock pursuant to the redemption of an aggregate of 900,000 exchangeable shares of Fate Therapeutics (Canada) Inc., or Fate Canada, resulting in a final fair value adjustment charge of $0.4 million on the exchangeable shares, and the resultant reclassification of the exchangeable share liability to additional paid-in capital;

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the conversion of warrants to purchase 230,000 shares of convertible preferred stock into warrants to purchase 36,074 shares of the our common stock, and the resultant reclassification of the warrant liability to additional paid-in capital; and

the filing of an amended and restated certificate of incorporation on October 3, 2013, authorizing 150,000,000 shares of common stock and 5,000,000 shares of undesignated preferred stock.

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. 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) December 31, 2018; (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.

**Information About Segments and Geographic Areas**

In accordance with *The Financial Accounting Standards Board (or FASB) Accounting Standards Codification, or ASC, Topic 280, Segment Reporting,* we have determined that we operate as one operating segment. Decisions regarding our overall operating performance and allocation of our resources are assessed on a consolidated basis. Our operations and assets are predominantly located in the United States.

**Available Information**

We post our Annual Report on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K, and any amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended, on the Investors and Media section of our public website (www.fatetherapeutics.com) as soon as reasonably practicable after we electronically file such material with, or furnish it to, the SEC. In addition, you can read our SEC filings over the Internet at the SEC's website at www.sec.gov. The contents of these websites are not incorporated into this Annual Report on Form 10-K. Further, our references to the URLs for these websites are intended to be inactive textual references only. You may also read and copy any document we file with the SEC at its public reference facility at 100 F Street, N.E., Room 1580, Washington, D.C. 20549. Please call the SEC at 1-800-SEC-0330 for further information on the operation of the public reference facilities.

## ITEM 1A. Risk Factors

*You should carefully consider the following risk factors, as well as the other information in this report, and in our other public filings. The occurrence of any of the following risks could harm our business, financial condition, results of operations and/or growth prospects or cause our actual results to differ materially from those contained in forward-looking statements we have made in this report and those we may make from time to time. You should consider all of the risk factors described when evaluating our business.*

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**Risks Related to Our Limited Operating History and the Discovery, Development and Regulation of Our Product Candidates**

***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 $20.9 million, $14.2 million and $13.4 million for the years ended December 31, 2013, 2012, and 2011, respectively. As of December 31, 2013, we had an accumulated deficit of approximately $86.5 million. We expect to continue to incur losses for the foreseeable future, including in connection with our Phase 2 clinical trial of ProHema using our NRM formulation and our other preclinical and clinical development activities for ProHema and our Wnt7a analogs, 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, preclinical studies 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 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.

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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 progress, costs, results and timing of our Phase 2 clinical trial and planned Phase 1b clinical trials of ProHema;
- the progress, costs, results and timing of our additional preclinical studies and planned clinical trials of our Wnt7a analogs;
- our ability to initiate, and the size, progress, costs, results and timing of additional future clinical trials, including any registrational clinical trials for ProHema or for Wnt7a analogs, that will be necessary to support any application for regulatory approval of our product candidates; and
- 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.

Some of these factors are outside of our control. 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 twelve months. This period could be shortened if there are any significant or unanticipated increases in spending on development programs. In addition, our current cash position 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. 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.

***If we fail to complete preclinical development and clinical trials, obtain regulatory approval, or successfully commercialize our product candidates from our HSC and Satellite Cell 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, and 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

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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 preclinical studies and 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.

***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.***

In March 2014, we initiated enrollment of our ProHema-03 Phase 2 clinical trial using our NRM formulation in adult patients undergoing double UCBT for hematologic malignancies. We originally initiated the ProHema-03 trial in December 2012 using standard processing media for the manufacture of ProHema, and notified the FDA in May 2013 of our election to pause enrollment 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 supporting the use of our NRM formulation in the manufacture of ProHema and that its use should not result in additional safety risks. In addition, we submitted an amended protocol defining how we planned to resume the ProHema-03 trial using our NRM formulation. In March 2014, we submitted to the FDA manufacturing and product data incorporating our NRM formulation for the manufacture of ProHema, where such data was generated using the NRM formulation intended for clinical use. We expect the FDA to review this submission, and any FDA review comments on this submission may require us to generate additional preclinical or clinical data to support the use of our NRM formulation in our ProHema-03 trial or any other planned or subsequent clinical trials of ProHema, or may impose upon us additional requirements for our clinical development activities for ProHema. Additionally, the FDA may comment, or impose requirements, on the protocol for conducting our ProHema-03 trial. Additional comments, requirements or impositions by the FDA may cause delays in our ProHema-03 trial or other clinical development activities for ProHema, and could require us to incur additional development costs and resources, seek funding for these increased costs or resources or delay our timeline for, or cease, our clinical development activities for ProHema, or could create uncertainty and additional complexity in our ability to obtain regulatory approval for ProHema. Specifically, any comments, requirements or impositions by the FDA may cause delays in the availability of data from our ProHema-03 trial. Any inability to advance ProHema or any other product candidate through clinical development would have a material adverse effect on our business.

***We will need to file a new IND application to initiate our planned clinical trial of ProHema for the treatment of LSDs in pediatric patients and may be required to file a new IND application to initiate clinical trials of ProHema for the treatment of hematologic malignancies in pediatric patients.***

We plan to conduct clinical trials of ProHema for hematopoietic reconstitution in both hematologic malignancies and LSDs in pediatric patients. Based on feedback from the FDA, we will need to file a new IND application to evaluate ProHema for the treatment of LSDs in pediatric patients. We believe we can conduct our planned clinical trial of ProHema for the treatment of hematologic malignancies in pediatric patients under our current IND application for ProHema, and thus file the appropriate amendment in order to commence this planned clinical trial. Although we currently believe that this amendment will suffice, we will need to submit clinical development plans to the FDA before we can commence this trial. Our clinical development plans, including our plans to file a new IND application to evaluate ProHema for the treatment of LSDs in pediatric patients and to amend our current IND application to initiate a clinical trial of ProHema for the treatment of hematologic malignancies in pediatric patients, may change in response to a variety of factors, including the results of additional preclinical research and our discussions with key opinion leaders, regulatory authorities and/or third-

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party service providers, or the FDA may disagree with our plans and require us to file a new IND application for the clinical evaluation of ProHema for the treatment of pediatric patients with hematologic malignancies. If we change our product development plans or are required to file new IND applications, this may 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 hinder 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 our Wnt7a analogs, or we may otherwise observe undesirable safety or efficacy profiles in these product candidates as our development activities progress. Subject to our selection of a lead candidate, the completion of IND-enabling studies and our proposed pre-IND meeting with the FDA, we plan to file an IND application with the FDA and initiate a Phase 1 clinical trial of a Wnt7a analog. We may delay or cancel our ongoing and planned preclinical and clinical development activities for our Wnt7a analogs for a variety of reasons, including:

- the results of our ongoing or future preclinical studies or clinical trials may not support further development of, or require us to significantly modify our development plans for, our Wnt7a analogs;
- the FDA may require us to conduct additional preclinical studies or generate additional data before we are allowed to proceed with clinical development;
- the ability to manufacture Wnt7a analogs in a sufficient quantity or supply, or to formulate Wnt7a analogs in a suitable form for administration, for use in our planned preclinical studies or clinical trials;
- our ability to establish and maintain manufacturing relationships with third parties on acceptable terms, or to establish and maintain our own manufacturing capability, to produce and supply our Wnt7a analogs;
- our ability to reach a consensus with regulatory agencies on clinical trial design, or to reach agreement on acceptable terms with prospective clinical research organizations and clinical trial sites, or to obtain required Institutional Review Board approval at each clinical trial site, or to recruit suitable and sufficient numbers of patients, for the conduct of clinical trials for our Wnt7a analogs; or
- the occurrence of adverse events associated with our Wnt7a analogs in clinical trials that are viewed to outweigh their potential benefits.

Any delay in, or cessation of, our Wnt7a analog development activities 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 enroll patients in our ProHema-03 trial;

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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;

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;

the ability of third-party manufacturers to manufacture to the appropriate specifications the critical reagents necessary for the manufacture of ProHema;

the commercial availability of other materials necessary for the manufacture of ProHema;

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 in our clinical trials have serious, life-threatening diseases and 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 Satellite Cell modulation platforms prior to FDA or other regulatory approval. We may not have adequate capital or other resources to

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commence or complete these additional or larger trials. If we experience delays in the completion of any clinical trial of our product candidates or any of these clinical trials are terminated before completion, 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 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

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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 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;
- later-stage trials that enroll a larger number of patients 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 in 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, and the addition of a concurrent control arm;
- 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 1b 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.

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

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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 application or the filing of a new IND application and may present technical challenges and may cause further delays in our planned clinical trial. In addition, any such amendment to our current IND application or new IND application 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.

***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 Satellite Cell 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

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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.

***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 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.

***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 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.

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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.

**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 to complete clinical development.***

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, and may not be able to enter into, clinical supply agreements for certain of the components necessary to produce our NRM formulation and may therefore fail to secure an adequate and reliable supply of these components to complete our planned clinical development of ProHema through to commercialization. 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 planned clinical development of ProHema, including our 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

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formulation or a modified NRM formulation, which could delay our clinical development plans for ProHema and increase the costs required to complete our planned clinical development of ProHema.

***We rely on a single third-party supplier for the FT1050 starting material required for the manufacture of ProHema and depend on third-party suppliers for other components necessary for the manufacture of ProHema.***

To date, we have obtained our supplies of 16, 16-dimethyl prostaglandin E2, which we refer to as 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. Additionally, to date, we have acquired, or our clinical cell processing facilities have acquired, other equipment, materials and disposables necessary for the manufacture of ProHema from third parties. These materials include but are not limited to automated cell washing devices, automated cell warming units, commercially available media, cord blood transfer and wash sets, and other disposables. We do not have any current contractual relationships for the commercial supply of these materials for manufacturing ProHema and rely on their general commercial availability. Although we believe that we have alternate suppliers for FT1050 and the other components necessary for the manufacture of ProHema should our current third-party manufacturers or suppliers become unavailable to us for any reason, we may incur delays associated with identifying and qualifying replacement suppliers and negotiating the terms of any supply contracts with the replacement suppliers. 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 clinical cell processing facilities use in the manufacture of ProHema. We rely heavily on these third parties to procure 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, which 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.

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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 may adversely affect the safety and efficacy profile of ProHema or cause the FDA or other regulatory authorities to impose restrictions or prohibitions on the manufacture and use of ProHema in both the clinical and the commercial setting, which would 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

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comply with current GMPs and other applicable laws and regulations. We will have no control over the ability of these third parties to comply with these requirements, or to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or any other applicable regulatory authorities do not approve the facilities of these third parties for the manufacture of our other product candidates or any products that we may successfully develop, or if it withdraws any such 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 and preclinical studies. 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 expect to continue to rely upon third parties to monitor and manage data for our ongoing and planned 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 trials 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 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 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. We also rely on third parties to assist in conducting our preclinical studies, including IND-enabling studies, in accordance with good laboratory practices, or GLP. Failure by our third-party services providers to comply with applicable legal, regulatory or scientific standards in our clinical trials or our preclinical studies could negatively impact the results obtained in these trials or studies, and may require us to suspend or terminate ongoing preclinical studies or clinical trials, or repeat nonclinical and clinical trials or preclinical studies, which would harm our ability to complete the development of, and obtain regulatory approval for, our product candidates.

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 or preclinical data they obtain is compromised due to the failure to adhere to our clinical or preclinical protocols, regulatory requirements or for other reasons, our clinical trials and the development of our product candidates 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.

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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.

***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 and preclinical activities 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 16 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 12 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 satellite cells. 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.

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

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

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

- others may be able to 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 81 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.

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We currently have exclusive licenses to 32 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 satellite cells.

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.

***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.

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

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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.

***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

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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.

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

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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 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

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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;

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

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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

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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.

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 Satellite Cell 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 Satellite Cell 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.

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***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 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;

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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

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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.

***If we fail to maintain an effective system of disclosure controls and procedures and internal controls, our ability to produce accurate financial statements or comply with applicable regulations could be impaired.***

As a public company, we are required 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 and maintaining 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 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 will need to retain additional finance capabilities and build our financial infrastructure as we continue to operate as a public company, including complying with the requirements of Section 404 of the Sarbanes-Oxley Act and continuing to improve 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. Management oversight will be required as part of the process and as a result, management's attention may be diverted from other business concerns, which could harm our business and results of operations.

***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.

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***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;
- 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

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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

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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.

***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 Ownership of Our Common Stock**

***Market volatility may affect our stock price and the value of your investment.***

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 and preclinical studies;

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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 exercise significant control over our company.***

Based on shares outstanding as of February 17, 2014, our executive officers, directors and entities affiliated with our five percent stockholders beneficially own, in the aggregate, shares representing approximately 67% of our outstanding voting stock. Although we are not aware of any voting

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arrangements in place among these stockholders, 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.

***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. 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.

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

Approximately 12.4 million shares of our common stock will be eligible for sale by our shareholders in the public market upon the expiration of lock-up agreements in March 2014, 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, after March 2014, 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 our existing cash. We may not use our existing cash effectively, which could affect our results of operations and cause our stock price to decline.***

We will have considerable discretion in the application of our existing cash, including the net proceeds from our IPO. We expect to use our existing cash to fund research and development activities and for working capital and general corporate purposes, including funding the costs of operating as a public company. We may use these 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 our cash resources in a manner that does not produce income or that loses value.

***The requirements of being a public company may strain our resources and divert management's attention.***

As a public company, we have incurred, and will continue to incur, significant legal, insurance, accounting and other expenses that we did not incur as a private company. 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, impose various requirements on public companies, increasing legal and

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financial compliance costs and making some activities more time consuming. These laws, regulations and standards are subject to varying interpretations, and could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices. We have invested, and intend to continue to invest, resources to comply with new and evolving laws, regulations and standards applicable to public companies, 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. Further, stockholder activism in public companies, the political climate, and the risk of governmental or regulatory proceedings could make it more expensive for us to secure and maintain director and officer liability insurance, and we may be required to accept reduced coverage or incur substantially higher costs to maintain 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, we are 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

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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 "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 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) December 31, 2018; (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. 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.

***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.

***We currently do not intend to pay dividends on our common stock and, consequently, an investor's only opportunity to achieve a return on the investment is if the price of our common stock appreciates.***

We currently do not plan to declare dividends on shares of our common stock in the foreseeable future. Consequently, an investor's only opportunity to achieve a return on the investment in us will be if the market price of our common stock appreciates and the investor sells shares at a profit. There is no guarantee that the price of our common stock in the market will ever exceed the price that an investor paid.

