Global Blood Therapeutics, Inc.
Form 10-Q
May 12, 2016
Table Of Contents

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 March 31, 2016
OR
o TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the transition period from to
Commission file number: 001-37539

Global Blood Therapeutics, Inc.
(Exact name of registrant as specified in its charter)

Delaware	27-4825712
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)

400 East Jamie Court, Suite 101, South San Francisco
South San Francisco, CA 94080
(Address of principal executive offices)
(650) 741-7700
(Registrant's telephone number, including area code)

Indicate by check mark whether the registrant: (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the Registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ý No ¨
Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes ý No ¨
Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, or a smaller reporting company. See the definitions of "large accelerated filer," "accelerated filer" and "smaller reporting company" in Rule 12b-2 of the Exchange Act. (Check one):

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

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ¨ No ý
As of May 6, 2016, there were 30,531,425 shares of the registrant's Common Stock, par value $0.001 per share, outstanding.

Table Of Contents

TABLE OF CONTENTS

Table Of Contents

PART I. – FINANCIAL INFORMATION
Item 1. Financial Statements
GLOBAL BLOOD THERAPEUTICS, INC.
Condensed Balance Sheets
(In thousands, except share and per share amounts)

	March 31, 2016 (Unaudited)	December 31, 2015
Assets		
Current assets:		
Cash and cash equivalents	$ 133,984	$ 148,502
Prepaid expenses	1,907	1,222
Other assets, current	647	1,096
Total current assets	136,538	150,820
Property and equipment, net	2,293	2,114
Restricted cash	140	140
Total assets	$ 138,971	$ 153,074
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 4,295	$ 3,361
Accrued liabilities	4,738	4,400
Accrued compensation	1,715	2,242
Other liabilities, current	747	720
Total current liabilities	11,495	10,723
Other liabilities, noncurrent	1,354	1,556
Total liabilities	12,849	12,279
Commitments and contingencies		
Stockholders' equity:		
Preferred stock, $0.001 par value, 5,000,000 shares authorized as of March 31, 2016 and December 31, 2015	—	—
Common stock, $0.001 par value, 150,000,000 shares authorized as of March 31, 2016 (unaudited) and December 31, 2015, respectively; 29,536,449 and 29,359,800 shares issued and outstanding as of March 31, 2016 (unaudited) and December 31, 2015, respectively	30	29
Additional paid-in capital	241,157	239,231
Accumulated deficit	(115,065)	(98,465)
Total stockholders' equity	126,122	140,795
Total liabilities and stockholders' equity	$ 138,971	$ 153,074

See accompanying notes to unaudited interim condensed financial statements.

Table Of Contents

GLOBAL BLOOD THERAPEUTICS, INC.
Condensed Statements of Operations and Comprehensive Loss
(Unaudited)
(In thousands, except share and per share amounts)

	Three Months Ended March 31,	
	2016	2015
Operating expenses:		
Research and development	$12,415	$6,069
General and administrative	4,302	1,298
Related party expenses	—	53
Total operating expenses	16,717	7,420
Loss from operations	(16,717)	(7,420)
Interest income	117	3
Net loss and comprehensive loss	$(16,600)	$(7,417)
Net loss attributable to common stockholders	$(16,600)	$(8,657)
Net loss per share attributable to common stockholders, basic and diluted	$(0.56)	$(4.22)
Weighted-average number of shares used in computing net loss per share attributable to common stockholders, basic and diluted	29,441,404	2,052,874

See accompanying notes to unaudited interim condensed financial statements.

Table Of Contents

GLOBAL BLOOD THERAPEUTICS, INC.
Condensed Statements of Cash Flows
(Unaudited)
(In thousands)

	Three Months Ended March 31,	
	2016	2015
CASH FLOWS FROM OPERATING ACTIVITIES:		
Net loss	$(16,600)	$(7,417)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	242	183
Loss on disposal of fixed assets	—	18
Stock-based compensation	1,267	322
Changes in operating assets and liabilities:		
Prepaid expenses	(685)	378
Other assets, current	(3)	(362)
Accounts payable	867	334
Payable due to related party	—	39
Accrued liabilities	814	754
Accrued compensation	(527)	(305)
Other liabilities	(9)	(5)
Net cash used in operating activities	(14,634)	(6,061)
CASH FLOWS FROM INVESTING ACTIVITIES:		
Purchase of property and equipment	(378)	(169)
Net cash used in investing activities	(378)	(169)
CASH FLOWS FROM FINANCING ACTIVITIES:		
Payments of deferred offering costs	—	(67)
Proceeds from issuance of restricted stock awards	—	45
Repurchase of unvested restricted stock awards	—	(17)
Proceeds from issuance of common stock in settlement of employee stock purchase plan and exercise of stock options	494	—
Net cash provided by (used in) financing activities	494	(39)
Net decrease in cash and cash equivalents	(14,518)	(6,269)
Cash and cash equivalents at beginning of period	148,502	52,069
Cash and cash equivalents at end of period	$133,984	$45,800
SUPPLEMENTAL DISCLOSURES OF NON-CASH INVESTING AND FINANCING INFORMATION:		
Accretion of Series A and Series B redeemable convertible preferred stock	$—	$1,240
Accrued purchase of property and equipment	$43	$356
Accrued offering costs	$—	$620

See accompanying notes to unaudited interim condensed financial statements.

Table Of Contents

GLOBAL BLOOD THERAPEUTICS, INC.
Notes to Unaudited Interim Condensed Financial Statements

1. Organization and Basis of Presentation

Global Blood Therapeutics Inc. (the "Company", "we", "us", and "our") was incorporated in Delaware in February 2011 and commenced operations in May 2012. We are a clinical-stage biopharmaceutical company dedicated to discovering, developing and commercializing novel therapeutics to treat grievous blood-based disorders with significant unmet needs. Our primary activities have been establishing our facilities, recruiting personnel, conducting development of our product candidates, including clinical trials, and raising capital. Our principal operations are based in South San Francisco, California, and we operate in one segment.

Need for Additional Capital

In the course of our development activities, we have sustained operating losses and we expect such losses to continue over the next several years. Our ultimate success depends on the outcome of our research and development activities. Since inception through March 31, 2016, we have incurred cumulative net losses of $115.1 million. We expect to incur additional losses in the future to conduct product research and development and we recognize the need to raise additional capital to fully implement our business plan. We intend to raise such capital through the issuance of additional equity, and potentially through borrowings, and strategic alliances with partner companies. However, if such financing is not available at adequate levels, we will need to reevaluate our operating plans. We believe that our existing cash and cash equivalents will be sufficient to fund our cash requirements through mid-2017.

