FATE THERAPEUTICS INC
Form 10-Q
August 12, 2014
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UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended June 30, 2014

OR

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

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

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 x No o

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 x No o

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 o	Accelerated filer o
Non-accelerated filer o	Smaller reporting company x
(Do not check if a 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 o No x

As of August 7, 2014, there were 20,562,773 shares of the registrant s common stock, par value $0.001 per share, outstanding.

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FATE THERAPEUTICS, INC.

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PART I. FINANCIAL INFORMATION

Item 1. Financial Statements

Fate Therapeutics, Inc.

Condensed Consolidated Balance Sheets

(in thousands, except share and per share data)

	June 30, 2014 (unaudited)	December 31, 2013
Assets		
Current assets:		
Cash and cash equivalents	$ 42,012	$ 54,036
Prepaid expenses and other current assets	233	615
Total current assets	42,245	54,651
Property and equipment, net	1,159	810
Restricted cash	122	122
Other assets	25	
Total assets	$ 43,551	$ 55,583
Liabilities and stockholders equity		
Current liabilities:		
Accounts payable	$ 980	$ 682
Accrued expenses	1,948	2,039
Current portion of deferred rent	69	53
Repurchase liability for unvested equity awards	69	94
Long-term debt, current portion	746	1,732
Total current liabilities	3,812	4,600
Deferred rent	101	135
Commitments and contingencies (Note 4)		
Stockholders equity:		
Preferred stock, $0.001 par value; authorized shares 5,000,000 at June 30, 2014 and December 31, 2013; no shares issued or outstanding		
Common stock, $0.001 par value; authorized shares 150,000,000 at June 30, 2014 and December 31, 2013; issued and outstanding shares 20,561,477 at June 30, 2014 and 20,434,080 at December 31, 2013	21	20
Additional paid-in capital	139,173	137,337
Accumulated deficit	(99,556)	(86,509)
Total stockholders equity	39,638	50,848
Total liabilities and stockholder s equity	$ 43,551	$ 55,583

See accompanying notes.

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Fate Therapeutics, Inc.

Condensed Consolidated Statements of Operations and Comprehensive Loss

(in thousands, except share and per share data)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2014	2013	2014	2013
	(unaudited)			
Revenues:				
Collaboration revenue	$	$ 208	$	$ 417
Grant revenue		82		345
Total revenue		290		762
Operating expenses:				
Research and development	3,968	3,067	8,490	5,598
General and administrative	2,072	1,492	4,487	2,789
Total operating expenses	6,040	4,559	12,977	8,387
Loss from operations	(6,040)	(4,269)	(12,977)	(7,625)
Other income (expense):				
Interest income	1		1	1
Interest expense	(28)	(88)	(71)	(188)
Change in fair value of exchangeable shares		(1,155)		(1,260)
Change in fair value of warrant liability		(22)		(10)
Total other expense, net	(27)	(1,265)	(70)	(1,457)
Net loss and comprehensive loss	$ (6,067)	$ (5,534)	$ (13,047)	$ (9,082)
Net loss per common share, basic and diluted	$ (0.30)	$ (4.46)	$ (0.64)	$ (7.41)
Weighted-average common shares used to compute basic and diluted net loss per share	20,467,782	1,239,524	20,407,632	1,226,451

See accompanying notes.

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Fate Therapeutics, Inc.

Condensed Consolidated Statements of Cash Flows

(in thousands)

	Six Months Ended June 30,	
	2014	2013
	(unaudited)	
Operating activities		
Net loss	$ (13,047)	$ (9,082)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	256	302
Issuances of common stock for technology		13
Stock-based compensation	1,295	187
Amortization of discounts	14	35
Noncash interest expense	17	42
Deferred rent	(18)	(119)
Deferred revenue		(42)
Stock-based milestone charges and change in fair value of exchangeable shares	375	1,260
Change in fair value of preferred stock warrants		10
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	356	(721)
Accounts payable and accrued expenses	8	792
Net cash used in operating activities	(10,744)	(7,323)
Investing activities		
Purchase of property and equipment	(424)	(71)
Net cash used in investing activities	(424)	(71)
Financing activities		
Issuance of common stock, net of repurchases and issuance costs	144	1
Proceeds from (cost of) initial public offering, net of offering costs		(295)
Issuance of convertible promissory notes		3,000
Payments on long-term debt	(1,000)	(1,000)
Net cash (used in) provided by financing activities	(856)	1,706
Net change in cash and cash equivalents	(12,024)	(5,688)
Cash and cash equivalents at beginning of the period	54,036	9,087
Cash and cash equivalents at end of the period	$ 42,012	$ 3,399

See accompanying notes.

