Table of Contents

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2014

OR

☐ **TRANSITION REPORT UNDER SECTION 13 OR 15(d) OF THE EXCHANGE ACT OF 1934**

From 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)	(IRS Employer Identification No.)
3535 General Atomics Court, Suite 200, San Diego, CA	**92121**
(Address of principal executive offices)	(Zip Code)

(858) 875-1800

(Registrant's telephone number, including area code)

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 ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§232.405 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 ☒ No ☐

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:

Large accelerated filer ☐	Accelerated filer ☐
Non-accelerated filer ☐ (Do not check if a smaller reporting company)	Smaller reporting company ☒

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

As of November 11, 2014, 20,569,399 shares of the registrant's common stock, par value $0.001 per share, were issued and outstanding.

FATE THERAPEUTICS, INC.

FORM 10-Q

TABLE OF CONTENTS

		Page
PART I. FINANCIAL INFORMATION		
Item 1.	Financial Statements	3
	Condensed Consolidated Balance Sheets as of September 30, 2014 (unaudited) and December 31, 2013	3
	Condensed Consolidated Statements of Operations and Comprehensive Loss for the three and nine months ended September 30, 2014 and 2013 (unaudited)	4
	Condensed Consolidated Statements of Cash Flows for the nine months ended September 30, 2014 and 2013 (unaudited)	5
	Notes to Condensed Consolidated Financial Statements (unaudited)	6
Item 2.	Management's Discussion and Analysis of Financial Condition and Results of Operations	13
Item 3.	Quantitative and Qualitative Disclosures About Market Risk	21
Item 4.	Controls and Procedures	21
PART II. OTHER INFORMATION		
Item 1.	Legal Proceedings	21
Item 1A.	Risk Factors	22
Item 2.	Unregistered Sales of Equity Securities and Use of Proceeds	56
Item 3.	Defaults Upon Senior Securities	56
Item 4.	Mine Safety Disclosures	56
Item 5.	Other Information	56
Item 6.	Exhibits	56
SIGNATURES		57

PART I. FINANCIAL INFORMATION

Item 1. Financial Statements

Fate Therapeutics, Inc.

Condensed Consolidated Balance Sheets
(in thousands, except share and per share data)

	September 30, 2014 (unaudited)	December 31, 2013
Assets		
Current assets:		
Cash and cash equivalents	$ 45,530	$ 54,036
Prepaid expenses and other current assets	94	615
Total current assets	45,624	54,651
Property and equipment, net	1,240	810
Restricted cash	122	122
Other assets	24	—
Total assets	$ 47,010	$ 55,583
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 1,157	$ 682
Accrued expenses	2,010	2,039
Current portion of deferred rent	77	53
Repurchase liability for unvested equity awards	57	94
Long-term debt, current portion	611	1,732
Total current liabilities	3,912	4,600
Deferred rent	76	135
Accrued expenses	57	—
Long-term debt, net of current portion	9,389	—
Commitments and contingencies (Note 4)		
Stockholders' equity:		
Preferred stock, $0.001 par value; authorized shares—5,000,000 at September 30, 2014 and December 31, 2013; no shares issued or outstanding	—	—
Common stock, $0.001 par value; authorized shares — 150,000,000 at September 30, 2014 and December 31, 2013; issued and outstanding shares — 20,562,773 at September 30, 2014 and 20,434,080 at December 31, 2013	21	20
Additional paid-in capital	139,714	137,337
Accumulated deficit	(106,159)	(86,509)
Total stockholders' equity	33,576	50,848
Total liabilities and stockholder's equity	$ 47,010	$ 55,583

See accompanying notes.

Fate Therapeutics, Inc.

Condensed Consolidated Statements of Operations and Comprehensive Loss
(in thousands, except share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2014	2013	2014	2013
			(unaudited)	
Revenues:				
Collaboration revenue	$ —	$ 209	$ —	$ 626
Grant revenue	—	—	—	345
Total revenue	—	209	—	971
Operating expenses:				
Research and development	4,080	3,378	12,570	8,976
General and administrative	1,904	1,979	6,391	4,768
Total operating expenses	5,984	5,357	18,961	13,744
Loss from operations	(5,984)	(5,148)	(18,961)	(12,773)
Other income (expense):				
Interest income	—	2	1	3
Interest expense	(187)	(230)	(258)	(418)
Loss on extinguishment of debt	(432)	—	(432)	—
Change in fair value of exchangeable shares	—	(728)	—	(1,988)
Change in fair value of warrant liability	—	31	—	21
Total other expense, net	(619)	(925)	(689)	(2,382)
Net loss and comprehensive loss	$ (6,603)	$ (6,073)	$ (19,650)	$ (15,155)
Net loss per common share, basic and diluted	$ (0.32)	$ (4.81)	$ (0.96)	$ (12.24)
Weighted-average common shares used to compute basic and diluted net loss per share	20,489,181	1,262,546	20,435,073	1,238,567

See accompanying notes.

Fate Therapeutics, Inc.

Condensed Consolidated Statements of Cash Flows
(in thousands)

	Nine Months Ended September 30,	
	2014	2013
	(unaudited)	
Operating activities		
Net loss	$ (19,650)	$ (15,155)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	371	442
Issuances of common stock for technology	—	13
Stock-based compensation	1,822	1,169
Amortization of discounts and debt issuance costs	17	129
Noncash interest expense	76	134
Deferred rent	(35)	(185)
Deferred revenue	—	(63)
Stock-based milestone charges and change in fair value of exchangeable shares	375	2,334
Change in fair value of preferred stock warrants	—	(21)
Loss on disposal of assets	—	18
Loss on extinguishment of debt	3	—
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	520	(960)
Accounts payable and accrued expenses	236	1,350
Net cash used in operating activities	(16,265)	(10,795)
Investing activities		
Purchase of property and equipment	(611)	(94)
Proceeds from sale of property and equipment	—	6
Net cash used in investing activities	(611)	(88)
Financing activities		
Issuance of common stock, net of repurchases and issuance costs	146	23
Proceeds from (cost of) initial public offering, net of offering costs	—	(1,381)
Issuance of convertible promissory notes	—	23,736
Proceeds from long-term debt	10,000	—
Payments on long-term debt	(1,750)	(1,500)
Payments for the issuance of debt	(26)	—
Net cash provided by financing activities	8,370	20,878
Net change in cash and cash equivalents	(8,506)	9,995
Cash and cash equivalents at beginning of the period	54,036	9,087
Cash and cash equivalents at end of the period	$ 45,530	$ 19,082

See accompanying notes.

Fate Therapeutics, Inc.

Notes to Condensed Consolidated Financial Statements
(Unaudited)

1. Organization and Summary of Significant Accounting Policies

Organization

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 to treat severe, life-threatening diseases. The Company's approach utilizes established pharmacologic modalities, such as small molecules, and targets well-characterized biological mechanisms to program the fate and enhance the therapeutic potential of adult stem cells. The Company's lead product candidate, ProHema, is an *ex vivo* programmed hematopoietic stem cell, or HSC, therapeutic, which is currently in clinical development for patients undergoing HSC transplantation. The Company is also applying its reprogramming modulators to develop human induced pluripotent stem cell-derived cellular therapeutics, and evaluating the *in vivo* programming of muscle satellite stem cells using its Wnt7a-based protein analogs for muscle regeneration.

As of September 30, 2014, 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.

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;
- 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;
- 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"), a subsidiary of the Company incorporated in 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;
- 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; 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.

Use of Estimates

The Company's consolidated financial statements are prepared in accordance with United States generally accepted accounting principles ("GAAP"). The preparation of the Company's 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 accrued expenses. 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 Canada, Fate Therapeutics Ltd., incorporated in the United Kingdom, and Destin Therapeutics Inc., incorporated in Canada, which was dissolved in June 2014. To date, the aggregate operations of these subsidiaries have not been significant and all intercompany transactions and balances have been eliminated in consolidation.

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.

Unaudited Interim Financial Information

The accompanying interim condensed consolidated financial statements are unaudited. These unaudited interim condensed consolidated financial statements have been prepared in accordance with GAAP and following the requirements of the United States Securities and Exchange Commission ("SEC") for interim reporting. As permitted under those rules, certain footnotes or other financial information that are normally required by GAAP can be condensed or omitted. In management's opinion, the unaudited interim financial statements have been prepared on the same basis as the audited financial statements and include all adjustments, which include only normal recurring adjustments, necessary for the fair presentation of the Company's financial position and its results of operations and comprehensive loss and its cash flows for periods presented. These statements do not include all disclosures required by GAAP and should be read in conjunction with the Company's financial statements and accompanying notes for the fiscal year ended December 31, 2013, contained in the Company's Annual Report on Form 10-K for the year ended December 31, 2013 filed by the Company with the SEC on March 17, 2014. The results for the three and nine months ended September 30, 2014 are not necessarily indicative of the results expected for the full fiscal year or any other interim period or any future year or period.