2. Summary of Significant Accounting Policies

Basis of Preparation

The accompanying unaudited interim condensed financial statements have been prepared in accordance with accounting principles generally accepted in the United States of America ("U.S. GAAP") and applicable rules and regulations of the Securities and Exchange Commission ("SEC") regarding interim financial reporting. As permitted under those rules, certain footnotes or other financial information that are normally required by U.S. GAAP have been condensed or omitted, and accordingly the balance sheet as of December 31, 2015 has been derived from audited financial statements at that date but does not include all of the information required by U.S. GAAP for complete financial statements. These unaudited interim condensed financial statements have been prepared on the same basis as our annual financial statements and, in the opinion of management, reflect all adjustments (consisting only of normal recurring adjustments) that are necessary for a fair presentation of our financial information. The results of operations for the three months ended March 31, 2016 are not necessarily indicative of the results to be expected for the year ending December 31, 2016 or for any other interim period or for any other future year.

The accompanying interim unaudited condensed financial statements and related financial information should be read in conjunction with the audited financial statements and the related notes thereto for the year ended December 31, 2015 included in our Annual Report on Form 10-K, filed with the SEC on March 29, 2016.

Use of Estimates

The preparation of the accompanying unaudited interim condensed financial statements in accordance with U.S. GAAP requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the condensed financial statements, and the reported amounts of costs and expenses during the reporting period. We base our estimates and assumptions on historical experience when available and on various factors that we believe to be reasonable under the circumstances. We evaluate our estimates and assumptions on an ongoing basis. Our actual results could differ from these estimates under different assumptions or conditions.

Cash and Cash Equivalents

We consider all highly liquid investments with original maturities of three months or less at the time of purchase to be cash equivalents. Cash equivalents, which consist primarily of amounts invested in money market accounts, are stated at fair value.

Table Of Contents
GLOBAL BLOOD THERAPEUTICS, INC.
Notes to Unaudited Interim Condensed Financial Statements

Accruals of Research and Development Costs
We record accruals for estimated costs of research, preclinical, nonclinical and clinical studies and manufacturing development. These costs are a significant component of our research and development expenses. A substantial portion of our ongoing research and development activities are conducted by third-party service providers, including contract research organizations. We accrue the costs incurred under our agreements with these third parties based on actual work completed in accordance with agreements established with these third parties. We determine the actual costs through discussions with internal personnel and external service providers as to the progress or stage of completion of the services and the agreed-upon fee to be paid for such services. We make significant judgments and estimates in determining the accrual balance in each reporting period. As actual costs become known, we adjust our accruals. We have not experienced any material deviations between accrued clinical trial expenses and actual clinical trial expenses. However, actual services performed, number of subjects enrolled, and the rate of subject enrollment may vary from our estimates, resulting in adjustments to clinical trial expense in future periods. Changes in these estimates that result in material changes to our accruals could materially affect our results of operations.
Concentration of Credit Risk
Financial instruments that potentially subject us to a concentration of credit risk consist of cash and cash equivalents. Our cash and cash equivalents are held primarily in one large financial institution in the United States. We believe that this financial institution is financially sound, and accordingly, minimal credit risk exists with respect to this financial institution.
Fair Value Measurement
The carrying amounts of certain financial instruments, including cash and cash equivalents, restricted cash, accounts payable and accrued liabilities approximate fair value due to their relatively short maturities.
Research and Development Costs
Research and development costs are expensed as incurred and consist of salaries and benefits, stock-based compensation expense, lab supplies and facility costs, as well as fees paid to other nonemployees and entities that conduct certain research and development activities on our behalf. Amounts incurred in connection with license agreements are also included in research and development expense. Nonrefundable advance payments for goods or services to be received in the future for use in research and development activities are capitalized and then expensed as the related goods are delivered or the services are performed.
Stock-based Compensation
We measure and recognize stock-based compensation expense, including employee and non-employee equity awards, based on fair value at the grant date. We use the Black-Scholes option-pricing model to calculate fair value. Stock-based compensation expense recognized in the statements of operations is based on options ultimately expected to vest, taking into consideration estimated forfeitures. Stock-based compensation expense is revised in subsequent periods, if necessary, if actual forfeitures differ from these estimates. When estimating forfeitures, we consider historic voluntary termination behaviors as well as trends of actual option forfeitures. For options granted to non-employees, we revalue the unearned portion of the stock-based compensation and the resulting change in fair value is recognized in the statements of operations over the period the related services are rendered.
Net Loss per Share Attributable to Common Stockholders
Net loss per share attributable to common stockholders is calculated by dividing the net loss attributable to common stockholders by the weighted average number of shares of common stock outstanding during the period, without consideration for common stock equivalents. The net loss attributable to common stockholders is calculated by adjusting our net loss for the accretion and dividends on redeemable convertible preferred stock. Diluted net loss per share attributable to common stockholders is the same as basic net loss per share attributable to common stockholders, since the effects of potentially dilutive securities are antidilutive given our net loss.

Table Of Contents
GLOBAL BLOOD THERAPEUTICS, INC.
Notes to Unaudited Interim Condensed Financial Statements

Recent Accounting Pronouncements
From time to time, new accounting pronouncements are issued by the Financial Accounting Standards Board ("FASB") or other standards setting bodies that are adopted by us as of the specified effective date. Unless otherwise discussed, we believe that the impact of recently issued standards that are not yet effective will not have a material impact on our financial statements upon adoption.
In February 2016, the FASB issued Accounting Standards Update, or ASU No. 2016-02, Leases. The new standard requires lessees to apply a dual approach, classifying leases as either finance or operating leases based on the principle of whether or not the lease is effectively a financed purchase by the lessee. This classification will determine whether lease expense is recognized based on an effective interest method or on a straight-line basis over the term of the lease. A lessee is also required to record a right-of-use asset and a lease liability for all leases with a term of greater than 12 months regardless of their classification. Leases with a term of 12 months or less will be accounted for similar to existing guidance for operating leases. The standard is effective for interim and annual periods beginning after December 15, 2018, with early adoption permitted. We are currently in the process of evaluating the impact the adoption of this new standard will have on our financial position or results of operations.
In March 2016, the FASB issued ASU No. 2016-09. The new standard simplifies the accounting for share-based payment transactions, including the income tax consequences, classification of awards as either equity or liabilities, and classification on the statement of cash flows. The standard is effective for annual periods beginning after December 15, 2016, and interim periods within those annual periods. We are currently in the process of evaluating the impact the adoption of this new standard will have on our financial position or results of operations and have not elected to early adopt the amendments.
3. Fair Value Measurements
Fair value accounting is applied for all financial assets and liabilities that are recognized or disclosed at fair value in the financial statements on a recurring basis (at least annually). Financial instruments include cash and cash equivalents, restricted cash, accounts payable and accrued liabilities that approximate fair value due to their relatively short maturities.
Assets and liabilities recorded at fair value on a recurring basis in the balance sheets are categorized based upon the level of judgment associated with the inputs used to measure their fair values. Fair value is defined as the exchange price that would be received for an asset or an exit price that would be paid to transfer a liability in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. The authoritative guidance on fair value measurements establishes a three-tier fair value hierarchy for disclosure of fair value measurements as follows:
Level 1 – Inputs are unadjusted, quoted prices in active markets for identical assets or liabilities at the measurement date;
Level 2 – Inputs are observable, unadjusted quoted prices in active markets for similar assets or liabilities, unadjusted quoted prices for identical or similar assets or liabilities in markets that are not active, or other inputs that are observable or can be corroborated by observable market data for substantially the full term of the related assets or liabilities; and
Level 3 – Unobservable inputs that are significant to the measurement of the fair value of the assets or liabilities that are supported by little or no market data.
Financial assets subject to fair value measurements on a recurring basis and the level of inputs used in such measurements are as follows (in thousands):