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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. Based on the Company s understanding of key biological mechanisms that guide the fate of adult stem cells, the Company has built two platforms that optimize the activity and enhance the therapeutic potential of adult stem cells: its hematopoietic stem cell, or HSC, modulation platform and its muscle satellite stem cell, or Satellite Cell, modulation platform.

As of June 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.

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

Revenue arrangements with multiple elements may include license fees, research and development payments, milestone payments, other contingent payments, and royalties on any product sales derived from collaborations. The Company recognizes nonrefundable license fees with stand-alone value as revenue at the time that the Company has satisfied all performance obligations, and recognizes license fees without stand-alone value as revenue in combination with any undelivered performance obligations. The Company recognizes a research and development payment as revenue over the term of the collaboration agreement as contracted amounts are earned, or reimbursable costs are incurred, under the agreement, where contracted amounts are considered to be earned in relative proportion to the performance required under the applicable agreement. The Company recognizes a milestone payment, which

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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 80,645 shares and 111,021 shares for the three months ended June 30, 2014 and 2013, respectively, and 84,344 shares and 115,922 shares for the six months ended June 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 six months ended June 30, 2014, the Company realized a net loss of $6.1 million and $13.0 million, respectively. Shares of potentially dilutive securities totaled 2.4 million for the three and six months ended June 30, 2014, including options to purchase 2.4 million shares of common stock.

For the three and six months ended June 30, 2013, the Company realized a net loss of $5.5 million and $9.1 million, respectively. Shares of potentially dilutive securities totaled 9.2 million for each of the three and six months ended June 30, 2013.

Recent Accounting Pronouncements

In June 2014, The Financial Accounting Standards Board (or FASB), issued Accounting Standards Update Number 2014-10 (ASU 2014-10). ASU 2014-10 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 state 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 has elected the early adoption of ASU 2014-10 beginning with this Quarterly Report on Form 10-Q, and will no longer disclose inception-to-date information or incremental financial reporting requirements related to development stage entities.

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In May 2014, the FASB issued ASU 2014-09, which creates 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 and future operating results.

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 six months ended June 30, 2014, based on the assessed 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 six months ended June 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 upon the achievement of stock-based milestones and the initial fair value of the 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

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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 June 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 June 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 June 30, 2014 and December 31, 2013, the Company had no 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):

	June 30, 2014	December 31, 2013
Long-term debt	$ 750	$ 1,750
Less current portion of long-term debt	(750)	(1,750)
Long-term debt, net of current portion	$	$
Current portion of long-term debt	$ 750	$ 1,750
Current portion of debt discount	(4)	(18)
Current portion of long-term debt, net	$ 746	$ 1,732

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 June 30, 2014, future minimum payments under the operating lease are $1.9 million.

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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	833,808	6.73
Canceled	(63,293)	5.26
Exercised	(88,934)	1.67
Balance at June 30, 2014	2,408,572	$ 3.78

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2014	2013	2014	2013
Research and development	$ 229	$ 67	$ 794	$ 105
General and administrative	195	45	501	82
	$ 424	$ 112	$ 1,295	$ 187

As of June 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 June 30, 2014 was $0.7 million.

As of June 30, 2014, the unrecognized compensation cost related to outstanding options (excluding those with performance-based conditions) was $5.0 million and is expected to be recognized as expense over approximately 2.9 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:

	Six Months Ended June 30,	
	2014	2013
Risk-free interest rate	1.9%	1.1%
Expected volatility	94.5%	89.8%
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:

	Six Months Ended June 30,	
	2014	2013
Risk-free interest rate	2.2%	1.5%
Expected volatility	94.3%	90.0%
Remaining contractual term (in years)	6.6	8.1
Expected dividend yield	0.0%	0.0%

6. Subsequent Events

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). 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 (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 from July 30, 2014 to December 31, 2014 (each, a Term B Loan), the first of which shall be at least $5.0 million.

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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 monthly payments 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.

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 the Company to the Bank, and the remainder will 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.

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 $400,000 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 (10) trading days prior to the funding date of the first Term B Loan.