***Our ability to use our net operating loss carryforwards may be subject to limitation and may result in increased future tax liability to us.***

Generally, a change of more than 50% in the ownership of a corporation's stock, by value, over a three-year period constitutes an ownership change for U.S. federal income tax purposes. An ownership change may limit a company's ability to use its net operating loss carryforwards attributable to the period prior to such change. We have not performed a detailed analysis to determine whether an ownership change under Section 382 of the Internal Revenue Code has occurred after each of our previous private placements of preferred stock or after the issuance of shares of common stock in connection with our initial public offering. In the event we have undergone an ownership change under Section 382, if we earn net taxable income, our ability to use our pre-change net operating loss carryforwards to offset U.S. federal taxable income may become subject to limitations, which could potentially result in increased future tax liability to us.

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**ITEM 1B. Unresolved Staff Comments**

Not applicable.

**ITEM 2. Properties**

Our administrative offices and research laboratory is located in San Diego, California. We occupy approximately 23,700 square feet of office and laboratory space under a lease that expires in 2016. We believe that our facilities are adequate for our current needs.

**ITEM 3. 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, 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.

**ITEM 4. Mine Safety Disclosures**

Not applicable.

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## PART II

### ITEM 5. Market for Registrant's Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities

**Market Information**

Our common stock began trading on The NASDAQ Global Market on October 1, 2013 and trades under the symbol "FATE". Prior to October 1, 2013, there was no public market for our common stock. The table below provides the high and low intra-day sales prices of our common stock for the period indicated, as reported by The NASDAQ Global Market.

| | High | Low |
|---|---|---|
| **Year ended December 31, 2013** | | |
| Fourth quarter | $ 9.19 | $ 4.30 |

**Holders of Common Stock**

As of March 14, 2014, there were approximately 98 stockholders of record of our common stock. The approximate number of holders is based upon the actual number of holders registered in our records at such date and excludes holders in "street name" or persons, partnerships, associations, corporations, or other entities identified in security positions listings maintained by depository trust companies.

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**Performance Graph**

Set forth below is a graph comparing the cumulative total return on an indexed basis of a $100 investment in the Company's common stock, the NASDAQ Composite® (US) Index and the NASDAQ Biotechnology Index commencing on October 1, 2013 (the date our common stock began trading on the NASDAQ Global Market) and continuing through December 31, 2013. The past performance of our common stock is no indication of future performance.

**Dividends**

We have never declared or paid any dividends on our capital or common 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.

**Securities Authorized for Issuance Under Equity Compensation Plans**

Information about our equity compensation plans is incorporated herein by reference to Item 12 of Part III of this Annual Report.

**Recent Sales of Unregistered Securities**

During the year ended December 31, 2013, we issued and sold the following unregistered securities (excluding those previously disclosed in a Quarterly Report on Form 10-Q or in a Current Report on Form 8-K):

In December 2013, we issued an aggregate of 76,922 shares of common stock to the former holders of exchangeable shares in our Canadian subsidiary, Fate Therapeutics (Canada) Inc., pursuant to a contractual obligation in connection with the achievement of a financial milestone event. These shares were deemed to be issued in a transaction exempt from registration

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pursuant to Section 4(2) of the Securities Act of 1933, as amended, as a transaction by an issuer not involving a public offering.

**Issuer Purchases of Equity Securities**

We did not repurchase any securities during the quarter ended December 31, 2013.

## ITEM 6. Selected Financial Data

*The following selected data should be read in conjunction with our financial statements located elsewhere in this Annual Report on Form 10-K and "Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations".*

| | Years Ended and as of December 31, | | |
|---|---|---|---|
| | **2013** | **2012** | **2011** |
| **Consolidated Statements of Operations Data (in thousands, except share and per share data):** | | | |
| Revenue: | | | |
| Collaboration revenue | $ 626 | $ 1,268 | $ 833 |
| Grant revenue | 345 | 1,402 | 337 |
| Total revenue | 971 | 2,670 | 1,170 |
| Operating expenses: | | | |
| Research and development | 12,007 | 11,999 | 9,858 |
| General and administrative | 6,639 | 4,228 | 4,605 |
| Total operating expenses | 18,646 | 16,227 | 14,463 |
| Loss from operations | (17,675) | (13,557) | (13,293) |
| Total other expense, net | (3,219) | (682) | (134) |
| Net loss and comprehensive loss | $ (20,894) | $ (14,239) | $ (13,427) |
| Net loss per common share, basic and diluted | $ (3.54) | $ (13.06) | $ (16.16) |
| Weighted-average common shares used to compute basic and diluted net loss per share | 5,896,171 | 1,090,317 | 830,959 |
| **Consolidated Balance Sheet Data (in thousands):** | | | |
| Cash and cash equivalents | $ 54,036 | $ 9,087 | $ 6,387 |
| Working capital | 50,051 | 4,943 | 3,013 |

| | | | |
|---|---|---|---|
| Total assets | 55,583 | 11,076 | 7,852 |
| Convertible notes, net of discount | | | 1,000 |
| Warrant liability | | 184 | 221 |
| Long-term debt, net of current portion | | 1,732 | 3,591 |
| Exchangeable share liability | | 551 | 563 |
| Convertible preferred stock | | 56,526 | 50,309 |
| Deficit accumulated during the development stage | (86,509) | (65,615) | (51,376) |
| Total stockholders' equity (deficit) | $ 50,848 | (52,825) | (50,683) |

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## ITEM 7. Management's Discussion and Analysis of Financial Condition and Results of Operations

*You should read the following discussion and analysis together with "Item 6. Selected Financial Data" and our financial statements and related notes included elsewhere in this Annual Report. The following discussion contains forward-looking statements that involve risks and uncertainties. Our actual results could differ materially from those expressed or implied in any forward-looking statements as a result of various factors, including those set forth under the caption "Item 1A. Risk Factors."*

### 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, or LSDs, 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 stem cell modulation platforms that optimize the activity of adult stem cells using both *ex vivo* and *in vivo* techniques: our HSC modulation platform and Satellite Cell modulation platform. 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.

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. To date, we have funded our operations primarily through the public sale of common stock, the private placement of preferred stock and convertible notes and through commercial bank debt. In October 2013, we completed our initial public offering, or IPO, whereby we received net proceeds of $40.5 million. In 2013 prior to the completion of our IPO, we issued convertible promissory notes in an aggregate principal amount of $23.7 million to certain existing stockholders and new investors. In connection with our IPO, $22.1 million of outstanding principal and accrued interest on the convertible promissory notes converted into shares of our common stock, and we repaid $1.7 million of outstanding principal and accrued interest on the convertible promissory notes in cash.

We have never been profitable and have incurred net losses in each year since inception. Our net losses were $20.9 million, $14.2 million, and $13.4 million for the years ended December 31, 2013, 2012, and 2011, 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. Our $20.9 million net loss for the year ended December 31, 2013 included $3.2 million of net non-operating expense. We expect to continue to incur 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;
- 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;

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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.

**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 Therapeutics (Canada) Inc., or Fate Canada, that were outstanding at December 31, 2013 and directs all of its operational activities, which are insignificant. 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 have been 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 received research funding for the conduct of joint development activities during the three-year period ending September 2013. We are eligible to receive certain commercialization milestones and royalties on the sale of any jointly-developed iPSC reagent products that are commercially launched. In connection with the arrangement with BD, we recognized $0.6 million, $1.3 million, and $0.8 million for the years ended December 31, 2013, 2012, and 2011, respectively, as collaboration revenue in our consolidated statements of operations. We do not currently anticipate generating any significant revenues under the arrangement with BD in the future.

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. No future revenues associated with the TATRC grant will be generated.

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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, including share-based compensation expense;
- costs associated with conducting our preclinical, clinical and regulatory activities, including fees paid to third-party professional consultants and service providers, clinical investigative sites and manufacturers of our preclinical study and clinical trial materials;
- costs for laboratory supplies;
- 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 December 31, 2013, we have incurred $57.0 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 LSDs to evaluate its safety and its curative potential in allogeneic HSCT; and
- conducting in 2014 IND-enabling studies 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 years ended December 31:

| **(in thousands)** | **2013** | **2012** | **2011** |
|---|---|---|---|
| HSC modulation platform | $ 4,980 | $ 5,869 | $ 3,084 |
| Other preclinical programs and technologies | 3,527 | 3,589 | 3,379 |
| Total direct research and development expenses | 8,507 | 9,458 | 6,463 |
| Unallocated expenses | 3,500 | 2,541 | 3,395 |

| | | | |
|---|---|---|---|
| Total research and development expenses | $ 12,007 | $ 11,999 | $ 9,858 |

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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; and change in fair value of the warrant liability relating to our preferred stock warrants, which were converted into common stock warrants in connection with our IPO.

**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 Annual Report, 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.

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*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.

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*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 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 contractual 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 has 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

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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 an adequate history of a public market for the trading of our common stock and a lack of adequate 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. See Note 5 of the Notes to the Consolidated Financial Statements for additional information.

Total stock-based compensation expense for the years ended December 31, 2013, 2012, and 2011, was $1.6 million, $0.2 million, and $0.2 million, respectively. Expense related to unvested employee stock option grants not yet recognized as of December 31, 2013 was approximately $1.9 million and the weighted-average period over which these grants are expected to vest is 2.9 years.

*Determination of the Fair Value of Common Stock*

Prior to our IPO, we were 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 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 prior to our IPO, on each grant date we developed 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 for grants made prior to our IPO 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;

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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 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 prior to our IPO.

There were significant judgments and estimates inherent in the determination of the fair value of our common stock. These judgments and estimates included assumptions made regarding our future operating performance, the time to completing an IPO or other liquidity events 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.

*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.

*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.

*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

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that the fair value of our common stock was $1.37 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 July 2012 valuation analysis and the dates of these stock option grants.

*March 2013 valuation.* The common stock fair value was estimated by our board of directors 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.

*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 and the date of these stock option grants.

*June 2013 valuation.* The common stock fair value was estimated by our board of directors 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 $1.63 per share utilized for the May 2013 option grants.

*August 2013 valuation and grants.* The common stock fair value 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 $4.49 per share utilized for the June 2013 valuation.

*Initial public offering price*

Our initial public offering price was $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 were classified as liabilities on the consolidated balance sheets at their estimated fair value since the underlying convertible preferred stock was classified as temporary equity. At the end of each reporting period or at the time of conversion to warrants to purchase shares of the Company's common stock, changes in the estimated fair value during the period were recorded as a component of other income (expense). The freestanding warrants for the purchase of convertible preferred stock were converted into warrants to purchase shares of the Company's common stock in connection with the completion of our IPO on October 4, 2013. After such date, we no longer adjust the fair value of the warrants. Prior to the completion of our IPO, we estimated the fair value 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.

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***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, were exchangeable for up to 884,605 shares of our common stock. Of these 884,605 shares of common stock, 480,763 became issuable upon the completion of the IPO, all of which were issued in common stock during the fourth quarter of 2013. The remaining 403,842 shares of common stock are now issuable in multiple increments upon the validation of certain scientific data and the achievement of certain preclinical, clinical and commercial milestones. The issuance of such shares is recorded as research and development expense based on the then-current fair value of our common stock at the date the milestone is achieved.

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.

Prior to the reclassification of the liability to equity as a result of the IPO, the fair value of all earned exchangeable shares was 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 was equal to the fair value of the number of shares of our common stock into which the exchangeable shares were exchangeable. During the fourth quarter of 2013, we recorded a charge of $0.4 million to adjust the exchangeable share liability to its then-current fair value upon the closing of our initial public offering, and reclassified the liability to stockholders' equity.

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 $0.3 million, $0.1 million, and $0.4 million related to increases in the number of shares of common stock issuable upon the exchange of the exchangeable shares of 76,922 shares, 57,691 shares and 207,688 shares, during the years ended December 31, 2013, 2012, and 2011, respectively. We recorded other income (expense) related to the change in fair value of the exchangeable shares for the years ended December 31, 2013, 2012, and 2011 of $2.4 million, $0.1 million, $(5,000), respectively.

**Other Company Information**

***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.

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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) December 31, 2018, (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.

**Recent Accounting Pronouncements**

In July 2013, the FASB issued guidance to provide clarity on the presentation of unrecognized tax benefits. An entity is required to present an unrecognized tax benefit, or a portion of an unrecognized tax benefit, as a reduction to a deferred tax asset for a net operating loss carryforward, a similar tax loss, or a tax credit carryforward, unless a net operating loss carryforward, a similar tax loss, or a tax credit carryforward is not available at the reporting date under the tax law of the applicable jurisdiction to settle any additional taxes that would result from the disallowance of a tax position or the tax law of the applicable jurisdiction does not require the entity to use, and the entity does not intend to use, the deferred tax asset for such purpose, in which cases, the unrecognized tax benefit should be presented in the financial statements as a liability, and should not be combined with deferred tax assets. The guidance is effective for fiscal years ending, and interim periods within those years, beginning after December 15, 2013, and is applied retrospectively. We do not expect the adoption of this guidance to have a material impact on our financial position or results of operations.

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.

**Results of Operations**

***Comparison of Years Ended December 31, 2013 and 2012***

The following table summarizes the results of our operations for the years ended December 31, 2013 and 2012:

| | Years Ended December 31, | |
|---|---|---|
| | 2013 | 2012 |
| | (in thousands) | |
| Collaboration revenue | $ 626 | $ 1,268 |
| Grant revenue | 345 | 1,402 |
| Research and development expenses | 12,007 | 11,999 |
| General and administrative expenses | 6,639 | 4,228 |
| Total other income (expense), net | (3,219) | (682) |
| | | 92 |

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*Revenue.* Total revenue was $1.0 million for the year ended December 31, 2013, compared to $2.7 million for the year ended December 31, 2012. The decrease of $1.7 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 under our license and collaboration agreement with BD. Our three-year joint development period under our agreement with BD concluded in September 2013, and we do not expect to generate any significant revenue under this agreement in the future.

*Research and development expenses.* Research and development expenses were $12.0 million for each of the years ended December 31, 2013 and 2012, with the following categorical changes:

- $1.3 million increase in employee compensation and benefits expense to support the clinical development of ProHema and the preclinical development of our other product candidates;
- $0.7 million increase in non-employee stock-based compensation expense; which were offset by
- $1.3 million decrease in third-party professional consultant and service provider expenses relating to the conduct of our ProHema-02 trial and the initiation of our ProHema-03 trial during 2012, including expenses to support clinical trial conduct, regulatory filings and other clinical start-up activities; and
- $0.8 million decrease in expenditures for laboratory supplies relating to the conduct of our ProHema-02 trial and the initiation of our ProHema-03 trial during 2012 and to the conduct of our preclinical programs.