	March 31, 2016			
	Level 1	Level 2	Level 3	Total
Financial Assets:				
Money market funds	$134,124	$ —	$ —	$134,124
Total financial assets	$134,124	$ —	$ —	$134,124

8

Table Of Contents
GLOBAL BLOOD THERAPEUTICS, INC.
Notes to Unaudited Interim Condensed Financial Statements

	December 31, 2015			
	Level 1	Level 2	Level 3	Total
Financial Assets:				
Money market funds	$148,642	$ —	$ —	$148,642
Total financial assets	$148,642	$ —	$ —	$148,642

Our financial instruments consist of Level 1 assets. Where quoted prices for identical assets are available in an active market, securities are classified as Level 1. Level 1 assets consist of highly liquid money market funds, which as of March 31, 2016 and December 31, 2015 includes $140,000 of funds that are collateral for our facility lease that are included within restricted cash. There were no unrealized gains and losses in our investments in these money market funds.

4. Balance Sheet Components

Property and Equipment

Property and equipment consists of the following:

(in thousands)	March 31, 2016	December 31, 2015
Laboratory equipment	$3,275	$ 3,151
Computer equipment	930	596
Leasehold improvements	348	340
Construction-in-progress	84	129
Total property and equipment	4,637	4,216
Less: accumulated depreciation and amortization	(2,344)	(2,102)
Property and equipment, net	$2,293	$ 2,114

Accrued liabilities

Accrued liabilities consist of the following:

(in thousands)	March 31, 2016	December 31, 2015
Accrued clinical and manufacturing expenses	$4,089	$ 4,025
Accrued professional and consulting services	646	287
Other	3	88
Total accrued liabilities	$4,738	$ 4,400

Other liabilities, noncurrent

Other noncurrent liabilities consist of the following:

(in thousands)	March 31, 2016	December 31, 2015
Restricted shares subject to repurchase, noncurrent	$1,278	$ 1,470
Deferred rent, noncurrent	76	86
Total other liabilities, noncurrent	$1,354	$ 1,556

Table Of Contents
GLOBAL BLOOD THERAPEUTICS, INC.
Notes to Unaudited Interim Condensed Financial Statements

5. Stockholders' Equity
Common Stock
We have reserved the following shares of common stock for issuance as follows:

	March 31, 2016
Restricted shares subject to future vesting	992,176
Options issued and outstanding	2,487,374
Options available for future grants	2,379,284
Employee stock purchase plan	112,100
Total common stock reserved for issuance	5,970,934

Restricted Stock
In May 2012, we issued 1,345,709 shares of restricted common stock to founders at $0.0035 per share, all of which are fully vested as of March 31, 2016. Under the related stock purchase agreements, we have the right to repurchase the common stock which right lapses according to individual vesting schedules. In addition, we have issued restricted stock awards to employees under our 2012 Stock Option and Grant Plan (the "2012 Plan"). Under the related stock purchase agreements, we have the right to repurchase the common stock at the lower of fair market value and the stockholders' original purchase price which right lapses according to individual vesting schedules.
In order to vest, the holders are required to provide continued service to us. Upon vesting, the appropriate amounts are transferred from liabilities to additional paid in capital. If the employment or other service relationship of the holder of any unvested restricted common stock is terminated for any reason, we have the right to repurchase the unvested shares at the lower of fair market value or the stockholder's original purchase price. As such, the shares subject to future vesting are not deemed outstanding for accounting purposes until the shares vest.
Restricted shares subject to repurchase and related liability were as follows:

(in thousands except share data)	March 31, 2016	December 31, 2015
Restricted shares subject to repurchase:		
Shares issued to founders	—	6,250
Shares issued pursuant to the 2012 Stock Option and Grant Plan	992,176	1,091,038
Total restricted shares subject to repurchase	992,176	1,097,288
Liability pertaining to restricted shares subject to repurchase		
Other liabilities, current	703	677
Other liabilities, noncurrent	1,278	1,470
Total liabilities pertaining to shares subject to repurchase	$ 1,981	$ 2,147

6. Stock-based Awards
Equity Incentive Plans
In July 2015, we adopted the 2015 Stock Option and Incentive Plan (the "2015 Plan"). Under the 2015 Plan, 1,430,000 shares of our common stock were initially reserved for the issuance of stock options, restricted stock, and other equity-based awards to employees, non-employee directors, and consultants under terms and provisions established by the Board of Directors and approved by our stockholders. The 2015 Plan also provides for automatic annual increases in the number of shares reserved for future issuance. As of March 31, 2016, there were 2,379,284 shares available for us to grant under the 2015 Plan.

Table Of Contents
GLOBAL BLOOD THERAPEUTICS, INC.
Notes to Unaudited Interim Condensed Financial Statements

In 2012, we adopted the 2012 Plan under which the Board of Directors was authorized to grant incentive stock options to employees, including officers and members of the Board of Directors who are also employees of ours, and non-statutory stock options (options that do not qualify as incentive options) and/or our restricted stock and other equity-based awards to employees, officers, directors, or consultants of ours. Upon adoption of the 2015 Plan, no new awards or grants are permitted under the 2012 Plan.

Employee Stock Purchase Plan

In July 2015, we adopted the 2015 Employee Stock Purchase Plan (the "2015 ESPP"). Under the 2015 ESPP, 50,000 shares of our common stock have been initially reserved for employee purchases of our common stock under terms and provisions established by the Board of Directors and approved by our stockholders. The 2015 ESPP also provides for automatic annual increases in the number of shares reserved for future issuance.

Stock Option Awards

The following summarizes option activity under the 2015 Plan and the 2012 Plan:

	Number of Options	Weighted-Average Exercise Price	Weighted-Average remaining contractual term (years)	Aggregate Intrinsic Value (in thousands)
Balance Outstanding, December 31, 2015	2,058,787	$ 8.71	9.0	
Options granted	606,425	$ 15.09		
Options exercised	(42,266)	$ 0.53		
Options canceled	(135,572)	$ 22.10		
Balance Outstanding, March 31, 2016	2,487,374	$ 9.67	9.0	$ 21,586
Exercisable, March 31, 2016	482,249	$ 3.96	8.2	$ 6,131
Vested and expected to vest, March 31, 2016	2,334,962	$ 9.50	9.0	$ 20,555

Stock Options Granted to Employees with Service-Based Vesting Conditions Valuation Assumptions

The fair values of stock options granted to employees were calculated using the following assumptions:

	Three Months Ended March 31,	
	2016	2015
Expected term (in years)	5.8-6.1	6.0-6.1
Volatility	79.4%-81.0%	75.9%-77.0%
Risk-free interest rate	1.3%-1.9%	1.5%-1.7%
Dividend yield	—	—

Performance-Contingent Awards

On April 9, 2015, our Board of Directors granted a total of 326,424 performance-contingent awards to members of our senior management team. Of the total performance-contingent awards granted, 227,139 were performance-contingent options and 99,285 were performance-contingent shares of restricted common stock. The exercise price of each performance-contingent option and the purchase price for the performance-contingent restricted shares is $3.40 per share, which the Board of Directors determined was the fair market value on the grant date.