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 orphan diseases, including certain hematologic malignancies, inherited metabolic disorders, or IMDs, including lysosomal storage disorders, or LSDs, and muscular dystrophies. Our novel approaches utilize established pharmacologic modalities, including small molecules and therapeutic proteins, and target well-characterized biological mechanisms to enhance the therapeutic potential of adult stem cells. Based on

our deep understanding of key biological mechanisms that guide the fate of adult stem cells, we have built two stem cell modulation platforms that optimize the activity of adult stem cells using both *ex vivo* and *in vivo* techniques: our hematopoietic stem cell, or HSC, modulation platform and our muscle satellite stem cell, or Satellite Cell, modulation platform. We believe that the product candidates generated by our stem cell modulation platforms have significant potential as life-changing or curative therapeutics across a broad range of orphan indications.

Since our inception in 2007, we have devoted substantially all of our resources to the development of our stem cell modulation platforms, the clinical and preclinical advancement of our product candidates, the creation, licensing and protection of related intellectual property and the provision of general and administrative support for these operations. To date, we have funded our operations primarily through the public sale of common stock, the private placement of preferred stock and convertible notes and through commercial bank debt.

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:

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- 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, 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 June 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 $0.4 million for the three and six months ended June 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.

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Research and Development Expenses

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

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

From inception through June 30, 2014, we have incurred $65.5 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

- conducting preclinical studies of our Wnt7a protein analog product candidates to evaluate their potential to promote muscle regeneration.

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

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2014	2013	2014	2013
HSC modulation platform	$ 2,151	$ 1,356	$ 4,301	$ 2,514
Other preclinical programs and technologies	1,103	964	2,689	1,751
Total direct research and development expenses	3,254	2,320	6,990	4,265
Unallocated expenses	714	747	1,500	1,333
Total research and development expenses	$ 3,968	$ 3,067	$ 8,490	$ 5,598

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.

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

Other Income (Expense), Net

Other income (expense), net consists primarily of interest income earned on cash and cash equivalents; interest expense on convertible notes and on amounts outstanding under our credit facility; 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 six months ended June 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 June 30, 2014 and 2013

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

	Three Months Ended June 30,		
	2014	2013	Increase / (Decrease)
Collaboration revenue	$	$ 208	$ (208)
Grant revenue		82	(82)
Research and development expense	3,968	3,067	901
General and administrative expense	2,072	1,492	580
Total other income (expense), net	(27)	(1,265)	(1,238)

Revenue. We did not generate any revenue for the three months ended June 30, 2014, compared to $0.3 million of revenue generated for the three months ended June 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 $4.0 million for the three months ended June 30, 2014, compared to $3.1 million for the three months ended June 30, 2013. The $0.9 million increase in research and development expenses primarily reflects the following:

- $0.4 million increase in compensation and benefits expense, including 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;

- $0.2 million increase in third-party professional consultant and service provider expenses relating to the conduct of our PUMA study; and

- $0.2 million increase in expenditures for laboratory equipment and supplies relating to the conduct of our PUMA study.

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General and administrative expenses. General and administrative expenses were $2.1 million for the three months ended June 30, 2014, compared to $1.5 million for the three months ended June 30, 2013. The $0.6 million increase in general and administrative expenses primarily reflects the following:

- $0.3 million increase in compensation and benefits expense, including stock-based compensation expense, relating to an increase in employee headcount to support the expansion of our financial and administrative operations; and

- $0.3 million increase in third-party financial and legal professional service providers and insurance expenses to support our operations as a public company.

Other income (expense), net. Other income (expense), net was $(27,000) for the three months ended June 30, 2014, compared to $(1.3) million for the three months ended June 30, 2013. The decrease in other expense was primarily due to a fair value charge on the exchangeable share liability of $1.2 million during the three months ended June 30, 2013 related to the total exchangeable shares held by the former stockholders of Verio that did not reoccur during the three months ended June 30, 2014.