*General and administrative expenses.* General and administrative expenses were $6.6 million for the year ended December 31, 2013, compared to $4.2 million for the year ended December 31, 2012. The increase of $2.4 million in general and administrative expenses primarily reflects the following:

- $0.8 million increase in employee compensation and benefits expense associated with the expansion of our executive management team;
- $0.8 million increase in third-party professional consultant and service provider expenses to support our financial and legal preparedness as a public company;
- $0.4 million increase in non-employee stock-based compensation expense; and
- $0.2 million increase in intellectual property related expenses.

*Other income (expense), net.* Other income (expense), net, was $(3.2) million for the year ended December 31, 2013, compared to $(0.7) million for the year ended December 31, 2012. The increase was primarily due to a $2.4 million increase in the fair value of the exchangeable share liability relating to the total exchangeable shares held by the former stockholders of Verio.

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***Comparison of Years Ended December 31, 2012 and 2011***

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

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

*Revenue.* Total 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 was 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:

$0.6 million increase in employee compensation-related expense in connection with an increase in headcount;

$2.4 million increase in external costs for professional consultants, clinical site start-up and clinical supply manufacture and regulatory activities for ProHema; which were partially offset by

$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:

$0.4 million decrease in employee-related costs;

$0.2 million decrease in intellectual property maintenance and prosecution costs; which were 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.

**Liquidity and Capital Resources**

We have incurred losses and negative cash flows from operations since inception. As of December 31, 2013, we had an accumulated deficit of $86.5 million and anticipate that we will continue to incur net losses for the foreseeable future.

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From our inception through December 31, 2013 we have funded our consolidated operations primarily through the public sale of common stock, the private placement of preferred stock and convertible notes, commercial bank debt and revenues from collaboration activities and grants. As of December 31, 2013, we had cash and cash equivalents of approximately $54.0 million. Our IPO on October 4, 2013 resulted in net proceeds of $40.5 million. We also repaid a total of $1.7 million in cash of outstanding principal and accrued interest on convertible notes in connection with the IPO.

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 December 31, 2013, the aggregate outstanding principal was $1.8 million.

The following table sets forth a summary of the net cash flow activity for each of the years ended December 31:

| | 2013 | 2012 | 2011 |
|---|---|---|---|
| | | (in thousands) | |
| Net cash used in operating activities | $ (15,373) | $ (13,274) | $ (12,145) |
| Net cash (used in) provided by investing activities | (238) | (709) | 200 |
| Net cash provided by financing activities | 60,560 | 16,683 | 7,107 |
| Net increase (decrease) in cash and cash equivalents | $ 44,949 | $ 2,700 | $ (4,838) |

***Operating Activities***

Cash used in operating activities increased $2.1 million from $13.3 million for the year ended December 31, 2012 to $15.4 million for the year ended December 31, 2013. The primary driver of operating cash requirements was our net loss in each period. During the year ended December 31, 2013, we used cash from operating activities of $15.4 million, while our net loss was $20.9 million. The difference was a result of $5.2 million of non-cash charges and deferrals, including depreciation expense, stock-based compensation expense and the change in fair value of our exchangeable shares, and by $0.3 million of net change in our operating assets and liabilities.

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.

***Investing Activities***

During the years ended December 31, 2013 and 2012, investing activities used cash of $0.2 million and $0.7 million, respectively, 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.

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***Financing Activities***

Financing activities provided cash of $60.6 million for the year ended December 31, 2013, primarily from net proceeds from our IPO of $40.5 million and $23.7 million of proceeds from the issuance of convertible promissory notes, offset by $2.0 million of principal payments on our long-term debt under our loan and security agreement and $1.7 million of payments on our convertible promissory notes.

Financing activities provided cash of $16.7 million during the year ended December 31, 2012 from the issuance of Series C convertible preferred stock. For the year ended December 31, 2011 the sale of $3.5 million of Series B convertible preferred stock, advances of $3.4 million under our loan and security agreement and the issuance of $1.0 million of a convertible note was offset by the pay-down of $0.8 million of principal under our loan and security agreement, generating $7.1 million from financing activities.

***Initial Public Offering and 2013 Convertible Note Financings***

On October 4, 2013, we completed our IPO, whereby we sold 7,666,667 shares of common stock at a public offering price of $6.00 per share. Gross proceeds from the offering were $46.0 million. After giving effect to underwriting discounts, commissions, and other cash costs related to the offering, net proceeds were $40.5 million.

In June and July 2013, we issued convertible promissory notes in an aggregate principal amount of $3.7 million to certain existing stockholders. In connection with the completion of our IPO on October 4, 2013, the outstanding principal and unpaid accrued interest on the notes converted to 625,828 shares of our common stock. The notes accrued interest at 2% per year.

In August 2013, we issued convertible promissory notes in an aggregate principal amount of $20.0 million to certain new investors. In connection with the completion of our IPO on October 4, 2013, we repaid $1.7 million of outstanding principal and unpaid accrued interest on the notes in cash, with the remaining outstanding principal converting to 3,053,573 shares of our common stock. The notes accrued interest at 2% per year.

***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. We have incurred, and expect to continue 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 our existing cash and cash equivalents will be sufficient to fund our projected operating requirements for at least the next twelve months. 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 our existing cash and cash equivalents. Additional capital may not be available on reasonable terms, if at

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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, duration, 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 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 continue or 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 at December 31, 2013 and the effect such obligations are expected to have on our liquidity and cash flows in future periods.

| (in thousands) | Total | Less than 1 Year | Years 1 - 3 | Years 3 - 5 | More than 5 Years |
|---|---|---|---|---|---|
| Long-term debt (including interest) | $ 2,011 | $ 2,011 | $ | $ | $ |
| Operating lease obligations | 2,334 | 911 | 1,423 | | |
| Total | $ 4,345 | $ 2,922 | $ 1,423 | $ | $ |

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 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

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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.

**ITEM 7A. Quantitative and Qualitative Disclosures About Market Risk**

We are exposed to market risk related to changes in interest rates. As of December 31, 2013, we had cash and cash equivalents of $54.0 million, including $52.3 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 cash equivalents are in short-term securities. Due to the short-term duration of our cash equivalents and the low risk profile of our investments, an immediate 100 basis point change in interest rates would not be expected to have a material effect on the fair market value of our portfolio.

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**ITEM 8. Financial Statements and Supplementary Data**

**Report of Independent Registered Public Accounting Firm**

The Board of Directors and Shareholders of Fate Therapeutics, Inc.

We have audited the accompanying consolidated balance sheets of Fate Therapeutics, Inc. as of December 31, 2013 and 2012, and the related consolidated statements of operations and comprehensive loss, convertible preferred stock and stockholders' equity (deficit), and cash flows for each of the three years in the period ended December 31, 2013, and the period from April 27, 2007 (inception) to December 31, 2013. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audits.

We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. We were not engaged to perform an audit of the Company's internal control over financial reporting. Our audits included consideration of internal control over financial reporting as a basis for designing audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion. An audit also includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, and evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the financial statements referred to above present fairly, in all material respects, the consolidated financial position of Fate Therapeutics, Inc. at December 31, 2013 and 2012, and the consolidated results of its operations and its cash flows for each of the three years in the period ended December 31, 2013 and for the period from April 27, 2007 (inception) to December 31, 2013, in conformity with U.S. generally accepted accounting principles.

/s/ ERNST & YOUNG LLP

San Diego, California

March 17, 2014

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**Fate Therapeutics, Inc.**
**(A Development Stage Company)**

**Consolidated Balance Sheets**

**(In thousands, except par value and share data)**

| | December 31, | |
|---|---|---|
| | **2013** | **2012** |
| **Assets** | | |
| Current assets: | | |
| Cash and cash equivalents | $ 54,036 | $ 9,087 |
| Prepaid expenses and other current assets | 615 | 706 |
| Total current assets | 54,651 | 9,793 |
| Property and equipment, net | 810 | 1,161 |
| Restricted cash | 122 | 122 |
| Total assets | $ 55,583 | $ 11,076 |
| **Liabilities, Convertible Preferred Stock and Stockholders' Equity (Deficit)** | | |
| Current liabilities: | | |
| Accounts payable | $ 682 | $ 1,228 |
| Accrued expenses | 2,039 | 1,040 |
| Current portion of deferred revenue | | 63 |
| Current portion of deferred rent | 53 | 251 |
| Repurchase liability for unvested equity awards | 94 | 143 |
| Preferred stock warrant liability | | 184 |
| Long-term debt, current portion | 1,732 | 1,941 |
| Total current liabilities | 4,600 | 4,850 |
| Deferred rent | 135 | 132 |
| Accrued expenses | | 110 |
| Exchangeable share liability | | 551 |
| Long-term debt, less current portion | | 1,732 |
| Commitments and contingencies (Note 4) | | |
| Convertible Preferred Stock, $0.001 par value; authorized shares zero at December 31, 2013 and 62,200,000 at December 31, 2012; issued and outstanding shares zero at December 31, 2013 and 44,967,690 at December 31, 2012 | | 56,526 |
| Stockholders' Equity (Deficit): | | |
| Preferred stock, $0.001 par value; authorized shares 5,000,000 at December 31, 2013 and zero at December 31, 2012; no shares issued or outstanding | | |
| Common stock, $0.001 par value; authorized shares 150,000,000 at December 31, 2013 and 100,000,000 at December 31, 2012; issued and outstanding 20,434,080 at December 31, 2013 and 1,334,115 at December 31, 2012 | 20 | 1 |
| Additional paid-in capital | 137,337 | 12,789 |
| Deficit accumulated during the development stage | (86,509) | (65,615) |

| | | |
|---|---|---|
| Total stockholders' equity (deficit) | 50,848 | (52,825) |
| Total liabilities, convertible preferred stock and stockholders' equity (deficit) | $ 55,583 | $ 11,076 |

See accompanying notes.

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**Fate Therapeutics, Inc.**
**(A Development Stage Company)**

**Consolidated Statements of Operations and Comprehensive Loss**

**(In thousands, except share and per share data)**

| | For the Years Ended December 31, | | | Period From April 27, 2007 (inception) to December 31, |
|---|---|---|---|---|
| | 2013 | 2012 | 2011 | 2013 |
| Revenue: | | | | |
| Collaboration revenue | $ 626 | $ 1,268 | $ 833 | $ 2,927 |
| Grant revenue | 345 | 1,402 | 337 | 2,084 |
| Total revenue | 971 | 2,670 | 1,170 | 5,011 |
| Operating expenses: | | | | |
| Research and development | 12,007 | 11,999 | 9,858 | 56,986 |
| General and administrative | 6,639 | 4,228 | 4,605 | 30,710 |
| Total operating expenses | 18,646 | 16,227 | 14,463 | 87,696 |
| Loss from operations | (17,675) | (13,557) | (13,293) | (82,685) |
| Other income (expense): | | | | |
| Interest income | 6 | 1 | 2 | 192 |
| Interest expense | (796) | (487) | (127) | (2,641) |
| Income from 48D tax credit | | | | 1,231 |
| Loss on extinguishment of debt | | (323) | (9) | (332) |
| Change in fair value of warrant liability | (8) | 37 | 5 | 35 |
| Change in fair value of exchangeable shares | (2,421) | 90 | (5) | (2,309) |
| Total other income (expense), net | (3,219) | (682) | (134) | (3,824) |
| Net loss and comprehensive loss | $ (20,894) | $ (14,239) | $ (13,427) | $ (86,509) |
| Net loss per common share, basic and diluted | $ (3.54) | $ (13.06) | $ (16.16) | |
| Weighted-average common shares used to compute basic and diluted net loss per share | 5,896,171 | 1,090,317 | 830,959 | |

See accompanying notes.

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**Fate Therapeutics, Inc.**
**(A Development Stage Company)**

**Consolidated Statements of Convertible Preferred Stock and Stockholders' Equity (Deficit)**

**(in thousands, except share data)**

| | Convertible Preferred Stock | | Common Stock | | Additional Paid-in Capital | Deficit Accumulated During the Development Stage | Total Stockholders' Equity (Deficit) |
|---|---|---|---|---|---|---|---|
| | Shares | Amount | Shares | Amount | | | |
| Balance, April 27, 2007 (inception) | | $ | | $ | $ | $ | $ |
| Issuance of common stock to founders and consultants at $0.001 per share for cash in September 2007 | | | 801,250 | 1 | 4 | | 5 |
| Issuance of Series A preferred stock for cash at $1.00 per share in September and November 2007 for cash, net of issuance costs of $115 | 1,655,435 | 1,541 | | | | | |
| Net loss | | | | | | (1,224) | (1,224) |
| Balance at December 31, 2007 | 1,655,435 | 1,541 | 801,250 | 1 | 4 | (1,224) | (1,219) |
| Repurchase of founders stock | | | (235,897) | | (2) | | (2) |
| Issuance of common stock to founders and consultants at $0.007 per share for cash | | | 420,323 | | 18 | | 18 |
| Issuance of Series A preferred stock for $1.00 per share in cash, net of issuance costs of $40 | 12,953,751 | 12,914 | | | | | |
| Exercise of stock options | | | 20,924 | | 1 | | 1 |
| Issuance of common stock for technology | | | 18,462 | | 1 | | 1 |
| Stock-based compensation | | | | | 17 | | 17 |
| Net loss | | | | | | (7,169) | (7,169) |
| Balance, December 31, 2008 | 14,609,186 | 14,455 | 1,025,062 | 1 | 39 | (8,393) | (8,353) |
| Repurchase of common stock | | | (64,866) | | (4) | | (4) |
| Issuance of Series B preferred stock for cash at $2.00 per share for cash and conversion of debt, net of issuance costs of $134 | 16,244,180 | 32,354 | | | | | |
| Exercise of stock options | | | 205,926 | | 21 | | 21 |
| Issuance of common stock for technology | | | 46,923 | | 17 | | 17 |
| Stock-based compensation | | | | | 196 | | 196 |
| Net loss | | | | | | (13,334) | (13,334) |
| Balance at December 31, 2009 | 30,853,366 | 46,809 | 1,213,045 | 1 | 269 | (21,727) | (21,457) |
| Repurchase of common stock | | | (4,808) | | | | |
| Exercise of stock options | | | 21,602 | | 10 | | 10 |
| Issuance of common stock for technology | | | 3,077 | | 5 | | 5 |
| Stock-based compensation | | | | | 229 | | 229 |
| Net loss | | | | | | (16,222) | (16,222) |
| Balance at December 31, 2010 | 30,853,366 | $ 46,809 | 1,232,916 | $ 1 | $ 513 | $ (37,949) | $ (37,435) |

See accompanying notes.