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) collectability 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 has been 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 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.

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 for which vesting is subject to 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 for which vesting is subject to 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 for which vesting is subject to 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 for which vesting is subject to performance-based milestones, the expense is recorded over the remaining service period after the point when the performance condition has been achieved.

Net Loss per Common Share

Basic net loss per common share is calculated by dividing the net loss by the weighted-average number of shares of common stock 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 73,248 shares and 102,998 shares for the three months ended September 30, 2014 and 2013, respectively, and 80,645 shares and 111,614 shares for the nine months ended September 30, 2014 and 2013, respectively. Diluted net loss per share is calculated by dividing the net loss by the weighted-average number of common stock equivalents outstanding for the period determined using the treasury-stock method. Dilutive common stock equivalents for the periods presented include convertible preferred stock, warrants for the purchase of convertible preferred stock and common stock, exchangeable shares and common stock options outstanding under the Company's stock option plan. For all periods presented, there is no difference in the number of shares used to calculate basic and diluted shares outstanding due to the Company's net loss position.

For the three and nine months ended September 30, 2014, the Company realized a net loss of $6.6 million and $19.7 million, respectively. Shares of potentially dilutive securities totaled 2.5 million for each of the three and nine months ended September 30, 2014, including options to purchase 2.4 million shares of common stock.

For the three and nine months ended September 30, 2013, the Company realized a net loss of $6.1 million and $15.2 million, respectively. Shares of potentially dilutive securities totaled 9.5 million for each of the three and nine months ended September 30, 2013.

Recent Accounting Pronouncements

In August 2014, the Financial Accounting Standards Board (the "FASB") issued Accounting Standards Update ("ASU") 2014-15, which defined management's responsibility to evaluate whether there is substantial doubt about an entity's ability to continue as a going concern and to provide related disclosure. ASU 2014-15 defined the term substantial doubt and requires an assessment for a period of one year after the date of the issuance of the financial statements. It requires certain disclosures when substantial doubt is alleviated as a result of consideration of management's plans and requires an express statement and other disclosures when substantial doubt is not alleviated. The guidance becomes effective for reporting periods beginning after December 15, 2016, with early adoption permitted. The Company is currently evaluating the impact the adoption of this guidance will have on its Consolidated Financial Statements.

In June 2014, the FASB issued ASU 2014-10, which eliminated all incremental financial reporting requirements from U.S. GAAP for development stage entities, including inception-to-date information, the labeling of financial statements as those of a development stage entity, and the disclosure of a description of the development stage activities in which the entity is engaged. Effectively, ASU 2014-10 removed the definition of a development stage entity from the Master Glossary of the Accounting Standards Codification. For public business entities, this guidance is effective for annual reporting periods beginning after December 15, 2014, and interim periods therein. Early adoption of the guidance is permitted for any annual reporting period or interim period for which the entity's financial statements have not yet been issued. Accordingly, the Company elected the early adoption of ASU 2014-10 beginning with the Quarterly Report on Form 10-Q for the quarter ended June 30, 2014, and will no longer disclose inception-to-date information or incremental financial reporting requirements related to development stage entities.

In May 2014, the FASB issued ASU 2014-09, which created a single, principle-based revenue recognition model that will supersede and replace nearly all existing U.S. GAAP revenue recognition guidance. Entities will recognize revenue in a manner that depicts the transfer of goods or services to customers at an amount that reflects the consideration to which the entity expects to be entitled to receive in exchange for those goods or services. The model provides that entities follow five steps: (i) identify the contract with a customer, (ii) identify the performance obligations in the contract, (iii) determine the transaction price, (iv) allocate the transaction price to the performance obligations, and (v) recognize revenue. For public business entities, the guidance becomes effective for annual reporting periods beginning after December 15, 2016, and interim periods therein. The Company is currently evaluating the impact the adoption of this guidance will have on its Consolidated Financial Statements.

2. Asset Acquisition of Verio Therapeutics Inc.

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.

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 the Company's 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 the Company's common stock.

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 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.

During the nine months ended September 30, 2014, based on the achievement of certain preclinical milestones, 38,463 shares of the Company's common stock were earned and issued, resulting in a $0.4 million charge to research and development expense.

In addition to the 38,463 shares of the Company's common stock earned and issued during the nine months ended September 30, 2014, the Company may issue an additional 365,379 shares of the Company's common stock based on the achievement of additional contractual milestones as follows: (i) 38,461 shares for the achievement of certain preclinical milestones, (ii) 211,538 shares for the achievement of certain clinical milestones and (iii) 115,380 shares for the achievement of certain commercialization milestones, such that the maximum aggregate number of shares of the Company's common stock issuable in connection with 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 and recorded in additional paid-in capital. 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). As of the IPO date, the exchangeable share liability was reclassified into additional paid-in capital.

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

	Common Stock	Fair Value Per Share of Underlying Common Stock	Initial Fair Value of Common Stock
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
March 2014	38,463	9.74	375
	519,226		$ 1,384

3. Fair Value Measurements

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 as described below, 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.

Financial assets measured at fair value on a recurring basis consist of the Company's cash equivalents. As of September 30, 2014 and December 31, 2013, the carrying amount of cash equivalents was $40.3 million and $52.3 million, respectively, which approximates fair value and was determined based upon Level 1 inputs. Cash equivalents primarily consisted of money market funds. As of September 30, 2014 and December 31, 2013, 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 included the preferred stock warrant liability and exchangeable shares for the periods the liabilities were outstanding. 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 September 30, 2014 and December 31, 2013, the Company had no material liabilities measured at fair value on a recurring basis.

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):

	September 30, 2014	December 31, 2013
Long-term debt	$ 10,000	$ 1,750
Less current portion of long-term debt	(611)	(1,750)
Long-term debt, net of current portion	$ 9,389	$ —
Current portion of long-term debt	$ 611	$ 1,750
Current portion of debt discount	—	(18)
Current portion of long-term debt, net	$ 611	$ 1,732

On July 30, 2014, the Company entered into an Amended and Restated Loan and Security Agreement (the "Restated LSA") with Silicon Valley Bank (the "Bank"), collateralized by substantially all of our assets, excluding certain intellectual property. The Restated LSA amends and restates the Loan and Security Agreement, dated as of January 5, 2009, as amended, by and between the Company and the Bank (the "Loan Agreement"). Pursuant to the Restated LSA, the Bank agreed to make loans to the Company in an aggregate principal amount of up to $20.0 million, comprised of (i) a $10.0 million term loan, funded at the closing date (the "Term A Loan") and (ii) subject to the achievement of a specified clinical milestone relating to the Company's Phase 2 clinical trial of ProHema, additional term loans totaling up to $10.0 million in the aggregate, which are available until December 31, 2014 (each, a "Term B Loan"), the first of which shall be at least $5.0 million.

The Term A Loan matures on January 1, 2018, and the Term B Loans mature on the first day of the 42nd month after the month in which each Term B Loan funds. The Term A Loan bears interest at a fixed annual rate of 6.94% and the Term B Loans will bear interest at a fixed annual rate, to be determined on the funding date, equal to the greater of (i) 6.75% or (ii) the sum of (a) U.S. Treasury note yield to maturity for a thirty-six (36) month term, plus (b) five hundred ninety (590) basis points. Interest is payable in cash on a monthly basis beginning the first day of each month following the month in which the funding date of each loan occurs. The Company is required to make a monthly payment of interest only during the first twelve months following the funding date of each loan, and thereafter is required to repay the principal and accrued interest under each loan in thirty equal monthly installments based on a thirty-month amortization schedule. The Company is required to make a final payment fee of 7.5% of the funded amount for the Term A Loan and any Term B Loan.

A portion of the proceeds from the Term A Loan were used to repay loans outstanding under the Loan Agreement and to pay for transaction fees related to the Restated LSA, including a commitment fee of $0.4 million paid by the Company to the Bank. The remaining proceeds are expected to be used for working capital purposes, including the advancement of the Company's research programs. Net proceeds from the Term A Loan, after repayment of loans outstanding under the Loan Agreement and transaction fees, were $8.8 million.

The Company determined the repayment of the Loan Agreement was a debt extinguishment, and accounted for the Term A Loan at fair value as of the issuance date accordingly. During each of the three and nine months ended September 30, 2014, the Company recorded a loss on debt extinguishment of $0.4 million, primarily related to the commitment fee paid to the Bank and $0.2 million in aggregate interest expense related to the Restated LSA. The Company determined the effective interest rate of the Term A Loan to be 10.3%.