These awards have dual triggers of vesting based upon the successful achievement of four corporate operating milestones within specified timelines, as well as a requirement for continued employment. When a performance goal is deemed to be probable of achievement, time-based vesting and recognition of stock-based compensation expense commences. In the event any of the corporate operating milestones are not achieved by the specified timelines, such vesting tranche will terminate and no longer be exercisable with respect to that portion of the shares. During the first quarter ended March 31, 2016, the Compensation Committee of our Board of Directors determined that the

achievement of one of the corporate operating milestones was met; accordingly, shares associated with this milestone vested and, as a result, $100,000 of compensation cost was recognized for the performance-contingent awards.

Table Of Contents
GLOBAL BLOOD THERAPEUTICS, INC.
Notes to Unaudited Interim Condensed Financial Statements

Market-Condition Award
On April 9, 2015, our Board of Directors granted a market-condition award to our Chief Executive Officer of 99,285 shares of restricted common stock. The market-condition award does not vest until our market capitalization (determined based on the number of shares of common stock outstanding multiplied by the closing market price for our common stock as reported on NASDAQ) exceeds at least $2.0 billion for 20 consecutive trading days on or before the date twenty-four (24) months after the closing of our initial public offering, or IPO.
The fair value of the market-condition award of $0.70 was determined on the grant date utilizing a lattice model that was prepared by a third party valuation firm with an expected term of 2.4 years. In August 2015, we began to recognize compensation costs for this award concurrent with the closing of our IPO.
Stock-Based Compensation Expense
Total stock-based compensation recognized by function was as follows (in thousands):

	Three Months Ended March 31,	
	2016	2015
Research and development	$545	$265
General and administrative	722	57
Total stock-based compensation expense	$1,267	$322

Total stock-based compensation recognized for employees and non-employees was as follows (in thousands):

	Three Months Ended March 31,	
	2016	2015
Employee options and restricted stock awards	$1,200	$293
Non-employee options	67	29
Total stock-based compensation expense	$1,267	$322

Unrecognized Stock-Based Compensation Expense and Weighted-Average Remaining Amortization Period
As of March 31, 2016 the unrecognized stock-based compensation cost, net of expected forfeitures, and the estimated weighted-average amortization period, using the straight-line attribution method, was as follows (in thousands, except amortization period):

	Unrecognized Compensation Cost	Weighted-average remaining amortization period (years)
Options	$ 14,022	2.9
Restricted stock awards	828	2.3
ESPP	215	0.2
Total unrecognized stock-based compensation expense	$ 15,065	

Table Of Contents
GLOBAL BLOOD THERAPEUTICS, INC.
Notes to Unaudited Interim Condensed Financial Statements

7. Net Loss per Share Attributable to Common Stockholders

The following table sets forth the computation of the basic and diluted net loss per share attributable to common stockholders during the three months ended March 31, 2016 and 2015, respectively (in thousands, except share and per share data):

	Three Months Ended March 31,	
	2016	2015
Numerator:		
Net loss	$(16,600)	$(7,417)
Accretion and dividends on redeemable convertible preferred stock	—	(1,240)
Net loss attributable to common stockholders	$(16,600)	$(8,657)
Denominator:		
Weighted average common shares outstanding	29,441,404	2,052,874
Net loss per share attributable to common stockholders, basic and diluted	$(0.56)	$(4.22)

Since we were in a loss position for all periods presented, diluted net loss per share attributable to common stockholders is the same as basic net loss per share for all periods as the inclusion of all potential common shares outstanding would have been anti-dilutive. Potentially dilutive securities that were not included in the diluted per share calculations because they would be anti-dilutive were as follows:

	Three Months Ended March 31,	
	2016	2015
Redeemable convertible preferred stock as if converted	—	19,746,614
Options to purchase common stock	2,487,374	1,190,051
Restricted stock subject to future vesting	992,176	985,338
2015 ESPP	58,921	—
Total	3,538,471	21,922,003

8. Related Party Transactions

Our largest investors include investment funds controlled by Third Rock Ventures, LLC (“TRV”) and two members of our Board of Directors are also partners in TRV. Management and advisory fee expense incurred with TRV was zero for the three months ended March 31, 2016 and $53,000 for the three months ended March 31, 2015.

Table Of Contents

Item 2. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion and analysis of our financial condition and results of operations in conjunction with our unaudited condensed financial statements and related notes included in Part I, Item 1 of this Quarterly Report on Form 10-Q and with our audited financial statements and related notes thereto for the year ended December 31, 2015, included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission on March 29, 2016, or our Annual Report.

This discussion and other parts of this Quarterly Report on Form 10-Q contain forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended and Section 21E of the Securities Exchange Act of 1934, as amended, that involve risks and uncertainties, such as statements of our plans, objectives, expectations and intentions. 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. Our actual results could differ materially from those discussed in these forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, those discussed in the section of this Quarterly Report on Form 10-Q titled "Risk Factors." We caution readers not to place undue reliance on any forward-looking statements made by us, which speak only as of the date they are made. Except as may be required by law, we assume no obligation to update these forward-looking statements or the reasons that results could differ from these forward-looking statements.

Overview

We are a clinical-stage biopharmaceutical company dedicated to discovering, developing and commercializing novel therapeutics to treat grievous blood-based disorders with significant unmet need. We are developing our initial product candidate, GBT440, as an oral, once-daily therapy for sickle cell disease, or SCD, and are currently evaluating GBT440 in SCD subjects in an ongoing Phase 1/2 clinical trial. SCD is a genetic disease marked by red blood cell, or RBC, destruction and occluded blood flow and hypoxia, leading to anemia, stroke, multi-organ failure, severe pain crises, and shortened patient life span. GBT440 inhibits abnormal hemoglobin polymerization, the underlying mechanism of RBC sickling. In our clinical trials of GBT440 in SCD subjects, we observed reduced markers of red blood cell destruction, improvements in anemia, improvements in markers of tissue oxygenation, reduced numbers of sickled RBCs, and reduced markers of inflammation. In addition to GBT440 for the treatment of SCD, we intend to evaluate GBT440 for the treatment of hypoxemic pulmonary disorders and intend initially to conduct a Phase 2a proof of concept study of idiopathic pulmonary fibrosis subjects. We are also engaged in other research and development activities targeted towards hereditary angioedema, or HAE. We own and have exclusively licensed rights to our portfolio of product candidates in the United States, Europe and other major markets. We own or co-own one issued U.S. patent that covers the composition of matter for GBT440, which is due to expire in 2032 (absent any applicable patent term extensions), and we own or co-own additional pending patent applications in the United States and selected foreign countries.

Since our inception in 2011, we have devoted substantially all of our resources to identifying and developing our product candidates, including conducting clinical trials and preclinical studies and providing general and administrative support for these operations.