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**Fate Therapeutics, Inc.**
**(A Development Stage Company)**

**Consolidated Statements of Convertible Preferred Stock and Stockholders' Equity (Deficit) (Continued)**

**(in thousands, except share data)**

| | Convertible Preferred Stock | | Common Stock | | Additional Paid-in Capital | Deficit Accumulated During the Development Stage | Total Stockholders' Equity (Deficit) |
|---|---|---|---|---|---|---|---|
| | Shares | Amount | Shares | Amount | | | |
| Balance at December 31, 2010 | 30,853,366 | $ 46,809 | 1,232,916 | $ 1 | $ 513 | $ (37,949) | $ (37,435) |
| Repurchase of common stock | | | (126,872) | | (7) | | (7) |
| Exercise of stock options | | | 18,645 | | 14 | | 14 |
| Issuance of Series B preferred stock at $2.33 per share for cash | 1,500,000 | 3,500 | | | | | |
| Stock-based compensation | | | | | 172 | | 172 |
| Net loss | | | | | | (13,427) | (13,427) |
| Balance at December 31, 2011 | 32,353,366 | 50,309 | 1,124,689 | 1 | 692 | (51,376) | (50,683) |
| Exercise of stock options | | | 11,154 | | 15 | | 15 |
| Issuance of common stock at $0.25 per share for cash | | | 118,360 | | 192 | | 192 |
| Repurchase liability for unvested equity awards | | | | | (143) | | (143) |
| Issuance of common stock for technology | | | 15,385 | | 21 | | 21 |
| Conversion of preferred stock into common stock | (5,694,180) | (11,889) | 87,604 | | 11,889 | | 11,889 |
| Exchange of debt and common stock for Series B-1 preferred stock | 1,500,000 | 1,380 | (23,077) | | (32) | | (32) |
| Issuance of Series C preferred stock for cash at $1.00 per share, net of issuance costs of $84 | 16,808,504 | 16,726 | | | | | |
| Stock-based compensation | | | | | 155 | | 155 |
| Net loss | | | | | | (14,239) | (14,239) |
| Balance at December 31, 2012 | 44,967,690 | 56,526 | 1,334,115 | 1 | 12,789 | (65,615) | (52,825) |
| Exercise of stock options | | | 35,852 | | 23 | | 23 |
| Repurchase liability for unvested equity awards | | | | | 49 | | 49 |
| Stock-based compensation | | | | | 1,554 | | 1,554 |
| Issuance of common stock for technology | | | 7,692 | | 13 | | 13 |
| Beneficial conversion feature related to convertible notes | | | | | 336 | | 336 |
| Impact of initial public offering: | | | | | | | |
| Initial public offering of common stock, net of $5,520 of offering costs | | | 7,666,667 | 8 | 40,472 | | 40,480 |
| Conversion of convertible preferred stock into common stock | (44,967,690) | (56,526) | 7,229,590 | 7 | 56,519 | | 56,526 |
| Conversion of convertible notes into common stock | | | 3,679,401 | 4 | 22,072 | | 22,076 |
| Exchange of exchangeable shares into common stock | | | 480,763 | | 3,318 | | 3,318 |
| Warrant liability reclassification | | | | | 192 | | 192 |
| Net loss | | | | | | (20,894) | (20,894) |
| Balance at December 31, 2013 | | $ | 20,434,080 | $ 20 | $ 137,337 | $ (86,509) | $ 50,848 |

See accompanying notes.

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**Fate Therapeutics, Inc.**
**(A Development Stage Company)**

**Consolidated Statements of Cash Flows**

**(in thousands)**

| | Years Ended December 31, | | | Period From April 27, 2007 (inception) to December 31, |
|---|---|---|---|---|
| | 2013 | 2012 | 2011 | 2013 |
| **Cash flows from operating activities** | | | | |
| Net loss | $ (20,894) | $ (14,239) | $ (13,427) | $ (86,509) |
| Adjustments to reconcile net loss to net cash used in operating activities | | | | |
| Depreciation and amortization | 571 | 590 | 672 | 2,508 |
| Issuances of common stock for technology | 13 | 21 | | 57 |
| Stock-based compensation | 1,554 | 155 | 172 | 2,323 |
| Amortization of discounts | 394 | 84 | 23 | 535 |
| Noncash interest expense | 147 | 121 | 13 | 1,169 |
| Deferred rent | (195) | (197) | 228 | 188 |
| Deferred revenue | (63) | (83) | (646) | |
| Initial fair value and change in fair value of exchangeable shares | 2,767 | (12) | 356 | 3,318 |
| Change in fair value of preferred stock warrants | 8 | (37) | (5) | (35) |
| Loss on disposal of assets | 18 | | 117 | 135 |
| Loss on extinguishment of debt | | 323 | 9 | 332 |
| Changes in assets and liabilities: | | | | |
| Prepaid expenses and other assets | (1,271) | (405) | 209 | (1,977) |
| Accounts payable and accrued expenses | 1,578 | 405 | 134 | 3,847 |
| Net cash used in operating activities | (15,373) | (13,274) | (12,145) | (74,109) |
| **Cash flows from investing activities** | | | | |
| Purchase of property and equipment | (244) | (709) | (2) | (3,661) |
| Proceeds from sale of property and equipment | 6 | | 202 | 208 |
| Restricted cash | | | | (122) |
| Net cash (used in) provided by investing activities | (238) | (709) | 200 | (3,575) |
| **Cash flows from financing activities** | | | | |
| Issuance of common stock for cash | 23 | 207 | 14 | 294 |
| Repurchase of common stock for cash | | | (7) | (7) |
| Proceeds from initial public offering, net of offering costs | 40,480 | | | 40,480 |
| Issuance of convertible promissory notes | 23,736 | | 1,000 | 32,236 |
| Proceeds from long-term debt | | | 3,400 | 6,400 |
| Payments on long-term debt | (2,000) | (250) | (800) | (4,650) |
| Payment on convertible promissory note | (1,679) | | | (1,679) |
| Issuance of preferred stock for cash, net of offering costs | | 16,726 | 3,500 | 58,646 |
| Net cash provided by financing activities | 60,560 | 16,683 | 7,107 | 131,720 |
| Net increase (decrease) in cash and cash equivalents | 44,949 | 2,700 | (4,838) | 54,036 |
| Cash and cash equivalents, beginning of period | 9,087 | 6,387 | 11,225 | |
| Cash and cash equivalents, end of period | $ 54,036 | $ 9,087 | $ 6,387 | $ 54,036 |

| | | | | |
|---|---|---|---|---|
| **Supplemental disclosure of cash flow information** | | | | |
| Interest paid | $ 250 | $ 282 | $ 90 | $ 930 |
| **Supplemental schedule of noncash investing and financing activities** | | | | |
| Conversion of notes payable for Series B preferred stock | $ | $ | $ | $ 8,388 |
| Issuance of warrants in connection with long-term debt | $ | $ | $ 172 | $ 226 |
| Beneficial conversion feature related to convertible notes | $ 336 | $ | $ | $ 336 |
| Conversion of convertible preferred stock into common stock | $ 56,526 | $ | $ | $ 56,526 |
| Conversion of convertible notes into common stock | $ 22,076 | $ | $ | $ 22,076 |
| Exchange of exchangeable shares into common stock | $ 3,318 | $ | $ | $ 3,318 |
| Warrant liability reclassification to equity | $ 192 | $ | $ | $ 192 |

See accompanying notes.

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**Fate Therapeutics, Inc.**
**(A Development Stage Company)**

**Notes to Consolidated Financial Statements**

**1. Organization and Summary of Significant Accounting Policies**

**Organization and Basis of Presentation**

Fate Therapeutics, Inc. (the "Company") was incorporated in the state of Delaware on April 27, 2007 and has its principal operations in San Diego, California. The Company is a clinical-stage biopharmaceutical company engaged in the discovery and development of pharmacologic modulators of adult stem cells. Based on the Company's understanding of key biological mechanisms that guide the fate of adult stem cells, the Company has built two platforms that optimize the activity and enhance the therapeutic potential of adult stem cells: its HSC modulation platform and its Satellite Cell modulation platform.

As of December 31, 2013, the Company has devoted substantially all of its efforts to product development, raising capital and building infrastructure and has not generated revenues from its planned principal operations. Accordingly, the Company is considered to be in the development stage.

**Initial Public Offering**

On October 4, 2013, the Company completed its initial public offering (the IPO) whereby it sold 7,666,667 shares of common stock at a public offering price of $6.00 per share. Gross proceeds from the offering were $46.0 million. After giving effect to underwriting discounts, commissions, and other cash costs related to the offering, net proceeds were $40.5 million. In addition, each of the following occurred in connection with the completion of the IPO on October 4, 2013:

- the conversion of all outstanding shares of the Company's convertible preferred stock into 7,229,590 shares of the Company's common stock (see Note 5);

- the conversion of the Company's $22.1 million of outstanding principal and accrued interest on its convertible notes into 3,679,401 shares of common stock, the write-off of $0.3 million of unamortized debt discount and the related cash repayment of $1.7 million of outstanding principal and accrued interest on the convertible notes (see Note 4);

- the issuance of 480,763 shares of the Company's common stock pursuant to the redemption of an aggregate of 900,000 exchangeable shares of Fate Therapeutics (Canada) Inc. ("Fate Canada"), resulting in a final fair value adjustment charge of $0.4 million on the exchangeable shares, and the resultant reclassification of the exchangeable share liability to additional paid-in capital (see Note 2);

- the conversion of warrants to purchase 230,000 shares of convertible preferred stock into warrants to purchase 36,074 shares of the Company's common stock, and the resultant reclassification of the warrant liability to additional paid-in capital (as discussed below in Note 1); and

- the filing of an amended and restated certificate of incorporation on October 3, 2013, authorizing 150,000,000 shares of common stock and 5,000,000 shares of undesignated preferred stock (see Note 5).

**Use of Estimates**

The Company's consolidated financial statements are prepared in accordance with accounting principles generally accepted in the United States ("GAAP"). The preparation of the Company's

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**Fate Therapeutics, Inc.**
**(A Development Stage Company)**

**Notes to Consolidated Financial Statements (Continued)**

**1. Organization and Summary of Significant Accounting Policies (Continued)**

consolidated financial statements requires it to make estimates and assumptions that impact the reported amounts of assets, liabilities, revenues and expenses and the disclosure of contingent assets and liabilities in the Company's consolidated financial statements and accompanying notes. The most significant estimates in the Company's consolidated financial statements relate to the valuation of equity awards and clinical trial accruals. Although these estimates are based on the Company's knowledge of current events and actions it may undertake in the future, actual results may ultimately materially differ from these estimates and assumptions.

**Principles of Consolidation**

The consolidated financial statements include the accounts of the Company and its subsidiaries, Fate Therapeutics (Canada) Inc. ("Fate Canada"), Fate Therapeutics Ltd, incorporated in the United Kingdom, and Destin Therapeutics Inc., incorporated in Canada. To date, the aggregate operations of these subsidiaries have not been significant and all intercompany transactions and balances have been eliminated in consolidation.

**Segment Reporting**

Operating segments are identified as components of an enterprise about which separate discrete financial information is available for evaluation by the chief operating decision-maker in making decisions regarding resource allocation and assessing performance. The Company views its operations and manages its business in one operating segment.

**Fair Value of Financial Instruments**

The carrying amounts of accounts payable and accrued liabilities are considered to be representative of their respective fair values because of the short-term nature of those instruments. Based on the borrowing rates currently available to the Company for loans with similar terms, which is considered a Level 2 input, the Company believes that the fair value of long-term debt approximates its carrying value.

The accounting guidance defines fair value, establishes a consistent framework for measuring fair value and expands disclosure for each major asset and liability category measured at fair value on either a recurring or nonrecurring basis. Fair value is defined as an exit price, representing the amount that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants. As such, fair value is a market-based measurement that should be determined based on assumptions that market participants would use in pricing an asset or liability. As a basis for considering such assumptions, the accounting guidance establishes a three-tier fair value hierarchy, which prioritizes the inputs used in measuring fair value as follows:

Level 1: Observable inputs such as quoted prices in active markets;

Level 2: Inputs, other than the quoted prices in active markets, that are observable either directly or indirectly; and

Level 3: Unobservable inputs in which there is little or no market data, which require the reporting entity to develop its own assumptions

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**Fate Therapeutics, Inc.**
**(A Development Stage Company)**

**Notes to Consolidated Financial Statements (Continued)**

**1. Organization and Summary of Significant Accounting Policies (Continued)**

As of December 31, 2013 and 2012, the carrying amount of cash equivalents was $52.3 million and $1.3 million, respectively, which approximates fair value and was determined based upon Level 1 inputs. Cash equivalents primarily consisted of money market funds. As of December 31, 2013 and 2012, the Company did not hold any Level 2 or Level 3 financial assets that are recorded at fair value on a recurring basis.

Financial liabilities that were measured at fair value on a recurring basis include the preferred stock warrant liability and exchangeable shares for the period the liabilities were outstanding (see Note 2). None of the Company's non-financial assets or liabilities is recorded at fair value on a non-recurring basis. No transfers between levels have occurred during the periods presented.

As of December 31, 2013, the Company had no liabilities measured at fair value on a recurring basis. Liabilities measured at fair value on a recurring basis as of December 31, 2012 are as follows (in thousands):

| | | Fair Value Measurements at Reporting Date Using | | |
|---|---|---|---|---|
| | Total | Quoted Prices in Active Markets for Identical Assets (Level 1) | Significant Other Observable Inputs (Level 2) | Significant Unobservable Inputs (Level 3) |
| As of December 31, 2012: | | | | |
| Warrant liability | $ 184 | $ | $ | $ 184 |
| Exchangeable share liability | 551 | | | 551 |
| | $ 735 | $ | $ | $ 735 |

The preferred stock warrant liability was recorded at fair value using the Black-Scholes option pricing model and the exchangeable share liability was recorded at fair value based on the fair value of the underlying common stock. These liabilities were reclassified from liabilities to stockholders' equity as a result of the completion of the Company's IPO on October 4, 2013, which was the final fair value measurement date for each.