As part of the financing, upon the Company's election to access the first Term B Loan, the Company will issue to the Bank and one or more of its affiliates warrants to purchase up to an aggregate of $0.4 million in shares of the Company's common stock (the "Warrants"), subject to adjustment, at an exercise price equal to the average price per share over the preceding ten trading days prior to the funding of the first Term B Loan.

Facility Lease

The Company leases certain office and laboratory space from a stockholder of the Company under a non-cancelable operating lease. 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. As of September 30, 2014, future minimum payments under the operating lease are $1.7 million.

5. Stockholders' Equity

Stock option activity under all equity and stock option plans is summarized as follows:

	Number of Options	Weighted-Average Price
Balance at December 31, 2013	1,726,991	$ 2.30
Granted	873,720	6.66
Canceled	(61,999)	5.29
Exercised	(90,230)	1.66
Balance at September 30, 2014	2,448,482	$ 3.81

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2014	2013	2014	2013
Research and development	$ 280	$ 581	$ 1,074	$ 686
General and administrative	247	401	748	483
	$ 527	$ 982	$ 1,822	$ 1,169

As of September 30, 2014, the outstanding options included 160,526 performance-based options for which the achievement of the performance-based vesting provisions was determined not to be probable. The aggregate grant date fair value of these unvested options at September 30, 2014 was $0.7 million.

As of September 30, 2014, the unrecognized compensation cost related to outstanding options (excluding those with performance-based conditions) was $4.5 million and is expected to be recognized as expense over approximately 2.8 years.

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:

	Nine Months Ended September 30,	
	2014	2013
Risk-free interest rate	1.9%	1.6%
Expected volatility	94.5%	90.2%
Expected term (in years)	6.0	6.1
Expected dividend yield	0.0%	0.0%

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:

	Nine Months Ended September 30,	
	2014	2013
Risk-free interest rate	2.2%	2.1%
Expected volatility	92.2%	90.3%
Remaining contractual term (in years)	6.7	7.3
Expected dividend yield	0.0%	0.0%

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations

The following discussion and analysis should be read in conjunction with our financial statements and accompanying notes included in this Quarterly Report on Form 10-Q and the financial statements and accompanying notes thereto for the fiscal year ended December 31, 2013 and the related Management's Discussion and Analysis of Financial Condition and Results of Operations, which are contained in our Annual Report on Form 10-K filed with the Securities and Exchange Commission on March 17, 2014.

This Quarterly Report on Form 10-Q contains "forward-looking statements" within the meaning of Section 27A of 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. Factors that could cause or contribute to differences in results include, but are not limited to, those set forth under "Risk Factors" under Item 1A of Part II below. Except as required by law, we undertake no obligation to update these forward-looking statements to reflect events or circumstances after the date of this report or to reflect actual outcomes.

Overview

We are a clinical-stage biopharmaceutical company engaged in the discovery and development of pharmacologic modulators of adult stem cells to treat severe, life-threatening diseases. Our novel approach utilizes established pharmacologic modalities, such as small molecules, and targets well-characterized biological mechanisms to program the fate and enhance the therapeutic potential of adult stem cells. Our lead product candidate, ProHema, is an *ex vivo* programmed hematopoietic stem cell, or HSC, therapeutic, which is currently in clinical development for patients undergoing HSC transplantation. We are also applying our reprogramming modulators to develop human induced pluripotent stem cell-derived cellular therapeutics and evaluating the *in vivo* programming of muscle satellite stem cells using our Wnt7a-based protein analogs for muscle regeneration. 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 discovery and development of pharmacologic modulators of adult stem cells, 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.

We have never been profitable and have incurred net losses in each year since inception. Substantially all of our net losses resulted from costs incurred in connection with our research and development programs and from general and administrative costs associated with our operations. We expect to continue to incur 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;
- 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, if at all, 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 Canada that were outstanding at September 30, 2014 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 in September 2013. We are eligible to receive certain commercialization milestones and royalties on the sale of iPSC reagent products. In connection with the arrangement with BD, we recognized $0.2 million and $0.6 million for the three and nine months ended September 30, 2013, respectively, as collaboration revenue in our consolidated statements of operations. Our three-year joint development period under our license and collaboration agreement with BD concluded in September 2013. We do not anticipate generating any significant revenues under the arrangement with BD in the future.

Grant revenue has been 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-induced hearing loss. All funding under the TATRC grant was expended in full as of May 2013.

Research and Development Expenses

Research and development expenses consist of development costs associated with our stem cell modulation platforms and our therapeutic product candidates. These costs are expensed as incurred and include:

- compensation and employee-related costs;
- costs associated with conducting our preclinical, clinical and regulatory activities, including fees paid to third-party professional consultants and service providers;
- costs incurred under clinical trial agreements with investigative sites;
- costs for laboratory supplies;
- costs to acquire, develop and manufacture preclinical study and clinical trial materials;
- charges associated with the achievement of 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 September 30, 2014, we have incurred $69.6 million in research and development expenses. We plan to increase our current level of research and development expenses for the foreseeable future as we continue the development of our stem cell modulation platforms and our initial therapeutic product candidates. Our current planned research and development activities over the next twelve months consist primarily of the following:

- conducting our Phase 2 clinical trial of ProHema to examine its safety and its curative potential in adult patients with orphan hematologic malignancies undergoing allogeneic hematopoietic stem cell transplants, or HSCT (the PUMA study);
- initiating and conducting our Phase 1b clinical trial of ProHema to examine its safety and curative potential in pediatric patients with orphan hematologic malignancies undergoing allogeneic HSCT (the PROMPT study);

- initiating and conducting our Phase 1b clinical trial of ProHema to examine its safety and curative potential in pediatric patients with inherited metabolic disorders, or IMDs, including lysosomal storage disorders, or LSDs, undergoing allogeneic HSCT (the PROVIDE study); and

- researching the therapeutic potential of human induced pluripotent stem cell-derived cellular therapeutics and evaluating the *in vivo* programming of muscle satellite stem cells using our Wnt7a-based protein analogs for muscle regeneration,

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

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2014	2013	2014	2013
HSC modulation platform	$ 2,573	$ 977	$ 6,874	$ 3,491
Other preclinical programs and technologies	784	1,128	3,473	2,879
Total direct research and development expenses	3,357	2,105	10,347	6,370
Unallocated expenses	723	1,273	2,223	2,606
Total research and development expenses	$ 4,080	$ 3,378	$ 12,570	$ 8,976

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 facilities; losses on debt extinguishments; changes in fair value of the exchangeable share liability, while outstanding, relating to the total exchangeable shares held by the prior stockholders of Verio; and changes in fair value of the warrant liability, while outstanding, relating to our preferred stock warrants.

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.

The estimates and judgments involved in the accounting policies as described in Item 7 of our Annual Report on Form 10-K for the year ended December 31, 2013 continue to be our critical accounting policies. There were no material changes to our critical accounting policies and estimates during the nine months ended September 30, 2014.

See Note 1 to the Condensed Consolidated Financial Statements for information related to recent accounting pronouncements.

Results of Operations

Comparison of the Three Months Ended September 30, 2014 and 2013

The following table summarizes the results of our operations for the three months ended September 30, 2014 and 2013 (in thousands):

	Three Months Ended September 30,		Increase / (Decrease)
	2014	2013	
Collaboration revenue	$ —	$ 209	$ (209)
Grant revenue	—	—	—
Research and development expense	4,080	3,378	702
General and administrative expense	1,904	1,979	(75)
Total other income (expense), net	(619)	(925)	(306)

Revenue. We did not generate any revenue for the three months ended September 30, 2014, compared to $0.2 million of revenue generated for the three months ended September 30, 2013. The decrease was due to the expiration of the three-year joint development period under our license and collaboration agreement with BD in September 2013. We do not expect to generate any significant revenue under this agreement in the future.

Research and development expenses. Research and development expenses were $4.1 million for the three months ended September 30, 2014, compared to $3.4 million for the three months ended September 30, 2013. The $0.7 million increase in research and development expenses primarily reflects the following:

- $0.7 million increase in compensation and benefits expense, including employee stock-based compensation expense, relating to an increase in employee headcount to support the clinical development of ProHema and the evaluation of our other product candidates;
- $0.6 million increase in third-party professional consultant and service provider expenses relating to the conduct of our PUMA clinical study and the preparation for the commencement of our PROMPT and PROVIDE clinical studies; and
- $0.3 million increase in expenditures for laboratory equipment and supplies relating to the conduct of our PUMA clinical study and the preparation for the commencement of our PROMPT and PROVIDE clinical studies; which were partially offset by a
- $0.5 million decrease in non-employee stock-based compensation expense; and a
- $0.3 million non-cash charge during the three months ended September 30, 2013 related to the achievement of a pre-clinical milestone under our agreement with the former Verio stockholders.