Prior to our initial public offering, or IPO, we had funded our operations primarily from the issuance and sale of redeemable convertible preferred stock. In August 2015, we completed our IPO pursuant to which we issued 6,900,000 shares of our common stock at a price of $20.00 per share. We received $126.2 million from the IPO, net of underwriting discounts and commissions, and offering expenses incurred by us.

We have never been profitable and have incurred net losses in each year since inception. Our net losses were $16.6 million and $7.4 million for the three months ended March 31, 2016 and 2015, respectively. As of March 31, 2016 we had an accumulated deficit of $115.1 million. To date, we have not generated any revenue. We do not expect to receive any revenue from any product candidates that we develop until we obtain regulatory approval and commercialize our products or enter into collaborative agreements with third parties. Substantially all of our net losses have resulted from costs incurred in connection with our research and development programs and from general and administrative costs associated with our operations. As of March 31, 2016, we had $134.0 million of cash and cash equivalents.

14

Table Of Contents

Critical Accounting Polices and Estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our financial statements, which have been prepared in accordance with United States generally accepted accounting principles, or U.S. GAAP. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported expenses incurred during the reporting periods. Our estimates are based on our historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

There have been no material changes to our critical accounting policies from those described in "Management's Discussion and Analysis of Financial Condition and Results of Operations" included in our Annual Report.

Results of Operations

Comparison of the Three Months Ended March 31, 2016 and 2015

	Three Months Ended March 31,		$ Change	% Change
	2016	2015		
	(in thousands, except percentages)			
Operating expenses:				
Research and development	$12,415	$6,069	$6,346	105 %
General and administrative	4,302	1,298	3,004	231 %
Related party expenses	—	53	(53)	(100)%
Total operating expenses	16,717	7,420	9,297	125 %
Loss from operations	(16,717)	(7,420)	(9,297)	125 %
Interest income	117	3	114	*
Net loss	$(16,600)	$(7,417)	$(9,183)	124 %

* Change is not meaningful

Research and development

Research and development expenses consist primarily of costs incurred for the development of our product candidates, which include:

- employee-related expenses, which include salaries, benefits and stock-based compensation;
- expenses incurred under agreements with consultants, third-party contract organizations, and investigative clinical trial sites that conduct research and development activities on our behalf;
- laboratory and vendor expenses related to the execution of preclinical studies and clinical trials;
- the costs related to production of clinical supplies, including fees paid to contract manufacturers;
- licensing of intellectual property rights; and
- facilities and other allocated expenses, which include expenses for rent and maintenance of facilities, depreciation and amortization expense and other supplies.

We expense all research and development costs in the periods in which they are incurred. Costs for certain development activities are recognized based on an evaluation of the progress to completion of specific tasks using information and data provided to us by our vendors and clinical sites. Nonrefundable advance payments for goods or services to be received in future periods for use in research and development activities are deferred and capitalized. The capitalized amounts are then expensed as the related goods are delivered and the services are performed.

The largest component of our total operating expenses has historically been our investment in research and development activities, including the clinical development of GBT440. We allocate research and development salaries, benefits, stock-based compensation and indirect costs to GBT440 and other product candidates that we may pursue on a program-specific basis, and we include these costs in the program-specific expenses.

15

Table Of Contents

We expect our research and development expenses will increase in future periods as we continue to invest in research and development activities related to developing our product candidates, and as programs advance into later stages of development and we begin to conduct larger clinical trials. The process of conducting the necessary clinical research to obtain regulatory approval is costly and time-consuming, and the successful development of our product candidates is highly uncertain. As a result, we are unable to determine the duration and completion costs of our research and development projects or when and to what extent we will generate revenue from the commercialization and sale of any of our product candidates.

The following table summarizes our research and development expenses incurred during the respective periods:

	Three Months Ended March 31,		$ Change	% Change
	2016	2015		
	(in thousands, except percentages)			
Research and development expenses:				
GBT440 for the treatment of SCD	$7,431	$4,316	$3,115	72 %
Oral treatment for HAE	2,529	1,481	1,048	71 %
IPF and other preclinical programs	2,455	272	2,183	803 %
Total research and development expenses	$12,415	$6,069	$6,346	105 %

Research and development expenses increased by $6.3 million or 105%, to $12.4 million for the three months ended March 31, 2016 from $6.1 million for the three months ended March 31, 2015. The increase was primarily due to increased external expenses related to our SCD program for GBT440 of $3.1 million as we advanced our Phase 1/2 clinical trial in early 2016, along with an increase in activity for other preclinical projects of $2.2 million and an increase in external expenses related to preclinical efforts for our HAE program of $1.0 million.

General and administrative

General and administrative expenses consist of personnel costs, allocated facilities costs and other expenses for outside professional services, including legal, patent, human resources, audit and accounting services. Personnel costs consist of salaries, benefits and stock-based compensation. Allocated expenses consist of expenses for rent and maintenance of facilities, depreciation and amortization expense and other supplies. We expect to incur additional expenses in the future as a result of operating as a public company, including expenses related to compliance with the rules and regulations of the Securities and Exchange Commission, The NASDAQ Global Select Market, additional insurance expenses, investor relations activities and other administrative and professional services.

General and administrative expenses increased by $3.0 million or 231%, to $4.3 million for the three months ended March 31, 2016 from $1.3 million for the three months ended March 31, 2015. The increase was primarily due to an increase of $1.6 million in salaries and benefits, including $0.7 million of related stock-based compensation expense, as a result of our hiring additional personnel and an increase of $1.4 million in professional and consulting services due to our transitioning to a public company from a private company.

Related party expenses

Related party expenses represent fees for management and advisory services provided by Third Rock Ventures, LLC, or TRV, a related party due to its significant equity ownership.

Related party expenses was zero for the three months ended March 31, 2016 compared to $53,000 for the three months ended March 31, 2015. The decrease was due to a reduction in management services which we requested from TRV as we expanded our internal business management team.

Liquidity, Capital Resources and Plan of Operations

We are not profitable and have incurred losses and negative cash flows from operations each year since our inception. Prior to our IPO, our operations were financed primarily by net proceeds from the sale and issuance of convertible preferred stock. In August 2015, we completed our IPO pursuant to which we issued 6,900,000 shares of our common stock at a price to the public of $20.00 per share. We received proceeds of $126.2 million, net of underwriting discounts and commissions, and offering expenses incurred by us. As of March 31, 2016, we had $134.0 million in cash and cash equivalents.

16

Table Of Contents

Our primary use of cash is to fund operations, which consist primarily of research and development expenditures. Cash used to fund operations is impacted by the timing of when we pay these expenses, as reflected in the change in our outstanding accounts payable and accrued expenses.