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 October 4, 2013 | As of December 31, 2012 |
|---|---|---|
| Risk-free interest rate | 2.1% | 1.2% |
| Expected volatility | 85.9% | 93.5% |
| Remaining contractual term (in years) | 7.55 | 8.31 |
| Expected dividend yield | 0.0% | 0.0% |

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**Fate Therapeutics, Inc.**
**(A Development Stage Company)**

**Notes to Consolidated Financial Statements (Continued)**

**1. Organization and Summary of Significant Accounting Policies (Continued)**

The following fair values per share of the Company's underlying convertible preferred stock and common stock were used to determine the fair value of the preferred stock warrant liability and the Exchangeable Shares (as defined in Note 2):

| | As of October 4, 2013 | As of December 31, 2012 |
|---|---|---|
| Series B convertible preferred stock | $ 6.90 | $ 0.92 |
| Series C convertible preferred stock | 6.90 | 0.99 |
| Common stock | 6.90 | 1.37 |

The fair value of the convertible preferred stock and common stock, prior to it being actively traded subsequent to the Company' IPO, was determined using a probability weighted expected return model. The key inputs into the model included the probability and timing of expected liquidity event dates, discount rates and the selection of appropriate market comparable transactions and multiples to apply to the Company's various historical and forecasted operational metrics.

The following table provides a reconciliation of all liabilities measured at fair value using Level 3 significant unobservable inputs (in thousands):

| | Warrant Liability | Exchangeable Share Liability |
|---|---|---|
| Balance at December 31, 2011 | $ 221 | $ 563 |
| Issuance of exchangeable shares | | 78 |
| Change in fair value | (37) | (90) |
| Balance at December 31, 2012 | 184 | 551 |
| Issuance of exchangeable shares | | 346 |
| Change in fair value | 8 | 2,421 |
| Transfer to stockholders' equity at fair value upon closing of IPO | (192) | (3,318) |
| Balance at December 31, 2013 | $ | $ |

**Cash and Cash Equivalents**

Cash and cash equivalents include cash in readily available checking and savings accounts, money market accounts and money market funds. The Company considers all highly liquid investments with an original maturity of three months or less from the date of purchase to be cash equivalents.

**Concentration of Credit Risk**

Financial instruments, which potentially subject the Company to significant concentration of credit risk, consist primarily of cash and cash equivalents. The Company maintains deposits in federally insured financial institutions in excess of federally insured limits. The Company has not experienced any losses in such accounts and management believes that the Company is not exposed to significant credit risk due to the financial position of the depository institutions in which those deposits are held.

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**Fate Therapeutics, Inc.**
**(A Development Stage Company)**

**Notes to Consolidated Financial Statements (Continued)**

**1. Organization and Summary of Significant Accounting Policies (Continued)**

**Property and Equipment**

Property and equipment are recorded at cost and depreciated using the straight-line method over the estimated useful lives of the assets (generally two to five years) and generally consist of furniture and fixtures, computers, scientific and office equipment. Repairs and maintenance costs are charged to expense as incurred.

**Impairment of Long-Lived Assets**

Long-lived assets consist primarily of property and equipment. An impairment loss is recorded if and when events and circumstances indicate that assets might be impaired and the undiscounted cash flows estimated to be generated by those assets are less than the carrying amount of those assets. While the Company's current and historical operating losses and negative cash flows are indicators of impairment, management believes that future cash flows to be received support the carrying value of its long-lived assets and, accordingly, has not recognized any impairment losses since inception.

**Other Assets**

Other assets capitalized during 2013 consisted of the Company's deferred IPO costs prior to the closing of the IPO on October 4, 2013. These costs represent legal, accounting and other direct costs related to the Company's efforts to raise capital through a public sale of its common stock. Upon closing of the IPO, the costs were reclassified to additional paid-in capital as a reduction of the IPO proceeds.

**Deferred Rent**

Deferred rent consists of the difference between cash payments and the recognition of rent expense on a straight-line basis for the facilities the Company occupies. The Company's lease for its facilities provides for fixed increases in minimum annual rental payments. The total amount of rental payments due over the lease term is being charged to rent expense ratably over the life of the lease.

**Preferred Stock Warrant Liability**

The Company historically issued freestanding warrants to purchase shares of its convertible preferred stock. The fair value of these warrants was classified as a current liability in the accompanying consolidated balance sheets since the underlying convertible preferred stock was classified as temporary equity in the accompanying consolidated balance sheets instead of in stockholders' equity (deficit) in accordance with authoritative guidance for the classification and measurement of potentially redeemable securities. Upon certain change in control events outside of the Company's control, including liquidation, sale or transfer of control of the Company, holders of the convertible preferred stock could have caused its redemption. The warrants were recorded at fair value using the Black-Scholes option pricing model with any changes in fair value being recognized as a component of other income (expense) in the accompanying consolidated statements of operations and comprehensive loss. Both the convertible preferred stock and the warrant liability were reclassified into stockholders' equity (deficit) as a result of the Company's IPO on October 4, 2013.

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**Fate Therapeutics, Inc.**
**(A Development Stage Company)**

**Notes to Consolidated Financial Statements (Continued)**

**1. Organization and Summary of Significant Accounting Policies (Continued)**

**Revenue Recognition**

The Company recognizes 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.

Revenue arrangements with multiple elements are analyzed to determine whether the elements can be divided into separate units of accounting or whether the elements must be accounted for as a single unit of accounting. The Company divides the elements into separate units of accounting and applies the applicable revenue recognition criteria to each of the elements, if the delivered elements have value to the customer on a stand-alone basis, if the arrangement includes a general right of return relative to the delivered elements, and if the delivery or performance of the undelivered elements is considered probable and substantially within the Company's control.

For transactions entered into prior to 2011, revenue was allocated to each element based on its relative fair value when objective and reliable evidence of fair value existed for all elements in an arrangement. If an element was sold on a stand-alone basis, the fair value of the element was the price charged for the element. When the Company was unable to establish fair value for delivered elements or when fair value of undelivered elements had not been established, revenue was deferred until all elements were delivered or until fair value could be objectively determined for any undelivered elements.

Beginning in 2011, revenue is allocated to each element at the inception of the arrangement using the relative selling price method that is based on a three-tier hierarchy. The relative selling price method requires that the estimated selling price for each element be based on vendor-specific objective evidence ("VSOE") of fair value, which represents the price charged for each element when it is sold separately or, for an element not yet being sold separately, the price established by management. When VSOE of fair value is not available, third-party evidence ("TPE") of fair value is acceptable, or a best estimate of selling price is used if neither VSOE nor TPE is available. A best estimate of selling price should be consistent with the objective of determining the price at which the Company would transact if the element were sold regularly on a stand-alone basis and should also take into account market conditions and company-specific factors. The Company has not entered into or materially modified any multiple element arrangements subsequent to 2010.

Revenue arrangements with multiple elements may include license fees, research and development payments, milestone payments, other contingent payments, and royalties on any product sales derived from collaborations. The Company recognizes nonrefundable license fees with stand-alone value as revenue at the time that the Company has satisfied all performance obligations, and recognizes license fees without stand-alone value as revenue in combination with any undelivered performance obligations. The Company recognizes a research and development payment as revenue over the term of the collaboration agreement as contracted amounts are earned, or reimbursable costs are incurred, under the agreement, where contracted amounts are considered to be earned in relative proportion to the performance required under the applicable agreement. The Company recognizes a milestone payment, which is contingent upon the achievement of a milestone in its entirety, as revenue in the period in which the milestone is achieved only if the milestone meets all criteria to be considered substantive. These criteria include the following: (i) the consideration being earned should be commensurate with either the Company's performance to achieve the milestone or the enhancement of

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**Fate Therapeutics, Inc.**
**(A Development Stage Company)**

**Notes to Consolidated Financial Statements (Continued)**

**1. Organization and Summary of Significant Accounting Policies (Continued)**

the value of the item delivered as a result of a specific outcome resulting from the Company's performance to achieve the milestone; (ii) the consideration being earned should relate solely to past performance; (iii) the consideration being earned should be reasonable relative to all deliverables and payment terms in the arrangement; and (iv) the milestone should be considered in its entirety and cannot be bifurcated into substantive and nonsubstantive components. Any amounts received pursuant to revenue arrangements with multiple elements prior to satisfying the Company's revenue recognition criteria are recorded as deferred revenue on the Company's consolidated balance sheets.

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.

**Research and Development Costs**

All research and development costs are expensed as incurred.

**Patent Costs**

Costs related to filing and pursuing patent applications are recorded as general and administrative expense and expensed as incurred since recoverability of such expenditures is uncertain.

**Stock-Based Compensation**

Stock-based compensation expense represents the cost of 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. The Company estimates 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.

The Company accounts 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.

**Income Taxes**

The Company accounts for income taxes under the asset and liability method, which requires the recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been included in the financial statements. Under this method, deferred tax assets and liabilities are determined on the basis of the differences between the financial statements and tax basis of assets

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**Fate Therapeutics, Inc.**
**(A Development Stage Company)**

**Notes to Consolidated Financial Statements (Continued)**

**1. Organization and Summary of Significant Accounting Policies (Continued)**

and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. The effect of a change in tax rates on deferred tax assets and liabilities is recognized in income in the period that includes the enactment date.

The Company recognizes net deferred tax assets to the extent that the Company believes these assets are more likely than not to be realized. In making such a determination, management considers all available positive and negative evidence, including future reversals of existing taxable temporary differences, projected future taxable income, tax-planning strategies, and results of recent operations. If management determines that the Company would be able to realize its deferred tax assets in the future in excess of their net recorded amount, management would make an adjustment to the deferred tax asset valuation allowance, which would reduce the provision for income taxes.

The Company records uncertain tax positions on the basis of a two-step process whereby (1) management determines whether it is more likely than not that the tax positions will be sustained on the basis of the technical merits of the position and (2) for those tax positions that meet the more-likely-than-not recognition threshold, management recognizes the largest amount of tax benefit that is more than 50 percent likely to be realized upon ultimate settlement with the related tax authority. The Company recognizes interest and penalties related to unrecognized tax benefits within income tax expense. Any accrued interest and penalties are included within the related tax liability.

**Comprehensive Loss**

Comprehensive loss is defined as a change in equity during a period from transactions and other events and circumstances from non-owner sources. Net loss and comprehensive loss were the same for all periods presented.

**Net Loss Per Common Share**

Basic net loss per common share is calculated by dividing the net loss by the weighted-average number of common shares outstanding for the period, without consideration for common stock equivalents. Excluded from the weighted-average number of shares outstanding are shares which have been issued upon the early exercise of stock options and are subject to future vesting and unvested restricted stock totaling 107,570 shares, 173,772 shares, and 303,253 shares for the years ended December 31, 2013, 2012, and 2011, respectively. Diluted net loss per common share is calculated by dividing the net loss by the weighted-average number of common share equivalents outstanding for the period determined using the treasury-stock method. Dilutive common stock equivalents are comprised of convertible preferred stock, warrants for the purchase of convertible preferred stock, exchangeable shares and options outstanding under the Company's stock option plans. For all periods presented, there is no difference in the number of common shares used to calculate basic and diluted common shares outstanding due to the Company's net loss position.

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**Fate Therapeutics, Inc.**
**(A Development Stage Company)**

**Notes to Consolidated Financial Statements (Continued)**

**1. Organization and Summary of Significant Accounting Policies (Continued)**

Potentially dilutive securities not included in the calculation of diluted net loss per common share because to do so would be anti-dilutive are as follows (in common stock equivalent shares):

| | As of December 31, | | |
|---|---|---|---|
| | **2013** | **2012** | **2011** |
| Convertible preferred stock outstanding | | 7,229,590 | 5,385,353 |
| Warrants for convertible preferred stock | | 36,074 | 36,074 |
| Warrants for common stock | 36,074 | | |
| Exchangeable shares | | 403,841 | 346,147 |
| Common stock options | 1,726,991 | 1,432,369 | 305,192 |
| | 1,763,065 | 9,101,874 | 6,072,766 |

The convertible preferred stock and exchangeable shares were converted into shares of the Company's stock as a result of the completion of the Company's IPO on October 4, 2013.

**2. Asset Acquisition of Verio Therapeutics Inc.**

Acquisitions are analyzed to determine whether an acquired set of activities and assets represents a business. A business is considered to be an integrated set of activities and assets that is capable of being conducted and managed for the purpose of providing a return in the form of dividends, lower costs, or other economic benefits directly to investors or other owners, members, or participants. A business commonly has three elements: inputs, processes applied to those inputs, and outputs. A set of activities and assets is required to have only the first two of those three elements, which together are or will be used to create outputs, to be considered a business. If an acquired set of activities and assets does not represent a business, the acquired set of activities and assets represents an asset.

On April 7, 2010, the Company acquired Verio Therapeutics Inc. ("Verio"), a development stage company headquartered in Ottawa, Ontario to gain access to its exclusively licensed intellectual property. Based on its evaluation of the set of activities and assets of Verio, the Company determined that Verio did not meet the definition of a business. Based on its assessment, the Company 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 Company accounted for the acquisition of Verio as an asset acquisition and charged the associated consideration paid for the assets to research and development expense.

In connection with the asset acquisition of Verio, the stockholders of Verio received 900,000 non-voting shares of Fate Canada (the "Exchangeable Shares") that were initially exchangeable into 138,462 shares of common stock of the Company, subject to increase upon the occurrence of certain events as described below, and the Company assumed $212,090 of net liabilities of Verio. The purchase price of the Verio asset acquisition is summarized as follows (in thousands):

| | |
|---|---|
| Net liabilities | $ 212 |
| Initial fair value of Exchangeable Shares | 234 |
| | $ 446 |

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**Fate Therapeutics, Inc.**
**(A Development Stage Company)**

**Notes to Consolidated Financial Statements (Continued)**

**2. Asset Acquisition of Verio Therapeutics Inc. (Continued)**

These amounts in the table above represent an estimate of the 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.

As a result of the Company's IPO on October 4, 2013, 480,763 shares of the Company's common stock were issued during the fourth quarter of 2013 pursuant to the redemption of the Exchangeable Shares. The total number of shares of the Company's common stock issued upon the exchange of the Exchangeable Shares as a result of the IPO was increased from 138,462 shares of the Company's common stock to a total of 480,763 shares of the Company's common stock based upon the achievement of certain contractual milestones.