General and administrative expenses. General and administrative expenses were $1.9 million for the three months ended September 30, 2014, compared to $2.0 million for the three months ended September 30, 2013. The $0.1 million decrease in general and administrative expenses primarily reflects the following:

- $0.4 million increase in compensation and benefits expense, including employee stock-based compensation expense, relating to an increase in employee headcount to support the expansion of our financial and administrative operations; which was offset by a
- $0.4 million decrease in non-employee stock-based compensation expense; and a
- $0.2 million decrease in intellectual property-related expenses.

Other expense, net. Other expense, net was $0.6 million for the three months ended September 30, 2014, compared to $0.9 million for the three months ended September 30, 2013. The change in other expense was primarily due to a fair value charge on the exchangeable share liability of $0.7 million during the three months ended September 30, 2013and a $0.4 million loss on debt extinguishment during the three months ended September 30, 2014 related to the Restated LSA.

Comparison of the Nine Months Ended September 30, 2014 and 2013

The following table summarizes the results of our operations for the nine months ended September 30, 2014 and 2013 (in thousands):

	Nine Months Ended September 30,		Increase / (Decrease)
	2014	2013	
Collaboration revenue	$ —	$ 626	$ (626)
Grant revenue	—	345	(345)
Research and development expense	12,570	8,976	3,594
General and administrative expense	6,391	4,768	1,623
Total other income (expense), net	(689)	(2,382)	(1,693)

Revenue. We did not generate any revenue for the nine months ended September 30, 2014, compared to $1.0 million of revenue generated for the nine months ended September 30, 2013. The decrease was due to the completion of our TATRC grant in May 2013 and the expiration of the three-year joint development period under our license and collaboration agreement with BD in September 2013. We do not expect to generate any significant revenue under these agreements in the future.

Research and development expenses. Research and development expenses were $12.6 million for the nine months ended September 30, 2014, compared to $9.0 million for the nine months ended September 30, 2013. The $3.6 million increase in research and development expenses primarily reflects the following:

- $1.8 million increase in compensation and benefits expense, including employee stock-based compensation expense, relating to an increase in employee headcount to support the clinical development of ProHema and the preclinical development of our other product candidates;
- $1.2 million increase in third-party professional consultant and service provider expenses relating to our preparation for, and the commencement and conduct of, our PUMA clinical study and our preparation for the commencement of our PROMPT and PROVIDE clinical studies; and
- $0.8 million increase in expenditures for laboratory equipment and supplies relating to our preparation and conduct of our clinical trials, and the conduct of our preclinical programs; which were partially offset by a
- $0.4 million decrease in non-employee stock-based compensation expense.

General and administrative expenses. General and administrative expenses were $6.4 million for the nine months ended September 30, 2014, compared to $4.8 million for the nine months ended September 30, 2013. The $1.6 million increase in general and administrative expenses primarily reflects the following:

- $1.2 million increase in compensation and benefits expense, including employee stock-based compensation expense, relating to an increase in employee headcount to support the expansion of our financial and administrative operations; and
- $0.8 million increase in third-party financial and legal professional service provider and insurance expenses to support our operations as a public company; which were partially offset by
- $0.3 million decrease in intellectual property-related expenses; and
- $0.3 million decrease in non-employee stock-based compensation expense.

Other expense, net. Other expense, net was $0.7 million for the nine months ended September 30, 2014, compared to $2.4 million for the nine months ended September 30, 2013. The change in other expense was primarily due to a fair value charge on the exchangeable share liability of $2.0 million during the nine months ended September 30, 2013 and a $0.4 million loss on debt extinguishment during the nine months ended September 30, 2014 related to the Restated LSA.

Liquidity and Capital Resources

We have incurred losses and negative cash flows from operations since inception. As of September 30, 2014, we had an accumulated deficit of $106.2 million and anticipate that we will continue to incur net losses for the foreseeable future.

Operating Activities

Cash used in operating activities increased $5.5 million from $10.8 million for the nine months ended September 30, 2013 to $16.3 million for the nine months ended September 30, 2014. The primary driver of operating cash requirements was the increase in our net loss for 2013 and 2014. During the nine months ended September 30, 2014, we used cash from operating activities of $16.4 million, while our net loss was $19.7 million. The difference was a result of $0.7 million net change in our operating assets and liabilities and $2.6 million of non-cash charges and deferrals, including $1.8 million of stock-based compensation, a $0.4 million non-cash exchangeable share charge related to the achievement of a preclinical milestone under our agreement with the former stockholders of Verio, and $0.4 million of depreciation expense.

Investing Activities

During the nine months ended September 30, 2014 and 2013, investing activities used cash of $0.6 million and $0.1 million, respectively, for the purchase of property and equipment.

Financing Activities

Financing activities provided cash of $8.4 million for the nine months ended September 30, 2014 related primarily to $10.0 million of proceeds from long-term debt, partially offset by the pay down of principal on outstanding long-term debt of $1.8 million. Financing activities provided cash of $20.9 million for the nine months ended September 30, 2013 primarily related to $23.8 million from the issuance of convertible promissory notes, partially offset by $1.5 million from the pay down of principal on our outstanding long-term debt and $1.4 million related to IPO costs.

From our inception through September 30, 2014, we have funded our consolidated operations primarily through the public sale of our common stock, the private placement of preferred stock and convertible notes, commercial bank debt and revenues from collaboration activities and grants. As of September 30, 2014, we had cash and cash equivalents of $45.5 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.

On July 30, 2014, we entered into an Amended and Restated Loan and Security Agreement (the "Restated LSA") with Silicon Valley Bank (the "Bank"), collateralized by substantially all of our assets, excluding certain intellectual property. The Restated LSA amends and restates the Loan and Security Agreement, dated as of January 5, 2009, as amended, by and between the Company and the Bank (the "Loan Agreement"). Pursuant to the Restated LSA, the Bank agreed to make loans to us in an aggregate principal amount of up to $20.0 million, comprised of (i) a $10.0 million term loan, funded at the closing date (the "Term A Loan") and (ii) subject to the achievement of a specified clinical milestone relating to our Phase 2 clinical trial of ProHema, additional term loans totaling up to $10.0 million in the aggregate, which are available until December 31, 2014 (each, a "Term B Loan"), the first of which shall be at least $5.0 million.

The Term A Loan matures on January 1, 2018, and the Term B Loans mature on the first day of the 42nd month after the month in which each Term B Loan funds. The Term A Loan bears interest at a fixed annual rate of 6.94% and the Term B Loans will bear interest at a fixed annual rate, to be determined on the funding date, equal to the greater of (i) 6.75% or (ii) the sum of (a) U.S. Treasury note yield to maturity for a thirty-six (36) month term, plus (b) five hundred ninety (590) basis points. Interest is payable in cash on a monthly basis beginning the first day of each month following the month in which the funding date of each loan occurs. We are required to make a monthly payment of interest only during the first twelve months following the funding date of each loan, and thereafter we are required to repay the principal and accrued interest under each loan in thirty equal monthly installments based on a thirty-month amortization schedule. We are required to make a final payment fee of 7.5% of the funded amount for the Term A Loan and any Term B Loan.

A portion of the proceeds from the Term A Loan were used to repay loans outstanding under the Loan Agreement and to pay for transaction fees related to the Restated LSA, including a commitment fee of $400,000 paid by us to the Bank. The remaining proceeds are expected to be used for working capital purposes, including the advancement of our research programs. Net proceeds from the Term A Loan, after repayment of loans outstanding under the Loan Agreement and transaction fees, were $8.8 million.

As part of the financing, upon our election to access the first Term B Loan, we will issue to the Bank and one or more of its affiliates warrants to purchase up to an aggregate of $0.4 million in shares of our common stock (the "Warrants"), subject to adjustment, at an exercise price equal to the average price per share over the preceding ten (10) trading days prior to the funding date of the first Term B Loan.

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 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 will 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 as of September 30, 2014 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, in addition to the funds available under the Restated LSA with the Bank entered into in July 2014, we 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 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 and maintain the necessary 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.

Contractual Obligations and Commitments

In July 2014, we entered into the Restated LSA with the Bank. Pursuant to the Restated LSA, the Bank agreed to make loans to us in an aggregate principal amount of up to $20.0 million, of which we have drawn on $10.0 million to date. See Note 4 of the Condensed Consolidated Financial Statements for further details.