Comparison of the Six Months Ended June 30, 2014 and 2013

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

	Six Months Ended June 30,		
	2014	**2013**	**Increase / (Decrease)**
Collaboration revenue	$	$ 417	$ (417)
Grant revenue		345	(345)
Research and development expense	8,490	5,598	2,892
General and administrative expense	4,487	2,789	1,698
Total other income (expense), net	(70)	(1,457)	(1,387)

Revenue. We did not generate any revenue for the six months ended June 30, 2014, compared to $0.8 million of revenue generated for the six months ended June 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 $8.5 million for the six months ended June 30, 2014, compared to $5.6 million for the six months ended June 30, 2013. The $2.9 million increase in research and development expenses primarily reflects the following:

- $1.2 million increase in compensation and benefits expense, including 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;

- $0.6 million increase in third-party professional consultant and service provider expenses relating to our preparation for, and the commencement and conduct of, our PUMA study;

- $0.4 million increase in expenditures for laboratory equipment and supplies relating to our preparation for, and commencement and conduct of, our PUMA study and the conduct of our preclinical programs; and

- $0.4 million increase from the non-cash charge related to the achievement of a preclinical milestone under our agreement with the former Verio stockholders.

General and administrative expenses. General and administrative expenses were $4.5 million for the six months ended June 30, 2014, compared to $2.8 million for the six months ended June 30, 2013. The $1.7 million increase in general and administrative expenses primarily reflects the following:

- $0.8 million increase in compensation and benefits expense, including stock-based compensation expense, relating to an increase in employee headcount to support the expansion of our financial and administrative operations; and

- $0.7 million increase in third-party financial and legal professional service provider and insurance expenses to support our operations as a public company.

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Other income (expense), net. Other income (expense), net was $(0.1) million for the six months ended June 30, 2014, compared to $(1.5) million for the six months ended June 30, 2013. The decrease in other expense was primarily due to a fair value charge on the exchangeable share liability of $1.3 million during the six months ended June 30, 2013 related to the total exchangeable shares held by the former stockholders of Verio that did not reoccur during the six months ended June 30, 2014.

Liquidity and Capital Resources

We have incurred losses and negative cash flows from operations since inception. As of June 30, 2014, we had an accumulated deficit of $99.6 million and anticipate that we will continue to incur net losses for the foreseeable future.

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

	Six Months Ended June 30,	
	2014	**2013**
Net cash used in operating activities	$ (10,744)	$ (7,323)
Net cash used in investing activities	(424)	(71)
Net cash (used in) provided by financing activities	(856)	1,706
Net change in cash and cash equivalents	$ (12,024)	$ (5,688)

Operating Activities

Cash used in operating activities increased $3.4 million from $7.3 million for the six months ended June 30, 2013 to $10.7 million for the six months ended June 30, 2014. The primary driver of operating cash requirements was our net loss in each period. During the six months ended June 30, 2014, we used cash from operating activities of $10.7 million, while our net loss was $13.0 million. The difference was a result of $0.4 million net change in our operating assets and liabilities and $1.9 million of non-cash charges and deferrals, including $1.3 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 $0.3 million of depreciation expense.

Investing Activities

During the six months ended June 30, 2014 and 2013, investing activities used cash of $0.4 million and $0.1 million, respectively, for the purchase of property and equipment.

Financing Activities

Financing activities used cash of $0.9 million for the six months ended June 30, 2014 related primarily to the pay down of principal on our outstanding long-term debt. Financing activities provided cash of $1.7 million for the six months ended June 30, 2013 related to $3.0 million from the issuance of convertible promissory notes, partially offset by $1.0 million from the pay down of principal on our outstanding long-term debt.

From our inception through June 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 June 30, 2014, we had cash and cash equivalents of $42.0 million.

Our IPO on October 4, 2013 resulted in net proceeds of $40.5 million. We also repaid a total of $1.7 million in cash of outstanding principal and accrued interest on convertible notes in connection with the IPO.

In 2009, we entered into a $3.0 million loan and security agreement (the Loan Agreement) collateralized by substantially all of our assets, excluding certain intellectual property, with Silicon Valley Bank (the Bank). We drew the full $3.0 million available under the Loan Agreement in 2009. In August 2011, the Loan Agreement was amended to: (i) increase the available credit under the Loan Agreement to $4.0 million, (ii) add an additional payment upon maturity equal to 5% of the maximum loan amount and (iii) repay the remaining $0.6 million of outstanding principal related to the original $3.0 million loan. We accessed the full $4.0 million of available credit under the amended Loan Agreement by taking a term advance of $2.0 million in August 2011 and a term advance of $2.0 million in December 2011, each of which are scheduled to be fully paid by August 2014 and December 2014, respectively. The term advances require interest-only payments during the first 12 months from access and equal monthly principal and interest payments during the final 24 months from access. The interest rate on the term advances is fixed at 7.0% per annum for their entire 36-month term of the debt. As of June 30, 2014, the aggregate outstanding principal was $0.8 million.