We believe that our existing capital resources will be sufficient to fund our planned operations until mid-2017. 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. We will continue to require additional financing to advance GBT440 through clinical development, to develop other potential product candidates from our research programs and to fund operations for the foreseeable future. We will continue to seek funds through equity or debt financings, collaborative or other arrangements with corporate sources, or through other sources of financing. Adequate additional funding may not be available to us on acceptable terms, or at all. Our failure to raise capital as and when needed could have a negative impact on our financial condition and our ability to pursue our business strategies. Our future funding requirements will depend on many factors, including:

- the time and cost necessary to complete our ongoing clinical trial that we characterize as a Phase 1/2 trial of GBT440, to initiate and complete any registrational clinical trials of GBT440 and to pursue regulatory approvals for GBT440, and the costs of post-marketing studies that could be required by regulatory authorities;
- the progress and results of our Phase 1/2 clinical trial of GBT440;
- the progress, timing, scope and costs of our nonclinical studies, clinical trials and other related activities, including the ability to enroll subjects in a timely manner for our Phase 1/2 clinical trial of GBT440 and potential future clinical trials;
- the costs of obtaining clinical and commercial supplies of GBT440 and any other product candidates we may identify and develop;
- our ability to advance our other programs, including our program for the development of an orally available kallikrein inhibitor for the prevention of angioedema attacks associated with HAE, through preclinical and clinical development, and the timing and scope of these development activities;
- our ability to successfully commercialize GBT440 and any other product candidates we may identify and develop;
- the manufacturing, selling and marketing costs associated with GBT440 and any other product candidates we may identify and develop, including the cost and timing of expanding our sales and marketing capabilities;
- the amount and timing of sales and other revenues from GBT440 and any other product candidates we may identify and develop, including the sales price and the availability of adequate third-party reimbursement;
- the cash requirements of any future acquisitions or discovery of product candidates;
- the time and cost necessary to respond to technological and market developments;
- the extent to which we may acquire or in-license other product candidates and technologies;
- our ability to attract, hire and retain qualified personnel; and
- the costs of maintaining, expanding and protecting our intellectual property portfolio.

Further, our operating plan may change, and we may need additional funds to meet operational needs and capital requirements for clinical trials and other research and development expenditures. We currently have no credit facility or committed sources of capital. Because of the numerous risks and uncertainties associated with the development and commercialization of our product candidate, we are unable to estimate the amounts of increased capital outlays and operating expenditures associated with our current and anticipated clinical studies and research and development activities.

The following table summarizes our cash flows for the periods indicated:

	Three Months Ended March 31,	
	2016	2015
	(unaudited, in thousands)	
Cash used in operating activities	$ (14,634)	$ (6,061)
Cash used in investing activities	(378)	(169)
Cash provided by (used in) financing activities	494	(39)

Net decrease in cash and cash equivalents	$ (14,518)	$ (6,269)

Table Of Contents

Cash flows from operating activities

Cash used in operating activities for the three months ended March 31, 2016 was $14.6 million, consisting of a net loss of $16.6 million, which was partially offset by non-cash charges of $1.3 million for stock-based compensation and $0.2 million for depreciation and amortization expense. The change in our net operating assets and liabilities was due primarily to an increase of $0.9 million in accounts payable due to timing of payments, an increase of $0.8 million in our accrued liabilities related to the growth of our business as a public company requiring greater legal and regulatory expenses, a decrease of $0.5 million in accrued compensation due to the timing of annual employee bonus payments and a decrease of $0.7 million in our prepaid expenses related to advance payments made in connection with our Phase 1/2 clinical trial of GBT 440 and deposits for the manufacturing of clinical trial materials.

Cash used in operating activities for the three months ended March 31, 2015 was $6.1 million, consisting of a net loss of $7.4 million, which was partially offset by non-cash charges of $0.3 million for stock-based compensation and $0.2 million for depreciation and amortization expense. The change in our net operating assets and liabilities was due primarily to an increase of $0.4 million in prepaid expenses for the advance payments made in connection with our Phase 1/2 clinical trial of GBT 440 and deposits for the manufacturing of clinical trial materials, an increase of $0.3 million in accounts payable and an increase of $0.8 million in accrued liabilities.

Cash flows from investing activities

Cash used in investing activities for the three months ended March 31, 2016 and 2015 was related to our purchase of property and equipment for our office and laboratory facility.

Cash flows from financing activities

Cash provided by financing activities for the three months ended March 31, 2016 was primarily from net proceeds of $0.5 million from the issuance of common stock to participants in the employee stock purchase plan.

Cash used in financing activities for the three months ended March 31, 2015 was primarily related to cash paid for deferred offering costs offset by proceeds from the issuance of restricted stock awards.

Off-Balance Sheet Arrangements

As of March 31, 2016, we had no off-balance sheet arrangements as defined in Item 303(a)(4) of Regulation S-K as promulgated by the SEC.

Contractual Obligations and Other Commitments

There have been no material changes outside the ordinary course of our business related to our contractual obligations during the three months ended March 31, 2016, as compared to those disclosed in our Annual Report.

Recent Accounting Pronouncements

From time to time, new accounting pronouncements are issued by the Financial Accounting Standards Board ("FASB") or other standards setting bodies that are adopted by us as of the specified effective date. Unless otherwise discussed, we believe that the impact of recently issued standards that are not yet effective will not have a material impact on our financial statements upon adoption.

In February 2016, the FASB issued ASU No. 2016-02, Leases. The new standard requires lessees to apply a dual approach, classifying leases as either finance or operating leases based on the principle of whether or not the lease is effectively a financed purchase by the lessee. This classification will determine whether lease expense is recognized based on an effective interest method or on a straight-line basis over the term of the lease. A lessee is also required to record a right-of-use asset and a lease liability for all leases with a term of greater than 12 months regardless of their classification. Leases with a term of 12 months or less will be accounted for similar to existing guidance for operating leases. The standard is effective for interim and annual periods beginning after December 15, 2018, with early adoption permitted. We are currently in the process of evaluating the impact the adoption of this new standard will have on our financial position or results of operations.

Table Of Contents

In March 2016, the FASB issued ASU No. 2016-09, Stock Compensation. The new standard simplifies the accounting for share-based payment transactions, including the income tax consequences, classification of awards as either equity or liabilities, and classification on the statement of cash flows. The standard is effective for annual periods beginning after December 15, 2016, and interim periods within those annual periods. We are currently in the process of evaluating the impact the adoption of this new standard will have on our financial position or results of operations.

Item 3. Quantitative and Qualitative Disclosures about Market Risk

Interest Rate Sensitivity

We are exposed to market risks in the ordinary course of our business. These risks primarily include interest rate sensitivities. We had cash and cash equivalents of $134.0 million as of March 31, 2016 and $148.5 million as of December 31, 2015, which consist of bank deposits and money market funds. Such interest-earning instruments carry a degree of interest rate risk; however, historical fluctuations in interest income have not been significant. We had no outstanding debt as of March 31, 2016 and December 31, 2015.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

As required by Rule 13a-15(b) under the Securities Exchange Act of 1934, as amended (the “Exchange Act”), our management, under the supervision and with the participation of our Chief Executive Officer and Chief Financial Officer, has evaluated 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 March 31, 2016. Based on the evaluation of our disclosure controls and procedures as of March 31, 2016, our Chief Executive Officer and Chief Financial Officer have concluded that, as of March 31, 2016, our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

There have been no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) during the quarter ended March 31, 2016 that have materially affected, or are reasonably likely to materially affect, our internal controls over financial reporting.