We have no material contractual obligations not fully recorded on our Condensed Consolidated Balance Sheets or fully disclosed in the notes to the financial statements.

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 3. Quantitative and Qualitative Disclosures about Market Risk

Interest Rate Risk

Our cash and cash equivalents as of September 30, 2014 consisted of cash and money market mutual funds. Our primary exposure to market risk is interest income sensitivity, which is affected by changes in the general level of U.S. interest rates. However, because of the short-term nature of the instruments in our portfolio, a 10% change in market interest rates would not have a material impact on our financial condition and/or results of operations.

Our outstanding debt bears interest at a fixed rate and therefore has no exposure to changes in interest rates.

Item 4. Controls and Procedures

Disclosure Controls and Procedures

We carried out an evaluation, under the supervision and with the participation of our management, including our chief executive officer and chief financial officer, of the effectiveness of the design and operation of our disclosure controls and procedures, as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act as of the end of the period covered by this Quarterly Report on Form 10-Q. In designing and evaluating the disclosure controls and procedures, management recognized 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 this evaluation, our chief executive officer and chief financial officer concluded that our disclosure controls and procedures were effective at the reasonable assurance level as of September 30, 2014.

Changes in Internal Control over Financial Reporting

There were no changes in our internal control over financial reporting that occurred during our latest fiscal quarter that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II. OTHER INFORMATION

Item 1. Legal Proceedings

We are currently not a party to any material legal proceedings or aware of any pending material legal proceedings, 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 results of operations or financial condition. From time to time, we may be subject to various legal proceedings and claims that arise in the ordinary course of our business activities.

Item 1A. Risk Factors

You should carefully consider the following risk factors, as well as the other information in this Quarterly Report on Form 10-Q, and in our other public filings. The occurrence of any of these 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 in our public filings when evaluating our business.

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

If we fail to complete the preclinical or clinical development of, obtain regulatory approval for, or successfully commercialize, ProHema, or otherwise fail to develop or commercialize additional product candidates from our hematopoietic stem cell, or HSC, and Satellite Cell modulation platforms, our business would be significantly harmed.

Our initial product candidate, ProHema, is currently in Phase 2 clinical development. 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 U.S. Food and Drug Administration (FDA) or comparable foreign regulatory authorities in well-designed and conducted clinical trials that the product candidate is manufactured in accordance with FDA requirements, 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, and our product candidates may fail to demonstrate in clinical trials the necessary attributes required for approval. A failure of any one of our product candidates in any 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 marketing 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 profiles that do not potentially outweigh the therapeutic benefit of the product candidates, and whether the regulatory agencies agree that the data from our clinical trials and our manufacturing processes are sufficient to support approval for any of our product candidates. The final results of our current and future clinical trials may not meet the FDA's or other regulatory agencies' requirements to approve a product candidate for marketing, and the regulatory agencies may otherwise determine that our manufacturing processes or facilities are insufficient to support approval. We may need to conduct more preclinical studies and clinical trials than we currently anticipate. Even if we do receive FDA or other regulatory agency approval to market our product candidates, we may not be successful in commercializing approved product candidates.

Our business would be materially harmed, and the value of our common stock would likely decline, if we fail to complete preclinical or clinical development, obtain regulatory approval, or successfully commercialize our product candidates.

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 continue enrolling patients in the PUMA study;
- delays in our ability to initiate, or enroll patients in, the PROMPT or PROVIDE studies;
- the introduction of our NRM formulation into our ongoing and planned clinical trials, and the introduction of our reduced volume formulation into our planned pediatric clinical trials, may not produce the benefits that we currently anticipate or may result in unanticipated adverse effects;

- the occurrence of unexpected safety events in any current or subsequent clinical trial of ProHema may impact our ability to conduct or complete any ongoing or planned clinical trial of ProHema;
- 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;
- delays in 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;
- failure of third-party service providers and clinical trial sites to ensure the proper and timely conduct of our clinical trials;
- failure of clinical trial sites to manufacture ProHema consistently under the correct conditions at their cell processing facilities for use in our clinical trials;
- delays by third-party manufacturers in the production of, or their failure 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 or difficulties in achieving study endpoints and completing data analysis for a trial;
- regulators or clinical site institutional review boards, or IRBs, may not authorize us to commence or recommence a clinical trial;
- data 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 fail to 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 that 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, and that ProHema used in registrational clinical trials must be manufactured in compliance with FDA regulatory requirements. In addition, the FDA may impose additional requirements on our processes for the manufacture of ProHema. Any requirements to modify our manufacturing processes, and any delays in, or inability to, establish manufacturing processes acceptable to the FDA could require us to incur additional development costs or result in

further delays to our clinical development plans, or could create uncertainty and additional complexity in our ability to obtain regulatory approval for ProHema. Any such events could delay or prevent our ability to obtain regulatory approval or commercialize our product candidates, which would adversely impact our business, financial condition and results of operations.

Moreover, our development costs may increase because we may be required to complete additional or larger clinical trials for product candidates prior to FDA or other regulatory approval. We may not have adequate capital or other resources to commence or complete these additional or larger trials. If we experience delays in the completion of 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 would significantly harm our business, financial condition and prospects.

The preclinical and clinical development of our product candidates, including ProHema, 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 research and development activities and operations.

We are currently advancing ProHema through clinical development and conducting other research and development activities. Developing therapeutic products, including conducting preclinical studies and clinical trials of cell therapeutics, 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.

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 the PUMA study, the PROMPT study, the PROVIDE study and any subsequent clinical trials of ProHema;
- the progress, costs, results and timing of our ongoing and any additional research and development activities, including activities in connection with our Wnt7a analogs and iPSC-derived cell therapeutics;
- our ability to initiate, and the size, progress, costs, results and timing of additional future clinical trials, including any registrational clinical trials for ProHema, 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 our development programs or operations. 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 any other product candidates that we may develop, 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, prospects, and market price of shares of our common stock.

Interim results from ongoing clinical trials and results from preclinical studies and earlier clinical trials do not ensure that our ongoing or 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 the development of any of our product candidates will ultimately be successful. For example, although an independent data monitoring committee, or iDMC, supported the continuation of our PUMA study based upon the first of two scheduled interim data reviews, the PUMA study has not been completed and the first interim data review, which was based upon data from a limited number of subjects who are still under evaluation in the study and subject to ongoing safety surveillance, may not be predictive of safety or efficacy for ProHema in the PUMA study. In addition, although the results of our completed Phase 1b ProHema-01 study in adults with hematologic malignancies undergoing double umbilical cord blood transplant demonstrated human proof-of-concept, we may not achieve or duplicate these results in the PUMA study or in planned additional clinical trials of ProHema, including the PROMPT or PROVIDE studies in pediatric patients.

The results of our ongoing and future clinical trials, including our PUMA, PROMPT and PROVIDE studies, may differ from interim results or from results achieved in earlier clinical trials or in preclinical studies for a variety of reasons, including:

- unexpected safety events may occur in patients enrolled in the PUMA study, including patients who have already been reviewed by the iDMC, but remain subject to ongoing safety surveillance, and patients who have not yet been reviewed by the iDMC;
- 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 ProHema-01 study;
- 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 currently manufactured at cell processing facilities at each clinical center, across all participating clinical centers in the PUMA study or in any future clinical trials that we may conduct, including the PROMPT or PROVIDE studies;
- differences in the design of the Phase 2 PUMA study, 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 the FDA for entry into Phase 2 or subsequent clinical trials may make it less effective than the product

manufactured during our Phase 1b ProHema-01 study or may otherwise adversely affect the safety, purity, potency or effectiveness of ProHema; and

- we have not previously evaluated ProHema, or the reduced volume formulation of ProHema, in pediatric patients, and pediatric patients in our planned PROMPT and PROVIDE studies or subsequent clinical trials may experience side effects or other adverse events not observed in adult patients.

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 our ongoing and planned clinical trials are successful, we will need to conduct registrational clinical trials and 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 ongoing and future clinical trials would substantially harm our business and prospects.

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

We are 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 diseases and rare genetic disorders, 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 hematopoietic stem cell transplants, or 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, including in our PUMA, PROMPT and PROVIDE studies of ProHema, is affected by factors including:

- the ability to identify, solicit and recruit a sufficient number of patients;
- severity of the disease under investigation;
- design of the trial protocol;
- the relatively small size and nature of the patient population for the trial in question;
- 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 our clinical trials are or will be conducted;
- 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.