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On July 30, 2014, we entered into an Amended and Restated Loan and Security Agreement (the Restated LSA) with the Bank. The Restated LSA amends and restates the Loan Agreement as amended. 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 (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 from July 30, 2014 to 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 monthly payments 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.

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, and the remainder will 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 $400,000 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 June 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.

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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

We did not enter into any material contractual obligations during the six months ended June 30, 2014. 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.

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. See Note 6 to the Condensed Consolidated Financial Statements for further details.

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

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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 June 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 preclinical or clinical development, obtain regulatory approval, or successfully commercialize our product candidates from our hematopoietic stem cell, or HSC, and Satellite Cell modulation platforms, our business would be significantly harmed.

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

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

In 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 improved nutrient-rich media, or 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 permission 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 permission 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 are acceptable. Although we have initiated enrollment in the PUMA study and have received the FDA s permission 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, or any other planned or 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 planned or 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 planned or 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.

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 likely decline.

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

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

- delays in our ability to enroll patients in 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 studies may not produce the benefits that we currently anticipate or may result in unanticipated adverse effects;

- delays in designing appropriate clinical trial protocols and reaching agreement on trial design with investigators and regulatory authorities;

- delays or failure in reaching agreement on acceptable clinical trial contracts or protocols with prospective sites;

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

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- 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 on-site cell processing facilities for use in our clinical trials;

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

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

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

- regulators or 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 not completing 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 across the multiple cell processing facilities at the clinical sites participating in our trials, and any delays in, or inability to, establish manufacturing protocols acceptable to the FDA will result in further delays to our clinical development plans. Any such events would increase our costs and could delay or prevent our ability to commercialize our product candidates, which would adversely impact our business, financial condition and results of operations.

Moreover, our development costs will increase because we will be required to complete additional or larger clinical trials for product candidates from our HSC and Satellite Cell modulation platforms prior to FDA or other regulatory approval. We may not have adequate capital or other resources to 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.

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

We will be required to identify and enroll a sufficient number of patients with the disease under investigation for each of our ongoing and planned clinical trials of our product candidates. Each indication for which we plan to evaluate our current product candidates represents a rare disease or condition with limited patient populations from which to draw participants in clinical trials. Due to our focus on the development of product candidates for the treatment of orphan hematologic malignancies, rare genetic diseases and muscular dystrophies, we may not be able to identify and enroll a sufficient number of patients, or those with required or desired characteristics and criteria, in a timely manner. In addition, there are currently only a limited number of specialized transplant centers that perform HSCTs, and among physicians who perform HSCTs, some may not choose to perform these procedures under conditions that fall within our protocols, which would have an adverse effect on our development of ProHema. Our ability to enroll patients in our clinical trials, including in our PUMA, PROMPT and PROVIDE studies of ProHema, is affected by factors including:

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

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

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 these trials 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 is not complete 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, and is not predictive of efficacy, for ProHema in the PUMA study. In addition, although the results of our 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 in patients who have already been reviewed by the iDMC, but remain subject to ongoing safety surveillance, and in 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 required to be 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 FDA for entry into Phase 2 clinical trials, may make it less effective than the product manufactured during our Phase 1b ProHema-01 study or otherwise adversely affect 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.

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

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

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 our adult trials, and finding sufficient, capable centers that would be interested in participating in our pediatric trials may take additional time. There will be fewer eligible patients with hematologic malignancies and rare genetic disorders for our planned clinical trials in pediatric patients because the total number of pediatric patients who undergo allogeneic hematopoietic stem cell transplantation for the treatment of such diseases and disorders is lower than it is in adults. This may increase the time to commencement of our PROMPT and PROVIDE studies and could also further delay or limit our ability to enroll patients in these planned clinical trials.

Further, to evaluate ProHema in pediatric patients, we have modified the current formulation of ProHema to one that is suitable 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.

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

Our Wnt7a analogs are still in preclinical development. To our knowledge, there are no Wnt proteins currently undergoing clinical development, primarily due to certain molecular characteristics that hinder their effective development as biologic therapeutics. Although we believe we are the first company to produce an analog of a Wnt protein that is amenable to manufacture, formulation and administration for *in vivo* therapeutic use, we may encounter difficulties in manufacturing, formulating or administering our Wnt7a analogs, or we may otherwise observe undesirable safety or efficacy profiles in these product candidates as our development activities progress. Currently, we are conducting preclinical studies of our Wnt7a protein analog product candidates to evaluate their potential to promote muscle regeneration.