Table Of Contents

PART II. OTHER INFORMATION

Item 1. Legal Proceedings

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

Item 1A. Risk Factors.

This Quarterly Report on Form 10-Q contains forward-looking information based on our current expectations. Because our business is subject to many risks and our actual results may differ materially from any forward-looking statements made by or on behalf of us, this section includes a discussion of important factors that could affect our business, operating results, financial condition and the trading price of our common stock. This discussion should be read in conjunction with our financial statements as of March 31, 2016 and December 31, 2015 and the notes accompanying those financial statements.

Risks Related to Our Financial Position and Need for Additional Capital

We are a clinical development-stage biopharmaceutical company with a limited operating history. We have incurred significant losses since our inception and anticipate that we will continue to incur losses for the foreseeable future. We have only one product candidate in clinical development and have not generated any revenue since our inception, which, together with our limited operating history, may make it difficult for you to assess our future viability.

We are a clinical development-stage biopharmaceutical company with a limited operating history upon which you can evaluate our business and prospects. Biopharmaceutical product development is a highly speculative undertaking and involves a substantial degree of risk. To date, we have focused principally on developing our lead product candidate, GBT440, which is our only product candidate in clinical development.

We are not profitable and have incurred losses in each year since our inception in February 2011 and the commencement of our principal operations in May 2012. Our net losses for the three months ended March 31, 2016 and 2015 were $16.6 million and $7.4 million, respectively. As of March 31, 2016, we had an accumulated deficit of $115.1 million. We have not generated any revenue since our inception, and have financed our operations primarily through the sale of equity securities. We continue to incur significant research and development and other expenses related to our ongoing operations and expect to incur losses for the foreseeable future. We anticipate these losses will increase as we:

- continue to advance GBT440 in clinical development;
- establish and maintain manufacturing and supply relationships with third parties that can provide adequate supplies (in amount and quality) of GBT440 to support further clinical development and, if approved, commercialization;
- seek and obtain regulatory and marketing approvals for GBT440;
- build a sales and marketing organization or enter into selected collaborations to commercialize GBT440, if approved;
- advance our other programs, including our programs for the clinical investigation of GBT440 in idiopathic pulmonary fibrosis (IPF) patients with hypoxemia and the development of a proprietary kallikrein inhibitor as an orally administered therapy intended for the prevention of hereditary angioedema (HAE) attacks, through preclinical and clinical development and commence development activities for any additional product candidates we may identify; and
- expand our organization to support our research, development and commercialization activities and our operations as a public company.

We have never generated any revenues from product sales and may never be able to develop or commercialize a marketable drug or achieve profitability. Even if we achieve profitability in the future, we may not be able to sustain profitability in subsequent periods. Our failure to achieve sustained profitability would depress the value of our company and could impair our ability to raise capital, expand our business, diversify our research and development pipeline, market GBT440 or any other product candidates we may identify and pursue, if approved, or continue our operations. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our stockholders' equity and working capital.

We will require substantial additional funding to achieve our business goals. If we are unable to obtain this funding when needed and on acceptable terms, we could be forced to delay, limit or terminate our product development efforts or other operations. Raising additional capital may subject us to unfavorable terms, cause dilution to our existing

stockholders, restrict our operations or require us to relinquish rights to our product candidates and technologies.
We are currently advancing GBT440 through clinical development and conducting preclinical research activities in our other programs. Developing biopharmaceutical products is expensive and time-consuming, and we expect our research and development expenses to increase substantially in connection with our ongoing activities, particularly as we advance GBT440

Table Of Contents

and other product candidates that we may identify and pursue in clinical trials. As of March 31, 2016 and December 31, 2015, we had working capital of $125.0 million and $140.1 million, respectively and capital resources consisting of cash and cash equivalents of $134.0 million and $148.5 million, respectively. Because the outcome of any clinical development and regulatory approval process is highly uncertain, we cannot reasonably estimate the actual capital amounts necessary to successfully complete the development, regulatory approval process and commercialization of GBT440 and any future product candidates.

In August 2015 we sold 6,900,000 shares of common stock in our IPO, the net proceeds of which totaled $126.2 million, after deducting underwriting discounts and commissions and offering expenses incurred by us. We expect that our existing cash and cash equivalents will be sufficient to fund our operations through mid-2017. However, our operating plan may change as a result of many factors currently unknown to us, and we may need to seek additional funds sooner than planned, through public or private equity or debt financings or other sources, such as strategic collaborations or license and development agreements. Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize GBT440 and other product candidates that we may identify and pursue. Moreover, such financing may result in dilution to stockholders, imposition of debt covenants and repayment obligations, or other restrictions that may affect our business. In addition, we may seek additional capital due to favorable market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans.

Our future funding requirements will depend on many factors, including, but not limited to:

- the time and cost necessary to complete our ongoing clinical trial that we characterize as a Phase 1/2 trial of GBT440, to initiate and complete any registrational clinical trials of GBT440 and to pursue regulatory approvals for GBT440, and the costs of post-marketing studies that could be required by regulatory authorities;
- the progress and results of our Phase 1/2 clinical trial of GBT440;
- the progress, timing, scope and costs of our nonclinical studies, clinical trials and other related activities, including the ability to enroll subjects in a timely manner for our Phase 1/2 clinical trial of GBT440 and potential future clinical trials;
- the costs of obtaining clinical and commercial supplies of GBT440 and any other product candidates we may identify and develop;
- our ability to advance our other programs, including our program for the clinical investigation of GBT440 in IPF patients with hypoxemia and the development of a proprietary kallikrein inhibitor as an orally administered therapy intended for the prevention of HAE attacks, through preclinical and clinical development, and the timing and scope of these development activities;
- our ability to successfully commercialize GBT440 and any other product candidates we may identify and develop;
- the manufacturing, selling and marketing costs associated with GBT440 and any other product candidates we may identify and develop, including the cost and timing of establishing our sales and marketing capabilities;
- the amount and timing of sales and other revenues from GBT440 and any other product candidates we may identify and develop, including the sales price and the availability of adequate third-party reimbursement;
- the cash requirements of any future acquisitions or discovery of product candidates;
- the time and cost necessary to respond to technological and market developments;
- the extent to which we may acquire or in-license other product candidates and technologies;
- our ability to attract, hire and retain qualified personnel; and
- the costs of maintaining, expanding and protecting our intellectual property portfolio.

Additional funds may not be available when we need them, on terms that are acceptable to us, or at all. If adequate funds are not available to us on a timely basis, we may be required to delay, limit or terminate the clinical development of GBT440 in SCD or one or more of our other research or development programs or the commercialization of any product candidates or be unable to expand our operations or otherwise capitalize on our business opportunities, as desired, which could materially and adversely affect our business, prospects, financial condition and results of operations.

Risks Related to Our Business and the Clinical Development, Regulatory Review and Approval of Our Product Candidates

If we are unable to obtain regulatory approval in one or more jurisdictions for GBT440 or any other product candidates that we may identify and develop, our business will be substantially harmed.