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

In December 2012, we initiated a Phase 2 clinical trial of ProHema in adult patients undergoing double umbilical cord blood transplant for hematologic malignancies, or the ProHema-03 study, where standard processing media was used for the manufacture of ProHema. We notified the FDA in May 2013 of our election to pause enrollment of the ProHema-03 study to incorporate the use of our NRM formulation in the manufacture of ProHema. 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. We also submitted an amended protocol defining how we planned to resume our clinical development of ProHema using our NRM formulation.

In March 2014, we initiated enrollment of our Phase 2 clinical trial of ProHema using our NRM formulation in adult patients undergoing double umbilical cord blood transplant for hematologic malignancies, or the PUMA study, following our submission to the FDA of manufacturing and product data for ProHema, which were generated using the NRM formulation intended for clinical use.

We submitted an amendment to our existing Investigational New Drug (IND) application for ProHema and received clearance from the FDA in April 2014 to proceed with conducting a clinical trial of ProHema in pediatric patients undergoing single umbilical cord blood transplant for the treatment of hematologic malignancies, or the PROMPT study, under this amended IND application. Our IND amendment for the PROMPT study details the clinical protocol for the conduct of the trial and includes information on how we plan to conduct the clinical trial with a formulation of ProHema having a reduced volume for the treatment of pediatric patients.

We also filed a new IND application in June 2014 for the clinical development of ProHema in pediatric patients undergoing single umbilical cord blood transplant for the treatment of inherited metabolic disorders, or IMDs, including certain lysosomal storage disorders, or LSDs. Our IND application for the treatment of pediatric patients with IMDs, or the PROVIDE study, details the clinical protocol for the conduct of the trial and includes data supporting our planned use of a reduced volume formulation of ProHema in pediatric patients. In July 2014, we received clearance from the FDA to proceed with conducting the PROVIDE study.

The FDA has indicated that our plans to conduct the PROMPT study and the PROVIDE study using a reduced volume formulation of ProHema is acceptable for patients to be enrolled in the study. Although we are presently conducting the PUMA study and have received FDA clearance to initiate enrollment in the PROMPT and PROVIDE studies, the FDA may require us to generate additional preclinical, product or clinical data, including data supporting the use of our NRM formulation in these studies, as a condition to continuing or initiating these trials and any other subsequent clinical trials of ProHema. The FDA may also require additional data to support the use of our reduced volume formulation of ProHema in our planned studies in pediatric patients, including the PROMPT and PROVIDE studies, or may impose other additional requirements for our clinical development activities for ProHema. Additionally, the FDA may in the future have comments, or impose requirements, on our protocols for conducting the PUMA, PROMPT, or PROVIDE studies, or any other subsequent clinical trials of ProHema. Any requirements to generate additional data or modify our protocols, or other additional comments, requirements or impositions by the FDA, may cause delays in the conduct of the PUMA study, the PROMPT study or the PROVIDE study, or other subsequent 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 patient enrollment and in the conduct of, and the availability of data from, the PUMA, PROMPT or PROVIDE studies.

Further, if the results of our clinical studies are inconclusive or if there are safety concerns or adverse events associated with our product candidates, we may:

- be delayed in obtaining regulatory approval for our product candidates, if at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings or contraindications;
- be required to perform additional clinical studies to support approval or be subject to additional post-marketing testing requirements; or
- have regulatory authorities withdraw their approval of the product or impose restrictions on its use.

Patients undergoing treatment with ProHema may also receive chemotherapy, radiation, and/or other high dose or myeloablative treatments in the course of treatment of their disease, and may therefore experience side effects or adverse events that are unrelated to our product candidates. While these side effects or adverse events are unrelated to our product candidates, they may still impact the success of our clinical studies. The inclusion of critically ill patients in our clinical studies may result in deaths or other adverse medical events due to other therapies or medications that such patients may be using. Any of these events could prevent us from advancing ProHema or other product candidates through clinical development, and from obtaining FDA approval, and would impair our ability to commercialize our product candidates. Any inability to advance ProHema or any other product candidate through clinical development would have a material adverse effect on our business, and the value of our common stock would decline.

Our planned clinical development activities for ProHema in pediatric patients, including our PROMPT and PROVIDE studies, present additional operational, technical and timing risks.

Many clinical centers that could potentially participate in our pediatric clinical trials of ProHema are distinct and separate from the centers participating in the PUMA study, and finding sufficient, capable centers that would be interested in participating in our pediatric trials may take additional time. There are fewer eligible patients with hematologic malignancies and rare genetic disorders for our PROMPT and PROVIDE studies because the total number of pediatric patients who undergo allogeneic HSCT for the treatment of such diseases and disorders is lower than it is in adults. This may increase the time to commencement of our planned and future pediatric studies, or may delay or limit our ability to enroll patients in these studies, and any of these events may impair our ability to complete our planned and future pediatric studies, including our PROMPT and PROVIDE studies.

Further, to evaluate ProHema in pediatric patients, we have created a reduced volume formulation of ProHema for children, due to their smaller size and requirement for smaller infusion volume. Although we have received permission from the FDA to use a formulation of ProHema having a reduced volume for the treatment of pediatric patients in our planned PROMPT and PROVIDE studies, the FDA may require us to generate additional preclinical, product, or clinical data to support the use of any reduced volume formulation of ProHema in these studies prior to or following their commencement, or in any subsequent trials of ProHema, or may impose other restrictions on the use of any reduced volume formulation of ProHema. Any such requirement or imposition may present technical challenges and may cause further delays in the commencement or conduct of our planned pediatric clinical trials, including the PROMPT and PROVIDE studies. Any delays in our planned clinical development activities for pediatric patients would have an adverse effect on our business operations.

In addition, the reduced volume formulation of ProHema that we plan to use in our PROMPT and PROVIDE studies may not have the same functional and biological characteristics as the formulation of ProHema used in our preclinical studies and our PUMA study. The reduced volume formulation of ProHema may not demonstrate the same or similar results as shown in earlier studies, or may result in unanticipated side effects or other adverse events not observed in studies using the standard volume formulation of ProHema.

Our product candidates from our HSC and Satellite Cell modulation platforms are currently undergoing research and preclinical development, which may not be successful. If we are unable to successfully complete preclinical studies and development of our product candidates, our business will be harmed.

Product candidates from our HSC and Satellite Cell modulation platform are still in research and preclinical development, and we cannot assure you that any product candidates identified from our HSC and Satellite Cell modulation platforms, including our Wnt7a analogs and any induced pluripotent stem cell-derived myogenic progenitor cell therapeutic, or iMPC, will demonstrate the safety, purity and efficacy profile necessary to support further preclinical study, clinical development or regulatory approval.

We may delay or cancel our ongoing research and development activities for our product candidates, including our Wnt7a analogs or any iMPC therapeutics, for a variety of reasons, including:

- the results of our ongoing or future research and development activities may not demonstrate safety or a meaningful therapeutic benefit or support further development of, or may require us to significantly modify our development plans for, our product candidates;
- difficulty identifying, designing or establishing appropriate, reproducible and predictive preclinical models for demonstration of safety and efficacy of our product candidates in one or more potential therapeutic areas for clinical development;
- the occurrence of product manufacturing difficulties, including the inability to manufacture our product candidates in a sufficient quantity or in a cost-effective manner, or to formulate our product candidates in a suitable form for use in our planned preclinical studies;
- the occurrence of adverse events associated with our product candidates in preclinical development including in, or even after successful initial toxicology studies, that are viewed to outweigh their potential benefits;
- difficulty in establishing or maintaining manufacturing relationships with third parties on acceptable terms, or in establishing or maintaining our own manufacturing capability, to produce and supply our product candidates in sufficient quantity to support research and development of such product candidates; or
- our inability to secure strategic partners which may be necessary for advancement of our product candidates into clinical development or commercialization.

Any delay in, or cessation of, our research and development activities could materially harm our business.

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 these new technologies 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 in the United States or other jurisdictions for ProHema or any other product candidates that we may develop. We face regulatory uncertainty associated with the development of our product candidates, including ProHema, due to the requirement that ProHema be manufactured in close proximity to transplant centers within a short period of time before transplantation, which may present unprecedented additional complexities associated with ensuring consistent manufacture in compliance with FDA regulatory requirements, the rapid release nature of ProHema, and the degree of qualification testing necessary for administration of ProHema.

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, the FDA or other regulatory bodies may change the requirements or modify the potential regulatory pathways for approval of any of our product candidates. These regulatory authorities and advisory groups, or anynew 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 pathways, 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. Delays or unexpected costs in obtaining, or the failure to obtain, 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, requirements relating to current good manufacturing practices, or cGMP, 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, delay regulatory approval or modify the potential regulatory pathway 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.