We may delay or cancel our ongoing and planned preclinical and clinical development activities for our Wnt7a analogs for a variety of reasons, including:

- the results of our ongoing or future preclinical studies or clinical trials may not demonstrate safety or a meaningful therapeutic benefit or support further development of, or may require us to significantly modify our development plans for, our Wnt7a analogs;

- the occurrence of product manufacturing difficulties including the inability to manufacture Wnt7a analogs in a sufficient quantity or supply or in a cost-effective manner, or to formulate Wnt7a analogs in a suitable form for administration, for use in our planned preclinical studies or clinical trials;

- the occurrence of adverse events associated with our Wnt7a analogs in preclinical development including in, or even after successful initial, toxicology studies, or in clinical trials that are viewed to outweigh their potential benefits;

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- the FDA may require us to conduct additional preclinical studies or generate additional data before we are allowed to proceed with clinical development;

- 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 Wnt7a analogs; and

- an inability to reach a consensus with regulatory agencies on clinical trial design, or to reach agreement on acceptable terms with prospective clinical research organizations and clinical trial sites, or to obtain required institutional review board, or IRB, approval at each clinical trial site, or to recruit suitable and sufficient numbers of patients, for the conduct of clinical trials for our Wnt7a analogs.

Any delay in, or cessation of, our Wnt7a analog 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 this new technology may arise that can cause us to delay, suspend or terminate our development efforts. Regulatory approval of novel product candidates such as ours can be more expensive and take longer than for other more well-known or extensively studied pharmaceutical or biopharmaceutical product candidates due to our and regulatory agencies lack of experience with them.

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

Regulatory requirements governing cell therapy products have changed frequently and may continue to change in the future. For example, the FDA established the Office of Cellular, Tissue and Gene Therapies within its Center for Biologics Evaluation and Research, or CBER, to consolidate the review of gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review. In addition, adverse developments in clinical trials of potential stem cell therapies conducted by others may cause the FDA or other regulatory bodies to change the requirements for approval of any of our product candidates. These regulatory authorities and advisory groups and the new requirements or guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our product candidates or lead to significant post-approval limitations or restrictions. As we advance our product candidates, we will be required to consult with the FDA and other regulatory authorities, and our products will likely be reviewed by the FDA s

advisory committee. We also must comply with applicable requirements, and if we fail to do so, we may be required to delay or discontinue development of our product candidates. 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, good manufacturing practices, or GMP, requirements relating to quality control, quality assurance and corresponding maintenance of records and documents, and recordkeeping. Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the product may be marketed or to conditions of approval, or contain requirements for costly post-marketing testing and surveillance to monitor the safety or efficacy of the product. The FDA closely regulates the post-approval marketing and promotion of pharmaceutical and biological products to ensure such products are marketed only for the approved indications and in accordance with the provisions of the approved labeling.

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

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

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

- injunctions or the imposition of civil or criminal penalties.

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

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

We are evaluating the possibility of seeking breakthrough therapy or fast track designation for some of our product candidates, although we may elect not to do so. A breakthrough therapy program is for a product candidate intended to treat a serious or life-threatening condition, and preliminary clinical evidence indicates that the product candidate may demonstrate substantial improvement on a clinically significant endpoint(s) over available therapies. In contrast, a fast track program is for a product candidate that treats a serious or life-threatening condition, and nonclinical or clinical data demonstrate the potential to address an unmet medical need. Although we believe 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. In addition, the breakthrough therapy program is a relatively new program. As a result, we cannot be certain whether any of our current or future product candidates can or will qualify for breakthrough therapy designation. Our business may be harmed if we are unable to avail ourselves of these or any other expedited development and regulatory pathways.

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

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

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For any product candidate for which we have been granted orphan drug designation in a particular indication, it is possible that another company also holding orphan drug designation for the same product candidate will receive marketing approval for the same indication before we do. If that were to happen, our applications for that indication may not be approved until the competing company s period of exclusivity expires. Even if we are the first to obtain marketing authorization for an orphan drug indication, there are circumstances under which a competing product may be approved for the same indication during the seven-year period of marketing exclusivity, such as if the later product is shown to be clinically superior to the orphan product, or if the later product is deemed a different product than ours. Further, the seven-year marketing exclusivity would not prevent competitors from obtaining approval of the same product candidate as ours for indications other than those in which we have been granted orphan drug designation, or for the use of other types of products in the same indications as our orphan product.