As of December 31, 2013, the Company may issue an additional 403,842 shares of the Company's common stock based on the achievement of additional contractual milestones as follows: (i) 76,923 shares for the achievement of certain pre-clinical milestones, (ii) 211,538 shares for the achievement of certain clinical milestones and (iii) 115,381 shares for the achievement of certain commercialization milestones, such that the maximum aggregate number of shares of the Company's common stock that may be issued related to the Verio acquisition is 884,605.

At the date of the achievement of a milestone, the fair value of the additional shares is charged to research and development expense. Prior to the Company's IPO, at the end of each reporting period, any changes in the fair value of Exchangeable Shares resulting from changes in the fair value of the underlying common stock of the Company were recorded as a component of other income (expense). The Company recorded a non-cash charge of $0.4 million in other income (expense) related to the final fair value adjustment of the exchangeable share liability as of the IPO date, and reclassified the then-corresponding $3.3 million exchangeable share liability into additional paid-in capital. The Company recorded a non-cash charge of $2.4 million, $0.1 million and $(5,000) for the years ended December 31, 2013, 2012, and 2011, respectively, in other income (expense) related to the change in fair value of the exchangeable shares. Upon any future achievement of a milestone, the fair value will be recorded in additional paid-in capital.

The changes in the number of shares of the Company's common stock issuable upon the exchange of the Exchangeable Shares and the initial fair value of the shares are summarized as follows (in thousands, except share and per share amounts):

| | Exchangeable Shares | Fair Value Per Share of Underlying Common Stock | Initial Fair Value of Exchangeable Shares |
|---|---|---|---|
| April 2010 | 138,462 | $ 1.69 | $ 234 |
| March 2011 | 92,308 | 1.69 | 156 |
| May 2011 | 115,380 | 1.69 | 195 |
| April 2012 | 57,691 | 1.37 | 78 |
| July 2013 | 76,922 | 4.49 | 346 |
| Total | 480,763 | | $ 1,009 |

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**Fate Therapeutics, Inc.**
**(A Development Stage Company)**

**Notes to Consolidated Financial Statements (Continued)**

**3. Property and Equipment**

Property and equipment consist of the following (in thousands):

| | December 31, | |
|---|---|---|
| | 2013 | 2012 |
| Furniture and fixtures | $ 247 | $ 242 |
| Computer and office equipment | 123 | 129 |
| Software | 103 | 103 |
| Leasehold improvements building | 60 | |
| Scientific equipment | 2,573 | 2,436 |
| Property and equipment, gross | 3,106 | 2,910 |
| Less accumulated depreciation and amortization | (2,296) | (1,749) |
| Property and equipment, net | $ 810 | $ 1,161 |

Depreciation expense related to property and equipment was $0.6 million, $0.6 million, and $0.7 million, for the years ended December 31, 2013, 2012, and 2011, respectively, and $2.5 million for the period from April 27, 2007 (inception) to December 31, 2013. In connection with the renovation of its laboratory space, the Company sold certain equipment that was no longer relevant to its operations. The Company recognized a loss on the disposal of $0.1 million for the year ended December 31, 2011, which is included in general and administrative expense. No material gains or losses on the disposal of property and equipment have been recorded for the years ended December 31, 2013 and 2012.

**4. Long-Term Debt, Commitments and Contingencies**

**Long-Term Debt**

Long-term debt and unamortized discount balances (excluding convertible debt) are as follows (in thousands):

| | December 31, | |
|---|---|---|
| | 2013 | 2012 |
| Long-term debt | $ 1,750 | $ 3,750 |
| Less debt discount, net of current portion | | (18) |
| Long-term debt, net of discount | 1,750 | 3,732 |
| Less current portion of long-term debt | (1,750) | (2,000) |
| Long-term debt, net of current portion | $ | $ 1,732 |

| | | |
|---|---|---|
| Current portion of long-term debt | $ 1,750 | $ 2,000 |
| Current portion of debt discount | (18) | (59) |
| Current portion of long-term debt, net | $ 1,732 | $ 1,941 |

In 2009, the Company entered into a $3.0 million loan and security agreement collateralized by substantially all of the Company's assets, excluding certain intellectual property. The Company drew the full $3.0 million available under the loan and security agreement in 2009 and issued a fully exercisable

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**Fate Therapeutics, Inc.**
**(A Development Stage Company)**

**Notes to Consolidated Financial Statements (Continued)**

**4. Long-Term Debt, Commitments and Contingencies (Continued)**

warrant for 30,000 shares of the Company's Series B convertible preferred stock at an exercise price of $2.00 per share.

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. In August 2011, the Company issued the lender a warrant that provided $0.2 million of warrant coverage for either (i) 100,000 shares of Series B convertible preferred stock at an exercise price of $2.00 per share or (ii) a number of shares and exercise price to be determined based on the pricing of a subsequent qualified financing. In May 2012, as a result of the Company's Series C qualified financing, the warrant became exercisable for 200,000 shares of the Company's Series C convertible preferred stock at an exercise price of $1.00 per share.

The Company 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 (together, the "Term Advances"), 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% for their entire 36-month term of the debt. As of December 31, 2013, the aggregate outstanding principal was $1.8 million.

The initial fair values of the warrants issued in 2009 and 2011 were $0.1 million and $0.2 million, respectively, and recorded as a debt discount and amortized to interest expense over the term of the related loans on the effective interest method. The initial fair values of the warrants were estimated using the Black-Scholes option pricing model.

Upon closing of the Company's IPO on October 4, 2013, the warrants to purchase convertible preferred stock converted into warrants to purchase 36,074 shares of the Company's common stock, and the then fair value of $0.2 million of the warrant liability was reclassified to additional paid-in capital. The weighted average exercise price of the warrants is $7.21 per share. Warrants to purchase 5,305 and 30,769 shares of the Company's common stock expire in January 2019 and August 2021, respectively, or earlier upon the acquisition of the Company.

**June and July 2013 Convertible Note Financing**

In June and July 2013, the Company issued convertible promissory notes in an aggregate principal amount of $3.7 million to certain existing stockholders. The notes accrued interest at 2% per year and were due on June 24, 2014. The principal balance and all accrued and unpaid interest due on the notes were converted into 625,828 shares of the Company's common stock as a result of the Company's IPO on October 4, 2013.

In connection with the issuance of the convertible notes, the Company recorded a debt discount of $0.3 million related to a beneficial conversion feature that was recorded as the proceeds allocated to the debt instrument were less than the gross fair value of the shares of Series C convertible preferred stock into which the notes could convert. This debt discount was amortized as interest expense utilizing the effective interest method over the one-year term of the notes. During the year ended December 31,

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**Fate Therapeutics, Inc.**
**(A Development Stage Company)**

**Notes to Consolidated Financial Statements (Continued)**

**4. Long-Term Debt, Commitments and Contingencies (Continued)**

2013, the entire $0.3 million debt discount was charged to interest expense in connection with its amortization during the period for which the notes were outstanding and the conversion of the notes into common stock pursuant to the Company's IPO on October 4, 2013.

**August 2013 Convertible Note Financing**

In August 2013, the Company issued convertible promissory notes in an aggregate principal amount of $20.0 million to certain new investors. The notes accrued interest at 2% per year and were due on August 8, 2016. In connection with the completion of our IPO on October 4, 2013, the Company repaid $1.7 million of then-outstanding principal and unpaid accrued interest in cash, with the remaining outstanding principal converting to 3,053,573 shares of the Company's common stock. During the year ended December 31, 2013, the Company recorded aggregate interest expense of $0.1 million on the stated interest rate of the notes issued in August 2013.

**Facility Lease**

The Company leases certain office and laboratory space from a stockholder of the Company under a non-cancelable operating lease. In September 2013, the Company exercised its option to extend the lease for two years. The lease expires in June 2016. The lease is subject to additional charges for common area maintenance and other costs. In connection with the lease, the Company entered into a cash-collateralized irrevocable standby letter of credit in the amount of $0.1 million. Rent expense was $0.9 million, $0.7 million, $0.7 million, and $3.8 million for the years ended December 31, 2013, 2012, and 2011 and the period from April 27, 2007 (inception) to December 31, 2013, respectively.

**License Agreements**

The Company has entered into exclusive license agreements with certain academic institutions and universities pursuant to which the Company acquired certain intellectual property. Pursuant to each agreement, as consideration for an exclusive license to the intellectual property, the Company paid a license fee, reimbursed the institution for historical patent costs and, in certain instances, issued the institution shares of restricted common stock. Additionally, under each agreement, the institution is generally eligible to receive future consideration including, but not limited to, annual maintenance fees, royalties, milestone payments and sublicensing fees. Each of the license agreements is generally cancelable by the Company, given appropriate prior written notice. Minimum annual payments to maintain these cancelable licenses total an aggregate of $0.3 million.

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**Fate Therapeutics, Inc.**
**(A Development Stage Company)**

**Notes to Consolidated Financial Statements (Continued)**

**4. Long-Term Debt, Commitments and Contingencies (Continued)**

In connection with the above license agreements, the Company has issued an aggregate of 91,539 shares of common stock and recorded the aggregate fair value of $57,000 as research and development expense. The share issuances are summarized as follows (in thousands, except share amounts):

| | Shares of Common Stock | Fair Value of Common Shares |
|---|---|---|
| 2008 | 18,462 | $ 1 |
| 2009 | 46,923 | 17 |
| 2010 | 3,077 | 5 |
| 2012 | 15,385 | 21 |
| 2013 | 7,692 | 13 |
| Total | 91,539 | $ 57 |

**Commitments**

Future minimum payments under the long-term debt and the non-cancelable operating lease as of December 31, 2013 are as follows (in thousands):

| | Long-Term Debt | Operating Lease | Total |
|---|---|---|---|
| Years Ending December 31, | | | |
| 2014 | $ 2,011 | $ 911 | $ 2,922 |
| 2015 | | 943 | 943 |
| 2016 | | 480 | 480 |
| Total | 2,011 | $ 2,334 | $ 4,345 |
| Less interest | (61) | | |
| Less additional payments due upon maturity | (200) | | |
| Less current portion of long-term debt | (1,750) | | |
| Long-term debt, net of current portion | $ | | |

**5. Convertible Preferred Stock and Stockholders' Equity (Deficit)**

**Reverse Stock Split**

On September 12, 2013, the Company filed an amendment to its amended and restated certificate of incorporation, effecting a one-for-6.5 reverse stock split of the Company's issued and outstanding shares of common stock. All issued and outstanding common stock and per share amounts contained in the Company's consolidated financial statements have been retroactively adjusted to reflect this reverse stock split for all periods presented.

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**Fate Therapeutics, Inc.**
**(A Development Stage Company)**

**Notes to Consolidated Financial Statements (Continued)**

**5. Convertible Preferred Stock and Stockholders' Equity (Deficit) (Continued)**

**Convertible Preferred Stock**

The authorized, issued and outstanding shares of convertible preferred stock by series immediately prior to October 4, 2013 (the date each convertible preferred outstanding share was converted to common stock as a result of the Company's IPO) is as follows:

| | Shares Authorized | Shares Outstanding |
|---|---|---|
| Series A | 14,609,186 | 14,609,186 |
| Series B | 12,080,000 | 12,050,000 |
| Series B-1 | 1,500,000 | 1,500,000 |
| Series C | 29,000,000 | 16,808,504 |
| Series C-1 | 11,171,000 | |
| | 68,360,186 | 44,967,690 |

In connection with the completion of the Company's IPO on October 4, 2013, all of the outstanding shares of convertible preferred stock converted into 7,229,590 shares of the Company's common stock. Each outstanding share of Series A and Series C convertible preferred stock converted into approximately 0.1538 shares of common stock, or 4,833,490 common shares, and each outstanding share of Series B and Series B-1 convertible preferred stock converted into approximately 0.1768 shares of common stock, or 2,396,100 common shares.

**Description of Securities**

*Dividends*

As of December 31, 2013, the Board of Directors of the Company has not declared any dividends.

**Convertible Preferred Stock and Related Transactions**

The Company sold 1,655,435 shares and 12,953,751 shares of Series A convertible preferred stock at $1.00 per share in 2007 and 2008, respectively.

In 2009, the Company sold 12,050,000 shares of Series B convertible preferred stock at $2.00 per share for $24.1 million in cash and sold 4,194,180 shares of Series B convertible preferred stock in exchange for the conversion of $7.5 million of convertible notes payable and $0.9 million of related accrued interest. The convertible notes payable were originally issued in April 2008 and accrued interest at a rate of 7.5% per annum.

In March 2011, the Company sold Takeda Ventures, Inc. ("Takeda") 1,500,000 shares of Series B convertible preferred stock at $2.33 per share for $3,500,000 in cash. Also in March 2011, the Company entered into a subordinated note purchase agreement (the "Note Purchase Agreement") with Takeda. Pursuant to such Note Purchase Agreement, the Company sold and issued Takeda one general, unsecured, subordinated note (the "Subordinated Note"). Such Subordinated Note had a principal amount of $1,000,000 and accrued interest at a rate of 2.0% per annum.

Pursuant to the terms of the Series C financing described below, the 1,500,000 shares of Series B convertible preferred stock purchased by Takeda in March 2011 were converted into 150,000 shares of

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**Fate Therapeutics, Inc.**
**(A Development Stage Company)**

**Notes to Consolidated Financial Statements (Continued)**

**5. Convertible Preferred Stock and Stockholders' Equity (Deficit) (Continued)**

common stock in July 2012. In May 2012, the Company and Takeda entered into a letter agreement pursuant to which, effective in July 2012, Takeda forfeited all of its rights and forgave all of the obligations of the Company under the Note Purchase Agreement in exchange for the Company converting Takeda's 23,077 shares of common stock into 1,500,000 shares of Series B-1 convertible preferred stock. As a result of the exchange of: (i) $1.0 million of debt and related $25,000 of accrued interest and (ii) 23,077 shares of common stock with a fair value of approximately $32,000 for 1,500,000 shares of Series B-1 convertible preferred stock with a fair value of approximately $1.4 million, the Company recorded a loss on exchange of approximately $0.3 million.

On May 4, 2012, the Company entered into a Series C stock purchase agreement pursuant to which it sold 6,120,369 shares of Series C convertible preferred stock for cash at $1.00 per share in May 2012 and sold 3,124,310 shares of Series C convertible preferred stock for cash at $1.00 per share in July 2012 (together, the "First Closing"). The Company sold an additional 7,563,825 shares of Series C convertible preferred stock for cash at $1.00 per share in a second closing in October 2012.