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. 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 over available therapies on a clinically significant endpoint(s). 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 that ProHema or future product candidates that we may develop 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. 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 certain of 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 ProHema and potential future product candidates that we may develop. Orphan drug status confers seven years of marketing exclusivity in the United States 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, any 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 in the United States, 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 our 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.

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 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 obtain approval for, or commercialize ProHema.

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, including our oversight for facility and raw material and vendor qualification through to final product analytical testing and release. The manufacture of ProHema for use in registrational clinical trials and commercialization will be subject to the requirements of applicable regulatory authorities, including the FDA, and the use of our current manufacturing processes to manufacture ProHema for commercialization may require the clinical cell processing facilities at which ProHema is manufactured to be approved by applicable regulatory authorities, including the FDA, pursuant to inspections that would be conducted after a BLA, or other marketing application is submitted. Although we are responsible for ensuring compliance with applicable regulatory requirements 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. Because we do not control the activities of these clinical cell processing facilities, we 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 may be required to identify alternative processes for the manufacture of ProHema, and our ability to commercialize ProHema may be impaired. To comply with applicable regulatory requirements and our protocols for the manufacture of ProHema, the clinical cell processing facility may be required to possess or obtain certain equipment, 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. Clinical cell processing facilities may be unwilling or unable to comply with these regulatory requirements or with our protocols for the manufacture of ProHema, which could restrict or prohibit a given clinical center from manufacturing ProHema and making it available for administration to patients. 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 depend on third-party suppliers for various components of our 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 for use in our ongoing and planned clinical trials, including our PUMA, PROMPT and PROVIDE studies, and any other subsequent clinical trials of ProHema. 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 any Phase 3 clinical trial or any other future clinical trials. In addition, if we are unable to secure adequate quantities of these components from our preferred suppliers, we may be required to identify alternate suppliers of these components, or to modify our NRM formulation. If we are required to change suppliers of our components, or modify our NRM formulation, the efficacy and potency of ProHema may be adversely affected. We also may be required to make further changes to our trial protocol or provide additional data to the FDA regarding the use of alternative components for our NRM formulation or a modified NRM formulation, which could delay our clinical development plans for ProHema and increase the costs required to complete our planned clinical development of ProHema. Any changes to our suppliers or components, modifications of our NRM formulation, or any delays to our clinical development plans, could adversely impact our clinical development of ProHema and harm our business.

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.

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 used both unlicensed and licensed 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 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 registrational clinical trials must be conducted with product produced under applicable regulatory 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 service 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 ongoing 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.

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.

We expect to rely on third parties for the manufacture of our product candidates.

We do not expect to independently conduct all aspects of our product manufacturing, and may rely on third-party manufacturers for the manufacture of any product candidates that we may develop. These third-party manufacturers will be required to comply with applicable FDA regulatory requirements 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, we anticipate that the manufacture of our product candidates will be difficult, and it is possible that any third-party manufacturers that we engage may experience delays or technical challenged in such manufacture. Any of these factors would significantly impact our ability to develop, obtain regulatory approval for or commercialize our product candidates, and would adversely affect our business.

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 for our current and future product candidates, the processes used to manufacture them and the methods for using them, as well as successfully defending our proprietary rights against third-party challenges. We are the owner of record of various 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 own a number of patent applications pending in the United States and in certain foreign jurisdictions relating to our product candidates. We are currently not the owner of record of any patents specifically covering our product candidates, and we cannot be certain that any patents will issue to us with claims that cover our ProHema and other product candidates.

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

The patent positions of biotechnology and pharmaceutical companies can be highly uncertain and involve complex legal and factual questions for which important legal principles remain unresolved. No consistent policy regarding the breadth of claims allowed in pharmaceutical patents has emerged to date in the United States or in foreign jurisdictions outside of the United States. Changes in either the patent laws or interpretations of patent laws in the United States and other countries may diminish the value of our intellectual property. Accordingly, we cannot

predict the breadth of claims that may be enforced in the patents that may be issued from the applications we currently, or may in the future, own or license from third parties. Further, if any patents we obtain or license are deemed invalid and unenforceable, our ability to commercialize or license our technology could be adversely affected.

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

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

- others may be able to 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 the exclusive licensee of over 80 issued or pending U.S. and non-U.S. patents or patent applications relating to ProHema and other therapeutic compositions of stem cells that have been pharmacologically modulated to enhance their therapeutic properties, methods of manufacturing the cellular compositions, and methods of promoting hematopoietic reconstitution, expansion and self-renewal using modulators that increase prostaglandin signaling activity.

We have exclusive licenses to over 30 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.

We have exclusive licenses to over 100 patents and patent applications relating to our induced pluripotent stem cell (iPSC) technology, covering compositions of matter, processes for preparing such iPSCs and uses of iPSCs.

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.

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
- 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 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 patents if a third party files with the patent office first. Other countries have similar laws that permit secrecy of patent applications, and such patent applications 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 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 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 product candidates 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 or recommend these therapeutics;
- the processes used to manufacture our product candidates, including ProHema, which may require cell processing facilities of transplant centers to comply with FDA regulatory requirements;

- 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 pathways and 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;
- 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. If pricing is set at unsatisfactory levels, our ability to successfully market and sell our product candidates will be adversely affected.

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

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

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

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

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 diseases and rare genetic disorders. 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.

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. In addition, many universities and private and public research institutes may develop technologies or secure intellectual property of interest to us, but license them to our competitors. Our competitors may succeed in developing, acquiring or licensing on an exclusive basis, technologies and drug products that are more effective or less costly than ProHema or any other product candidates that we are currently developing or that we may develop, which could render our products obsolete and noncompetitive.

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

- the results of our preclinical studies and clinical trials;
- our ability to recruit and enroll patients for our clinical trials;
- the efficacy, safety and reliability of our product candidates;
- the speed at which we develop our product candidates;

- our ability to design and successfully execute appropriate clinical trials;
- our ability to protect and develop intellectual property rights related to our products;
- our ability to maintain a good relationship with regulatory authorities;
- the timing and scope of regulatory approvals, if any;
- our ability to commercialize and market any of our product candidates that receive regulatory approval;
- market perception and acceptance of stem cell therapeutics;
- acceptance of our product candidates by physicians and institutions that perform HSCTs;
- the price of our products;
- adequate levels of reimbursement under private and governmental health insurance plans, including Medicare; and
- our ability to manufacture and sell commercial quantities of any approved products to the market.

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

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

As we increase the number of our ongoing clinical trials, advance our product candidates through preclinical studies and clinical trials and undertake any additional product development programs, 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;
- develop a marketing and sales infrastructure in the event we receive regulatory approval for any product candidate; 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 our growth and the increased complexity of our operations, our business may be adversely affected.

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

We may not be able to attract or retain qualified management, finance, scientific and clinical personnel and consultants due to the intense competition for qualified personnel and consultants among biotechnology,

pharmaceutical and other businesses. If we are not able to attract and retain necessary personnel and consultants to accomplish our business objectives, we may experience constraints that will significantly impede the achievement of our development objectives, our ability to raise additional capital and our ability to implement our business strategy.

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

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

We are party to a loan and security agreement that contains operating and financial covenants that may restrict our business and financing activities.

In July 2014, we entered into an amended and restated loan and security agreement with Silicon Valley Bank, pursuant to which we have been extended a term loan in the principal amount of $10.0 million and may borrow up to an additional $10.0 million in term loans, subject to certain conditions. Borrowings under this loan and security agreement are secured by substantially all of our assets, excluding certain intellectual property rights. The loan and security agreement restricts our ability, among other things, to:

- sell, transfer or otherwise dispose of any of our business or property, subject to limited exceptions;
- make material changes to our business or management;
- enter into transactions resulting in significant changes to the voting control of our stock;
- make certain changes to our organizational structure;
- consolidate or merge with other entities or acquire other entities;
- incur additional indebtedness or create encumbrances on our assets;
- pay dividends, other than dividends paid solely in shares of our common stock, or make distributions on and, in certain cases, repurchase our stock;
- enter into transactions with our affiliates;
- repay subordinated indebtedness; or
- make certain investments.

In addition, we are required under our loan agreement to comply with various affirmative operating covenants. The operating covenants and restrictions and obligations in our loan and security agreement, as well as any future financing agreements that we may enter into, may restrict our ability to finance our operations, engage in business activities or expand or fully pursue our business strategies. Our ability to comply with these covenants may be affected by events beyond our control, and we may not be able to meet those covenants. A breach of any of these covenants could result in a default under the loan and security agreement, which could cause all of the outstanding indebtedness under the facility to become immediately due and payable and eliminate our eligibility to receive additional loans under the agreement.