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

If we obtain FDA approval for any of our product candidates and begin commercializing those products in the United States, our operations may be subject to various federal and state fraud and abuse laws, including, without limitation, the federal anti-kickback statute. These laws may impact, among other things, our proposed sales, marketing and education programs. In addition, we may be subject to patient privacy regulation by both the federal government and the states in which we conduct our business. The laws that may affect our ability to operate include:

- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created new federal criminal statutes that prohibit executing a scheme to defraud any healthcare benefit program and making false statements relating to healthcare matters;

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act and its implementing regulations, which impose certain requirements relating to the privacy, security and transmission of individually identifiable health information; and

- state law equivalents of each of the above federal laws, such as anti-kickback and false claims laws which may apply to items or services reimbursed by any third-party payer, including commercial insurers, and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts.

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

Risks Related to Our Reliance on Third Parties

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

We currently rely, and expect to continue to rely, on third-party suppliers for components that enable us to develop and use our NRM formulation for the manufacture of ProHema 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.

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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 only used unlicensed CBUs in the manufacture of ProHema for our clinical trials, the FDA may later prohibit the use of unlicensed CBUs for transplantation. Additionally, although CBUs from foreign cord blood banks, which are generally unlicensed, are currently available in the United States for use in transplantation and we have used CBUs from foreign cord blood banks in our clinical trials, changes in U.S. and foreign regulations may prohibit or limit the future use of foreign CBUs in the United States. Any inability to procure adequate supplies of CBUs will adversely impact our ability to develop and commercialize ProHema.

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

ProHema is currently manufactured at clinical cell processing facilities operated by or affiliated with our clinical sites and is required to be manufactured in close proximity to the treatment site on the same day as product administration. The FDA has stated that we will be required to standardize the manufacture of ProHema to maximize consistency across the multiple clinical cell processing facilities, including our oversight for facility and raw material and vendor qualification through to final product analytical testing and release. Although we are responsible for ensuring compliance with GMPs and for overseeing all aspects of product manufacture and release prior to applying for marketing approval, we do not control the activities of these third-party cell processing facilities. The clinical cell processing facilities at which ProHema is manufactured must be approved by applicable regulatory

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authorities, including the FDA, pursuant to inspections that will be conducted after a BLA, or comparable foreign regulatory marketing application is submitted. We do not control the manufacturing process for ProHema and are completely dependent on third parties for compliance with the FDA s requirements and proper execution of the protocol for the manufacture of ProHema. Because of the requirement that ProHema be manufactured in close proximity to the transplant center within a short period of time before transplant, if the applicable clinical cell processing facilities are unable to manufacture ProHema in a manner that conforms to our specifications and the FDA s strict regulatory requirements, we will not be able to secure backup manufacturing capabilities. For example, to comply with GMPs and other regulatory requirements and our manufacturing protocols, the clinical cell processing facility may be required to possess or obtain certain equipment necessary for the manufacture of ProHema including but not limited to biosafety cabinets, warming devices, cell washing devices, freezers or other materials; or to modify aspects of its operations, including its physical facility or layout, environmental systems, monitoring systems, quality systems or training procedures, for the compliant manufacture of ProHema. Clinical cell processing facilities may be unwilling or unable to comply with these requirements, which could restrict or prohibit a given clinical center from manufacturing ProHema and making it available for administration to patients within the required timeframes. Any failure by these clinical cell processing facilities to properly manufacture ProHema may adversely affect the safety and efficacy profile of ProHema or cause the FDA or other regulatory authorities to impose restrictions or prohibitions on the manufacture and use of ProHema in both the clinical and the commercial setting, which would have an adverse impact on our business.

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

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

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

We rely on third-party service providers for clinical trials 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 will be substantially dependent on third parties for the manufacture of our clinical supplies of our Wnt7a analogs.

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

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 Wnt analogs, covering compositions of matter, processes for preparing such Wnt proteins and formulations, and the modulation of satellite cells. 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 Wnt product candidates.

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

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

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

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

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

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

We are 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.

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.

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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.

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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.

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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 HSC therapeutics that we may develop are administered;

- relative convenience and ease of administration;

- the willingness of the target patient population to try new therapeutics and of physicians to prescribe these therapeutics;