Pursuant to the Series C stock purchase agreement, certain non-participating stockholders' holdings of preferred stock were converted into common stock on a basis of one share of common stock for each 65 shares of preferred stock. As a result, in addition to Takeda, one investor had its 4,194,180 shares of Series B convertible preferred stock converted into 64,527 shares of common stock upon the First Closing.

**2007 Equity Incentive Plan and 2013 Stock Option and Incentive Plan**

The Company adopted an Equity Incentive Plan in 2007 (the 2007 Plan) under which, as amended in August 2013, 2,423,072 shares of common stock were reserved for issuance to employees, nonemployee directors and consultants of the Company. The 2007 Plan provides for the grant of incentive stock options, nonstatutory stock options, rights to purchase restricted stock, stock appreciation rights, dividend equivalents, stock payments, and restricted stock units to eligible recipients. In connection with the issuance of restricted common stock, the Company maintains a repurchase right where shares of restricted common stock are released from such repurchase right over a period of time of continued service by the recipient. Effective upon the completion of the Company's IPO, the board of directors determined not to grant any further awards under the 2007 Plan.

On August 28, 2013, the Company's board of directors and stockholders approved and adopted the 2013 Stock Option and Incentive Plan (the "2013 Plan" and collectively with the 2007 Plan "the Plans"). The 2013 Plan became effective immediately prior to the Company's IPO. Under the 2013 Plan, the Company may grant stock options, stock appreciation rights, restricted stock, restricted stock units and other awards to individuals who are then employees, officers, directors or consultants of the Company or its subsidiaries. A total of 1,020,000 shares of common stock were initially reserved for issuance under the 2013 Plan. 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.

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**Fate Therapeutics, Inc.**
**(A Development Stage Company)**

**Notes to Consolidated Financial Statements (Continued)**

**5. Convertible Preferred Stock and Stockholders' Equity (Deficit) (Continued)**

In addition, the number of shares of stock available for issuance under the 2013 Plan will be automatically increased each January 1, beginning on January 1, 2014, by 4% of the outstanding number of shares of the Company's common stock on the immediately preceding December 31 or such lesser number as determined by the compensation committee of the Company's board of directors.

Recipients of incentive stock options under the Plans shall be eligible to purchase shares of the Company's common stock at an exercise price equal to no less than the estimated fair value of such stock on the date of grant. Under the Plans, stock options generally vest 25% on the first anniversary of the original vesting date, with the balance vesting monthly over the remaining three years, or vest monthly over four years, unless they contain specific performance and/or market-based vesting provisions. The maximum term of stock options granted under the Plans is ten years.

**Employee Stock Purchase Plan**

On September 13, 2013, the Company's board of directors approved and adopted the 2013 Employee Stock Purchase Plan (the "ESPP"). The ESPP became effective immediately prior to the completion of the IPO. A total of 729,000 shares of common stock initially reserved for issuance under the ESPP. In addition, the number of shares of stock available for issuance under the ESPP will be automatically increased each January 1, beginning on January 1, 2015, by the lesser of (i) 2% of the outstanding number of shares of the Company's common stock on the immediately preceding December 31, (ii) 450,000 shares, or (iii) such lesser number as determined by the compensation committee of the Company's board of directors.

No purchases were made under the ESPP during the year ended December 31, 2013.

**Stock Options and Restricted Stock Awards**

The following table summarizes stock option activity and related information under the Plans for the year ended December 31, 2013:

| | Options | Weighted Average Exercise Price Per Share | Weighted Average Remaining Contractual Term | Aggregate Intrinsic Value (in 000s) |
|---|---|---|---|---|
| Outstanding at December 31, 2012 | 1,432,369 | $ 1.43 | 9.14 | $ 51 |
| Granted | 355,780 | 5.60 | | |
| Exercised | (35,853) | 0.65 | | |
| Cancelled | (25,305) | 2.24 | | |
| Outstanding at December 31, 2013 | 1,726,991 | $ 2.30 | 8.47 | $ 7,203 |
| Options vested and expected to vest at December 31, 2013 | 1,555,091 | $ 1.92 | 8.37 | $ 6,935 |
| Options exercisable at December 31, 2013 | 668,667 | $ 1.52 | 7.89 | $ 3,190 |

As of December 31, 2013 and 2012, the outstanding options included 174,730 and 272,001, respectively, of performance-based options for which the achievement of the performance-based vesting

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**Fate Therapeutics, Inc.**
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**Notes to Consolidated Financial Statements (Continued)**

**5. Convertible Preferred Stock and Stockholders' Equity (Deficit) (Continued)**

provisions was not determined to be probable. The aggregate grant date fair value of these unvested options at December 31, 2013 and December 31, 2012 was $0.8 million and $0.2 million, respectively.

As of December 31 2012, the outstanding options included 163,573 options, with both performance-based milestones and market conditions that were not determined to be probable of achievement. The aggregate grant date fair value of these unvested options at December 31, 2012 was $0.2 million. As of December 31, 2013, the performance-based milestone components of these options had been achieved, and such options are therefore being expensed over the derived service period. As of December 31, 2013, the market conditions of these options have not been achieved.

As of December 31, 2013 and 2012, the unrecognized compensation cost related to outstanding options (excluding those with unachieved performance-based and/or market conditions), was $1.9 million and $0.8 million, respectively, and is expected to be recognized as expense over approximately 2.9 years and 3.4 years, respectively.

Information about the Company's stock option activity is as follows (in thousands, except share and per share data):

| | Years Ended December 31, | | |
|---|---|---|---|
| | 2013 | 2012 | 2011 |
| Weighted-average grant date fair value per share of employee options granted | $ 4.40 | $ 1.11 | $ 1.24 |
| Cash received upon exercise of options | $ 23 | $ 15 | $ 14 |

During the year ended December 31, 2012, the Company received $0.2 million from the issuance of restricted common stock.

Outstanding restricted stock awards both inside and outside of the 2007 Plan are summarized as follows:

| | Inside the Plan | | Outside the Plan | |
|---|---|---|---|---|
| | Number of Shares | Weighted-Average Price | Number of Shares | Weighted-Average Price |
| Balance at December 31, 2013 and December 31, 2012 | 434,884 | $ 0.468 | 564,904 | $ 0.007 |

Unvested outstanding restricted stock awards, issued inside the 2007 Plan, as of December 31, 2013 and 2012 were 91,740 and 121,332 shares, respectively. Unvested restricted stock awards as of December 31, 2013 consists of 56,713 shares that vest monthly over a four year period and 35,027 shares that cliff vest in April 2018 or earlier upon the achievement of specified milestones. All restricted stock awards outside the 2007 Plan were fully vested as of December 31, 2013 and 2012.

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**Fate Therapeutics, Inc.**
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**Notes to Consolidated Financial Statements (Continued)**

**5. Convertible Preferred Stock and Stockholders' Equity (Deficit) (Continued)**

**Stock-Based Compensation Expense**

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

| | Years Ended December 31, | | |
|---|---|---|---|
| | 2013 | 2012 | 2011 |
| Risk-free interest rate | 1.6% | 1.0% | 1.1% |
| Expected volatility | 90% | 94% | 90% |
| Expected term (in years) | 5.9 | 6.1 | 6.1 |
| Expected dividend yield | 0.0% | 0.0% | 0.0% |

*Risk-free interest rate.* The Company bases the risk-free interest rate assumption on observed interest rates appropriate for the expected term of the stock option grants.

*Expected dividend yield.* The Company bases the expected dividend yield assumption on the fact that it has never paid cash dividends and has no present intention to pay cash dividends.

*Expected volatility.* Due to the Company's limited operating history and lack of company-specific historical or implied volatility, the expected volatility assumption is based on historical volatilities of a peer group of similar companies whose share prices are publicly available. The peer group was developed based on companies in the biotechnology industry.

*Expected term.* The expected term represents the period of time that options are expected to be outstanding. Because the Company does not have historical exercise behavior, it determines the expected life assumption using the simplified method, which is an average of the contractual term of the option and its vesting period.

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

| | Years Ended December 31, | | |
|---|---|---|---|
| | 2013 | 2012 | 2011 |
| Risk-free interest rate | 2.1% | 1.2% | 1.1% |
| Expected volatility | 91% | 94% | 90% |
| Expected term (in years) | 7.3 | 7.5 | 6.1 |
| Expected dividend yield | 0.0% | 0.0% | 0.0% |

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**Fate Therapeutics, Inc.**
**(A Development Stage Company)**

**Notes to Consolidated Financial Statements (Continued)**

**5. Convertible Preferred Stock and Stockholders' Equity (Deficit) (Continued)**

The allocation of stock-based compensation for all options granted and restricted stock awards are as follows (in thousands):

| | Years Ended December 31, | | | Period from April 27, 2007 (inception) to December 31, 2013 |
|---|---|---|---|---|
| | 2013 | 2012 | 2011 | |
| Research and development | $ 912 | $ 97 | $ 156 | $ 1,569 |
| General and administrative | 642 | 58 | 16 | 754 |
| Total stock-based compensation expense | $ 1,554 | $ 155 | $ 172 | $ 2,323 |

**Common Stock Reserved for Future Issuance**

Common stock reserved for future issuance is as follows:

| | December 31, | |
|---|---|---|
| | 2013 | 2012 |
| Conversion of convertible preferred stock | | 7,229,590 |
| Stock warrants | 36,074 | 36,074 |
| Common stock options | 1,726,991 | 1,432,369 |
| Awards available under the 2013 Plan | 1,003,526 | 154,448 |
| Exchangeable shares | 403,842 | 884,605 |
| Employee stock purchase plan | 729,000 | |
| | 3,899,433 | 9,737,086 |

**6. Collaboration Agreement**

On September 30, 2010, the Company entered into a worldwide exclusive collaboration and license agreement with Becton, Dickinson and Company ("BD") for the joint development and worldwide commercialization of certain induced pluripotent stem cell ("iPSC") tools and technologies for use in drug discovery and development. In connection with the agreement, the Company received a $0.3 million upfront, nonrefundable license payment and received research funding of $0.8 million per year, during a three-year joint development period, for the conduct of its development activities. In addition, the Company is eligible to receive (i) milestone payments in the amount of $0.5 million, $0.7 million and $0.8 million in connection with the first commercial sale of up to three different iPSC products developed under the agreement, (ii) milestone payments of up to an aggregate amount of $4.0 million in connection with the achievement of certain annual net sales of iPSC products and (iii) royalties on the sale of such iPSC products. In 2012, the Company received a milestone payment of $0.5 million in connection with the first commercial sale of an iPSC product. The Company does not believe it is probable that it will receive any future milestone payments in connection with the first commercial sale of an iPSC product or the achievement of certain annual net sales of iPSC products, or any material royalties, under the agreement.

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**Fate Therapeutics, Inc.**
**(A Development Stage Company)**

**Notes to Consolidated Financial Statements (Continued)**

**6. Collaboration Agreement (Continued)**

License payments under the BD agreement were recorded as deferred revenue upon receipt and recognized ratably as revenue over the three-year program period as a result of the Company's continuing involvement with the collaboration. Funding received for the Company's research efforts under the program was recognized as revenue as costs were incurred, which approximated the level of effort over the three year period of the program. The Company recognized 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 the Company's performance or on the occurrence of a specific outcome resulting from the Company's performance and its achievability was not reasonably assured at the inception of the agreement, (ii) the Company does 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. Royalties received under the agreement will generally be recognized as revenue upon receipt of the related royalty payment. In connection with the BD agreement for the years ended December 31, 2013, 2012, and 2011 and the period from April 27, 2007 (inception) to December 31, 2013, the Company recognized $0.6 million, $1.3 million, $0.8 million, and $2.9 million, respectively, as revenue in its consolidated statements of operations.

**7. Income Taxes**

The following is a reconciliation of the Company's expected federal income tax provision (benefit) to the actual income tax provision (in thousands):

| | Years Ended December 31, | | |
|---|---|---|---|
| | **2013** | **2012** | **2011** |
| Tax computed at federal statutory rate | $ (7,104) | $ (4,842) | $ (4,540) |
| State tax, net of federal tax benefit | (1,011) | (777) | (742) |
| Permanent differences | 755 | 113 | 135 |
| Stock compensation | 161 | 36 | |
| R&D tax credits | (621) | (243) | (515) |
| Other | (8) | 207 | 5 |
| Valuation allowance | 7,828 | 5,506 | 5,657 |
| Income tax expense | $ | $ | $ |

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**Fate Therapeutics, Inc.**
**(A Development Stage Company)**

**Notes to Consolidated Financial Statements (Continued)**

**7. Income Taxes (Continued)**

Significant components of the Company's deferred tax assets are summarized as follows (in thousands):

| | December 31, | |
|---|---|---|
| | 2013 | 2012 |
| Deferred tax assets: | | |
| Section 59e amortization | $ 12,070 | $ 9,766 |
| Foreign net operating losses | 72 | 101 |
| Depreciation and amortization | 880 | 787 |
| Other | 845 | 495 |
| Deferred tax assets | 13,867 | 11,149 |
| Valuation allowance | (13,867) | (11,149) |
| Net deferred tax assets | $ | $ |

A valuation allowance of $13.9 million and $11.1 million at December 31, 2013 and 2012, respectively, has been established to offset the deferred tax assets, as realization of such assets is uncertain.

At December 31, 2013, the Company had federal, California and Canadian net operating loss ("NOL") carryforwards of approximately $45.2 million, $42.7 million and $0.3 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, 2013, the Company had federal and California research and development ("R&D") credit carryforwards of approximately $1.7 million and $1.6 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 Internal Revenue Code of 1986, as amended (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 the Company's formation, the Company has raised capital through the issuance of capital stock on several occasions, including the IPO in 2013, which on their 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.

The Company has not completed a study to assess whether an ownership change has occurred or whether there have been multiple ownership changes since the Company's 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 the Company has experienced an ownership change at any time since its formation, utilization of the NOL or R&D credit carryforwards would be subject to an annual

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**Fate Therapeutics, Inc.**
**(A Development Stage Company)**

**Notes to Consolidated Financial Statements (Continued)**

**7. Income Taxes (Continued)**

limitation under Section 382 of the Code, which is determined by first multiplying the value of the Company's 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 the Company's unrecognized tax benefits will not impact its 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.

The Company files income tax returns in the United States, California and Canada. The Company currently has no years under examination by any jurisdiction; however, the Company is subject to income tax examination by federal, state and Canadian tax authorities for years beginning in 2010, 2009, and 2010, respectively. However, to the extent allowed by law, the taxing authorities may have the right to examine prior periods where NOLs and tax credits were generated and carried forward, and make adjustment up to the amount of the carryforwards.