If we are unable to generate sufficient cash available to repay our debt obligations when they become due and payable, either as when such obligations become due, when they mature, or in the event of a default, we may not be able to obtain additional debt or equity financing on favorable terms, if at all, which may negatively impact our business operations and financial condition.

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 cause 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 and we believe our product liability insurance coverage is sufficient in light of our current clinical programs; however, we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses due to liability. If and when we obtain marketing approval

for product candidates, we intend to expand our insurance coverage to include the sale of commercial products; however, we may be unable to obtain product liability insurance on commercially reasonable terms or in adequate amounts. On occasion, large judgments have been awarded in class action lawsuits based on drugs or medical treatments that had unanticipated adverse effects. In addition, under some of our agreements with clinical trial sites, we are required to indemnify the sites and their personnel against product liability and other claims. A successful product liability claim or series of claims brought against us or any third parties whom we are required to indemnify could cause our stock price to decline and, if judgments exceed our insurance coverage, could adversely affect our results of operations and business.

Patients with the diseases targeted by our product candidates are often already in severe and advanced stages of disease and have both known and unknown significant pre-existing and potentially life-threatening health risks. During the course of treatment, patients may suffer adverse events, including death, for a variety of reasons. Such events, whether or not resulting from our product candidates, could subject us to costly litigation, require us to pay substantial amounts of money to injured patients, delay, negatively impact or end our opportunity to receive or maintain regulatory approval to market our products, or require us to suspend or abandon our commercialization efforts. Even in a circumstance in which we do not believe that an adverse event is related to our products, the investigation into the circumstance may be time-consuming or inconclusive. These investigations may interrupt our development and commercialization efforts, delay our regulatory approval process, or impact and limit the type of regulatory approvals our product candidates receive or maintain. As a result of these factors, a product liability claim, even if successfully defended, could have a material adverse effect on our business, financial condition or results of operations.

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

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

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

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

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

A significant natural disaster, such as an earthquake, fire, flood or drought, 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, drought 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 Our Financial Condition and the Ownership of Our Common Stock

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 and as of September 30, 2014, we had an accumulated deficit of approximately $106.2 million. We expect to continue to incur losses for the foreseeable future, including in connection with our ongoing and planned clinical trials of ProHema and our other ongoing and planned research and development activities. 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 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 research and 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.

Market volatility may affect our stock price.

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, and the availability of data from, our clinical trials and preclinical studies;
- 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 regulatory pathways and 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 other diseases targeted by our product candidates;
- actual or anticipated fluctuations in our financial condition and operating results;
- actual or anticipated changes in our product development activities and business prospects 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 or business 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.

As of November 7, 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 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 or affecting the liquidity and volatility of our common stock, 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 expect that significant additional capital will be needed in the future to continue our planned operations, and 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, the market price of our common stock and on our operations. In addition, if we seek funds through arrangements with collaborative partners, these arrangements may require us to relinquish rights to some of our technologies, intellectual property or product

candidates, issue additional equity or debt securities, or otherwise agree to terms unfavorable to us. We have filed a registration statement on Form S-3 that provides for the sale of up to $100 million aggregate of common stock, preferred stock, debt securities, warrants and/or units by us from time to time in one or more offerings. The registration statement became effective on October 15, 2014. If we sell common stock, preferred stock or other securities that may be converted, exercised or exchanged for shares of our capital stock, such sale or issuance of securities may result in dilution to our stockholders and may cause the market price of our stock to decline, and new investors could gain rights superior to our existing stockholders. In addition, in July 2014, we entered into an amended and restated loan and security agreement with Silicon Valley Bank, which imposes restrictive covenants on our operations. Any future debt financings may impose additional restrictive covenants or otherwise adversely affect the holdings or the rights of our stockholders.

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

In March 2014, approximately 12.4 million shares of our common stock became eligible for sale by our shareholders in the public market upon the expiration of lock-up agreements that were entered into in connection with our initial public offering, or IPO, although a portion of such shares held by our affiliates are subject to volume limitations and other conditions pursuant to Rule 144 of the Securities Act. Certain holders of our common stock have the right to require us to register these shares under the Securities Act pursuant to an investor rights agreement. In addition, a number of our employees, including executive officers, have adopted and may continue to adopt stock trading plans pursuant to which they have arranged to sell shares of our common stock from time to time in accordance with the guidelines specified under Rule 10b5-1 of the Securities Exchange Act of 1934, as amended. Further, the shares subject to outstanding options under our equity incentive plans will, and the shares reserved for future issuance under our equity incentive plans may, become eligible for sale in the public market in the future, subject to certain legal and contractual limitations. If our existing stockholders, including our employees and executive officers, 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. Actual or potential sales by our existing stockholders, including our employees and executive officers, could also be viewed negatively by other investors.

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 have considerable discretion in the application of our existing cash, including the net proceeds from our IPO and the proceeds available to us under our loan and security agreement with Silicon Valley Bank, and expect to have similar discretion over the use of any additional funds that we may raise. 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 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 other key 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 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 intend to continue availing 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 have taken, and intend to continue 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 lower than otherwise or 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, and we are subject to restrictions on the payment of dividends under our loan and security agreement with Silicon Valley Bank. 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 IPO. 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.

Table of Contents

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds

a) None.

b) None.

c) None.

Item 3. Defaults Upon Senior Securities

None.

Item 4. Mine Safety Disclosure

Not applicable.

Item 5. Other Information

None.

Item 6. Exhibits

See Exhibit Index.

SIGNATURES

Pursuant to the requirements 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.

Fate Therapeutics, Inc.

Date: November 12, 2014

By: /s/ Christian Weyer
Christian Weyer
President and Chief Executive Officer
(Principal Executive Officer)

By: /s/ J. Scott Wolchko
J. Scott Wolchko
Chief Financial Officer and Chief Operating Officer
(Principal Financial and Accounting Officer)

Table of Contents

Index to Exhibits

Exhibit Number	Description of Document
3.1	Amended and Restated Certificate of Incorporation (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).
3.2	Amended and Restated Bylaws (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).
4.1	Specimen Common Stock Certificate (filed as Exhibit 4.1 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).
4.2	Form of Warrant to Purchase Common Stock issuable to Silicon Valley Bank and its affiliates. (1)
10.1	Amended and Restated Loan and Security Agreement, dated as of July 30, 2014 by and between the registrant and Silicon Valley Bank. (1)
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
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document

(1) Filed as an exhibit to the Company's Current Report on Form 8-K (File No. 001-36076), filed with the SEC on August 5, 2014 and incorporated herein by reference.

Exhibit 31.1

CERTIFICATION OF PRINCIPAL EXECUTIVE OFFICER
PURSUANT TO RULE 13a-14(a) AND 15d-14(a) OF THE SECURITIES EXCHANGE ACT, AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Christian Weyer, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Fate Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:

a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

b. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

c. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 12, 2014

/s/ Christian Weyer
Christian Weyer
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION OF PRINCIPAL FINANCIAL OFFICER
PURSUANT TO RULE 13a-14(a) AND 15d-14(a) OF THE SECURITIES EXCHANGE ACT, AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, J. Scott Wolchko, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Fate Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:

a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

b. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

c. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 12, 2014

/s/ J. Scott Wolchko
J. Scott Wolchko
Chief Financial Officer and Chief Operating Officer
(Principal Financial Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the quarterly report of Fate Therapeutics, Inc. (the "Company") on Form 10-Q for the period ended September 30, 2014 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Christian Weyer, President and Chief Executive Officer of the Company, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to my knowledge:

(1) The Report fully complies with the requirements of Section 13(a) or 15(d), as applicable, of the Securities Exchange Act of 1934, as amended; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: November 12, 2014

/s/ Christian Weyer
Christian Weyer
President and Chief Executive Officer

The foregoing certification is being furnished solely pursuant to 18 U.S.C. Section 1350 and is not being filed as part of the Report or as a separate disclosure document.

Exhibit 32.2

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the quarterly report of Fate Therapeutics, Inc. (the "Company") on Form 10-Q for the period ended September 30, 2014, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, J. Scott Wolchko, Chief Financial Officer of the Company, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to my knowledge:

(1) The Report fully complies with the requirements of Section 13(a) or 15(d), as applicable, of the Securities Exchange Act of 1934, as amended; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: November 12, 2014

/s/ J. Scott Wolchko
J. Scott Wolchko
Chief Financial Officer and Chief Operating Officer

The foregoing certification is being furnished solely pursuant to 18 U.S.C. Section 1350 and is not being filed as part of the Report or as a separate disclosure document.