The change in the Company's unrecognized tax benefits is summarized as follows (in thousands):

| | |
|---|---|
| Balance at December 31, 2011 | $ |
| Increase related to prior year positions | 11 |
| Balance at December 31, 2012 | $ 11 |
| Increase related to prior year positions | 5 |
| Balance at December 31, 2013 | $ 16 |

The Company does not anticipate that the amount of unrecognized tax benefits as of December 31, 2013 will change within the next twelve months. The Company has not recognized interest or penalties in its consolidated statements of operations and comprehensive loss since inception.

**8. Employee Benefits**

Effective January 1, 2009, the Company adopted a defined contribution 401(k) plan for employees who are at least 21 years of age. Employees are eligible to participate in the plan beginning on the first day of the calendar quarter following date of hire. Under the terms of the plan, employees may make voluntary contributions as a percent of compensation. No matching contributions have been made by the Company since the adoption of the 401(k) plan.

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**Fate Therapeutics, Inc.**
**(A Development Stage Company)**

**Notes to Consolidated Financial Statements (Continued)**

**9. Selected Quarterly Financial Data**

| (in thousands, except per share data) (unaudited) | First Quarter | Second Quarter | Third Quarter | Fourth Quarter |
|---|---|---|---|---|
| **2013** | | | | |
| Revenues | $ 472 | $ 290 | $ 209 | $ |
| Total operating expenses | 3,828 | 4,559 | 5,357 | 4,902 |
| Net loss | (3,548) | (5,534) | (6,073) | (5,739) |
| Basic and diluted net loss per common share | $ (2.92) | $ (4.46) | $ (4.81) | $ (0.29) |
| **2012** | | | | |
| Revenues | $ 418 | $ 1,081 | $ 528 | $ 643 |
| Total operating expense | 3,538 | 3,824 | 4,178 | 4,687 |
| Net loss | (3,234) | (2,737) | (4,113) | (4,155) |
| Basic and diluted net loss per common share | $ (3.43) | $ (2.58) | $ (3.51) | $ (3.51) |

**10. Subsequent Events**

The Company evaluates transactions and events that occur after the balance sheet date as potential subsequent events that may require disclosure and or adjustments to the consolidated financial statements as of the balance sheet date. We have completed this evaluation of all events through the issuance date of these financial statements and determined that no subsequent transactions or events require disclosure.

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**ITEM 9. Changes in and Disagreements with Accountants on Accounting and Financial Disclosure**

None.

**ITEM 9A. Controls and Procedures**

**Evaluation of Disclosure Controls and Procedures.** We are responsible for maintaining disclosure controls and procedures, as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act. Disclosure controls and procedures are controls and other procedures designed to ensure that the information required to be disclosed by us in the reports that we file or submit under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by us in the reports that we file or submit under the Exchange Act is accumulated and communicated to our management, including our Chief Executive Officer and Chief Financial Officer, as appropriate to allow timely decisions regarding required disclosure. In designing and evaluating the disclosure controls and procedures, management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable and not absolute assurance of achieving the desired control objectives, and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

Based on our management's evaluation (with the participation of our Chief Executive Officer and Chief Financial Officer) of our disclosure controls and procedures as required by Rule 13a-15 under the Exchange Act, our Chief Executive Officer and Chief Financial Officer have concluded that our disclosure controls and procedures were effective at the reasonable assurance level as of December 31, 2013, the end of the period covered by this report.

**Management's Report on Internal Control over Financial Reporting.** This annual report does not include a report of management's assessment regarding internal control over financial reporting or an attestation report of the Company's registered public accounting firm due to a transition period established by rules of the SEC for newly public companies.

**Changes in Internal Control over Financial Reporting.** There were no changes in our internal control over financial reporting during the quarter ended December 31, 2013 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

**ITEM 9B. Other Information**

None.

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## PART III

### ITEM 10. Directors, Executive Officers and Corporate Governance

Except as set forth below, the information required by this item will be contained in our definitive proxy statement to be filed with the SEC in connection with the Annual Meeting of Stockholders within 120 days after the conclusion of our fiscal year ended December 31, 2013, or the Proxy Statement, and is incorporated in this Annual Report on Form 10-K by reference.

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. A current copy of the code is 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 our principal executive officer, principal financial officer, principal accounting officer, controller or persons performing similar functions, or 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.

### ITEM 11. Executive Compensation

The information required by this item will be contained in the Proxy Statement and is incorporated in this Annual Report on Form 10-K by reference.

### ITEM 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters

The information required by this item will be contained in the Proxy Statement and is incorporated in this Annual Report on Form 10-K by reference.

### ITEM 13. Certain Relationships and Related Party Transactions, and Director Independence

The information required by this item will be contained in the Proxy Statement and is incorporated in this Annual Report on Form 10-K by reference.

### ITEM 14. Principal Accounting Fees and Services

The information required by this item will be contained in the Proxy Statement and is incorporated in this Annual Report on Form 10-K by reference.

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## PART IV

### ITEM 15. EXHIBITS AND FINANCIAL STATEMENT SCHEDULES

(a)

The following documents are filed as part of this report:

(1)

*Index list to Financial Statements:*



(2)

*Financial Statement Schedules*

All other schedules are omitted because they are not required or the required information is included in the financial statements or notes thereto.

(3)

*Exhibits*

The exhibits listed in the accompanying Exhibit Index are filed or incorporated by reference as part of this report.

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**SIGNATURES**

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the Registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

Date: March 13, 2014

**Fate Therapeutics, Inc.**

By: /s/ CHRISTIAN WEYER

Christian Weyer

*President and Chief Executive Officer (Principal Executive Officer and Authorized Signatory)*

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Christian Weyer and J. Scott Wolchko, jointly and severally, his or her attorneys-in-fact, each with the power of substitution, for him or her in any and all capacities, to sign any amendments to this report, and to file the same, with exhibits thereto and other documents in connection therewith with the Securities and Exchange Commission, hereby ratifying and confirming all that each of said attorneys-in-fact, or his or her substitute or substitutes may do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the registrant in the capacities and on the dates indicated:

| SIGNATURE | TITLE | DATE |
|---|---|---|
| /s/ CHRISTIAN WEYER<br>Christian Weyer, M.D., M.A.S. | President and Chief Executive Officer and Director (Principal Executive Officer) | March 13, 2014 |
| /s/ J. SCOTT WOLCHKO<br>J. Scott Wolchko | Chief Financial Officer and Chief Operating Officer (Principal Financial and Accounting Officer) | March 13, 2014 |
| /s/ WILLIAM H. RASTETTER<br>William H. Rastetter, Ph.D. | Chairman of the Board and Director | March 13, 2014 |
| /s/ JOHN D. MENDLEIN<br>John D. Mendlein, Ph.D., J.D. | Vice Chairman of the Board and Director | March 13, 2014 |
| /s/ TIMOTHY P. COUGHLIN<br>Timothy P. Coughlin | Director | March 13, 2014 |

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| SIGNATURE | TITLE | DATE |
|---|---|---|
| /s/ MARK J. ENYEDY<br>Mark J. Enyedy | Director | March 13, 2014 |
| /s/ AMIR NASHAT<br>Amir Nashat, Sc.D. | Director | March 13, 2014 |
| /s/ ROBERT T. NELSEN<br>Robert T. Nelsen | Director | March 13, 2014 |

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**EXHIBIT INDEX**

| Exhibit No. | Exhibit Index |
|---|---|
| 3.1 | Amended and Restated Certificate of Incorporation of the Registrant, as currently in effect(1). |
| 3.2 | Amended and Restated Bylaws of the Registrant, as currently in effect(2). |
| 4.1 | Specimen Common Stock Certificate(3). |
| 4.2 | Warrant to Purchase Stock issued to Silicon Valley Bank on January 5, 2009(4). |
| 4.3 | First Amendment to Warrant to Purchase Stock dated January 5, 2009 by and between the Registrant and SVB Financial Group, dated August 25, 2011(4). |
| 4.4 | Warrant to Purchase Stock issued to Silicon Valley Bank on August 25, 2011(4). |
| 10.1# | 2007 Equity Incentive Plan and forms of agreements thereunder(3). |
| 10.2# | 2013 Stock Option and Incentive Plan and forms of agreements thereunder(5). |
| 10.3# | Employment Offer Letter by and between the Registrant and Christian Weyer, dated October 2, 2012(4). |
| 10.4# | Employment Offer Letter by and between the Registrant and Scott Wolchko, dated September 17, 2007(4). |
| 10.5# | Amendment to Employment Offer Letter by and between the Registrant and Scott Wolchko, dated November 11, 2008(4). |
| 10.6# | Employment Offer Letter by and between the Registrant and Pratik S. Multani, dated March 23, 2009(4). |
| 10.7 | Consulting Agreement by and between the Registrant and John D. Mendlein, dated December 31, 2012(4). |
| 10.8 | Director Letter Agreement by and between the Registrant and Mark Enyedy, dated May 24, 2012(4). |
| 10.9 | Exclusive License Agreement by and between the Registrant and Children's Medical Center Corporation, dated May 13, 2009(4). |
| 10.10 | Exclusive License Agreement by and between the Registrant and The Board of Trustees of the Leland Stanford Junior University, dated May 2, 2013(4). |
| 10.11 | Restated License Agreement by and between The Ottawa Hospital Research Institute and Fate Therapeutics (Canada) Inc. (as successor to Verio Therapeutics, Inc.), effective April 6, 2010(4). |
| 10.12 | First Amendment to Restated License Agreement by and between The Ottawa Hospital Research Institute and Fate Therapeutics (Canada) Inc. (as successor to Verio Therapeutics, Inc.), effective February 14, 2012(4). |
| 10.13 | Second Amendment to Restated License Agreement by and between The Ottawa Hospital Research Institute and Fate Therapeutics (Canada) Inc. (as successor to Verio Therapeutics, Inc.), effective June 3, 2013(4). |
| 10.14 | Lease Agreement by and between the Registrant and ARE-3535/3565 General Atomics Court, LLC, dated December 3, 2009(4). |

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| Exhibit No. | Exhibit Index |
|---|---|
| 10.15 | First Amendment to Lease Agreement by and between the Registrant and ARE-3535/3565 General Atomics Court, LLC, dated October 1, 2011(4). |
| 10.16 | Loan and Security Agreement by and between the Registrant and Silicon Valley Bank, dated January 5, 2009(4). |
| 10.17 | Amendment No. 1 to Loan and Security Agreement by and between the Registrant and Silicon Valley Bank, dated May 4, 2010(4). |
| 10.18 | Second Amendment to Loan and Security Agreement by and between the Registrant and Silicon Valley Bank, dated August 25, 2011(4). |
| 10.19 | Amended and Restated Investor Rights Agreement, dated August 8, 2013 by and between the Registrant and the stockholders named therein(4). |
| 10.20 | Form of Indemnification Agreement(3). |
| 10.21 | Director Letter Agreement by and between the Registrant and Timothy Coughlin, dated August 5, 2013(6). |
| 10.22# | 2013 Employee Stock Purchase Plan(7). |
| 10.23 | Second Amendment to Lease Agreement by and between the Registrant and ARE-3535/3565 General Atomics Court, dated September 26, 2013(8). |
| 10.24# | Senior Executive Cash Incentive Bonus Plan(9). |
| 14.1 | Code of Business Conduct and Ethics. |
| 21.1 | Subsidiaries of the Registrant(4). |
| 23.1 | Consent of Ernst & Young LLP, Independent Registered Public Accounting Firm. |
| 24.1 | Power of Attorney (included in page 133). |
| 31.1 | Certification of Principal Executive Officer pursuant to Rules 13a-14 and 15-d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002. |
| 31.2 | Certification of Principal Financial Officer pursuant to Rules 13a-14 and 15-d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002. |
| 32.1 | Certification of Principal Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002. |
| 32.2 | Certification of Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002. |
| 101.INS* | XBRL Instance Document |
| 101.SCH* | XBRL Taxonomy Extension Schema Document |
| 101.CAL* | XBRL Taxonomy Extension Calculation Linkbase Document |
| 101.DEF* | XBRL Taxonomy Extension Definition Linkbase Document |
| 101.LAB* | XBRL Taxonomy Extension Label Linkbase Document |

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| Exhibit No. | Exhibit Index |
|---|---|
| 101.PRE* | XBRL Taxonomy Extension Presentation Linkbase Document |

Certain provisions of this Exhibit have been omitted pursuant to a request for confidential treatment.

#

Indicates a management contract or any compensatory plan, contract or arrangement.

*

In accordance with Rule 406T of Regulation S-T, the XBRL related information in Exhibit 101 to this Annual Report on Form 10-K is furnished and shall not be deemed to be "filed" for purposes of Section 18 of the Exchange Act, or otherwise subject to the liability of the section, and shall not be part of any registration statement or other document filed under the Securities Act or the Exchange Act, except as shall be expressly set forth by specific reference in such filing.

(1)

Filed as Exhibit 3.2 to the registrant's Registration Statement on Form S-1/A (File No. 333-190608) filed with the SEC on August 29, 2013 and incorporated herein by reference.

(2)

Filed as Exhibit 3.4 to the registrant's Registration Statement on Form S-1/A (File No. 333-190608) filed with the SEC on August 29, 2013 and incorporated herein by reference.

(3)

Filed as same numbered exhibit to the registrant's Registration Statement on Form S-1/A (File No. 333-190608) filed with the SEC on August 29, 2013 and incorporated herein by reference.

(4)

Filed as same numbered exhibit to the registrant's Registration Statement on Form S-1 (File No. 333-190608) filed with the SEC on August 13, 2013 and incorporated herein by reference.

(5)

Filed as Exhibit 10.2 to the registrant's Registration Statement on Form S-1/A (File No. 333-190608) filed with the SEC on September 16, 2013 and incorporated herein by reference.

(6)

Filed as Exhibit 10.22 to the registrant's Registration Statement on Form S-1 (File No. 333-190608) filed with the SEC on August 13, 2013 and incorporated herein by reference.

(7)

Filed as Exhibit 10.24 to the registrant's Registration Statement on Form S-1/A (File No. 333-190608) filed with the SEC on September 16, 2013 and incorporated herein by reference.

(8)

Filed as Exhibit 10.25 to the registrant's Registration Statement on Form S-1/A (File No. 333-190608) filed with the SEC on September 30, 2013 and incorporated herein by reference.

(9)

Filed as Exhibit 10.1 to the registrant's Current Report on Form 8-K (File No. 001-36076), filed with the SEC on January 10, 2014 and incorporated herein by reference.