We cannot commercialize a product until the appropriate regulatory authorities have reviewed and approved the product candidate. Approval by the FDA and comparable foreign regulatory authorities is lengthy and unpredictable, and depends upon numerous factors. Approval policies, regulations, or the type and amount of clinical data necessary to gain approval may

Table Of Contents

change during the course of a product candidate's clinical development and may vary among jurisdictions, which may cause delays in the approval or the decision not to approve an application. We have not obtained regulatory approval for any product candidate, including GBT440, and it is possible that neither GBT440 nor any other product candidates we may seek to develop in the future will ever obtain regulatory approval.

Applications for GBT440 or any other product candidates we may develop could fail to receive regulatory approval for many reasons, including but not limited to:

- our inability to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that GBT440 or any other product candidate we may develop is safe and effective for each of its intended indications;
- the FDA or comparable foreign regulatory authorities may disagree with our plans regarding the pathways for approval or the design or implementation of our clinical trials;
- the population studied in the clinical program may not be sufficiently broad or representative to assure safety in the full population for which we seek approval;
- the FDA or comparable foreign regulatory authorities may require additional preclinical studies or clinical trials beyond those we anticipate;
- the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from nonclinical studies or clinical trials;
- the data collected from clinical trials of GBT440 and other product candidates that we may identify and pursue may not be sufficient to support the submission of a new drug application, or NDA, or other submission for regulatory approval in the United States or elsewhere;
- we may be unable to demonstrate to the FDA or comparable foreign regulatory authorities that a product candidate's risk-benefit ratio for its proposed indication is acceptable;
- the FDA or comparable foreign regulatory authorities may fail to approve the manufacturing processes, test procedures and specifications, or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may change in a manner that renders our clinical trial design or data insufficient for approval.

The lengthy approval process, as well as the unpredictability of the results of clinical trials, may result in our failing to obtain regulatory approval to market GBT440 and other product candidates that we may pursue in the United States or elsewhere, which would significantly harm our business, prospects, financial condition and results of operations.

We are heavily dependent on the success of our lead product candidate, GBT440, and all of our other programs are still in the preclinical development stage. If we are unable to successfully complete clinical development, obtain regulatory approval for, or commercialize GBT440, or experience delays in doing so, our business will be materially harmed.

To date, we have invested a majority of our efforts and financial resources in the preclinical and clinical development of GBT440, including conducting preclinical studies and clinical trials and providing general and administrative support for these operations. Our future success is dependent on our ability to successfully develop, obtain regulatory approval for, and commercialize GBT440. Before we can generate any revenues from sales of GBT440, we will be required to conduct additional clinical development, including, among other things, additional toxicology studies that may be required before we can conduct longer-term clinical trials and a larger registrational clinical trial if our ongoing clinical trial of GBT440 is successful, seek and obtain regulatory approval, secure adequate manufacturing supply to support larger clinical trials and commercial sales and build a commercial organization. Further, the success of GBT440 will depend on patent and trade secret protection, acceptance of GBT440 by patients, the medical community and third-party payors, its ability to compete with other therapies, healthcare coverage and reimbursement, and maintenance of an acceptable safety profile following approval, among other factors. If we do not achieve one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize GBT440, which would materially harm our business.

GBT440 is currently our only product candidate to have advanced into what we characterize as a Phase 1/2 clinical trial, and it may be years before GBT440 can advance into a registrational study, if at all. All of our other programs

are in an early stage of research and development. Although we have nominated for Investigational New Drug application, or IND, enabling toxicology studies a novel, small molecule, orally available kallikrein inhibitor product candidate for the prevention of angioedema attacks associated with HAE, the data generated in these studies may not be adequate to support the filing of an IND or for clinical evaluation, and we have not yet selected any other product candidates that would enable the filing of an IND. We cannot be certain that GBT440 will be successful in clinical trials or receive regulatory approval. If we do not receive regulatory approval for, or otherwise fail to successfully commercialize, GBT440 or any other product candidate, we may need to spend significant additional time and resources to identify other product candidates, advance them through preclinical and

Table Of Contents

clinical development and apply for regulatory approvals, which would adversely affect our business, prospects, financial condition and results of operations.

The development of GBT440 as a potential disease-modifying anti-sickling agent represents a novel therapeutic approach to SCD treatment, and there is a risk that the outcome of our clinical trials will not be favorable.

We have concentrated our therapeutic product research and development efforts on developing novel, mechanism-based therapeutics for the treatment of grievous blood-based disorders with significant unmet need, including SCD, and our future success depends on the successful development of this therapeutic approach. The clinical trial requirements of the FDA and other regulatory agencies and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of the potential product. At the moment, there is only one approved therapy for SCD, hydroxyurea, and there are no approved therapeutics directed toward preventing the polymerization of hemoglobin molecules as a mechanism to reduce RBC sickling in SCD patients. As a result, the design and conduct of clinical trials for a therapeutic that targets this mechanism in SCD are subject to unknown risks, and we may experience setbacks with our ongoing or planned clinical trials of GBT440 because of the limited clinical experience with its mechanism of action in SCD patients. In particular, regulatory authorities in the United States have not issued definitive guidance as to how to measure and achieve efficacy in SCD. Although we are evaluating exploratory endpoints, including anti-sickling and anti-hemolytic effects, changes in hemoglobin levels, and reticulocyte counts, for GBT440 in our Phase 1/2 clinical trial, regulators have not determined that such data signifies a clinically meaningful result in SCD patients or can support advancement into registrational trials or regulatory approval. We may not achieve our pre-specified endpoints in our Phase 1/2 clinical trial or in other clinical trials where there is limited or no regulatory guidance regarding appropriate clinical endpoints, which would decrease the probability of obtaining marketing approval for GBT440 or any other product candidate we may develop. Any inability to design clinical trials with protocols and endpoints acceptable to applicable regulatory authorities, and to obtain regulatory approvals for GBT440 and other product candidates that we may pursue, would have an adverse impact on our business, prospects, financial condition and results of operations.

Results of earlier studies may not be predictive of future clinical trial results, and initial studies may not establish an adequate safety or efficacy profile for GBT440 and other product candidates that we may pursue to justify proceeding to advanced clinical trials or an application for regulatory approval.

The results of nonclinical and preclinical studies and clinical trials of GBT440 and other product candidates that we may pursue may not be predictive of the results of later-stage clinical trials, and interim results of a clinical trial do not necessarily predict final results. For example, our preclinical studies and clinical trials of GBT440 to date have involved only one genotype of SCD, HbSS, and the results of these studies may not be replicated in other genotypes of SCD or in subsequent clinical trials. Additionally, any positive results generated in our Phase 1/2 clinical trial of GBT440 in adults would not ensure that we will achieve similar results in larger, registrational clinical trials or in clinical trials of GBT440 in pediatric populations. In addition, preclinical and clinical data are often susceptible to various interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval. Product candidates in later stages of clinical trials may fail to demonstrate the desired safety and efficacy despite having progressed through nonclinical studies and initial clinical trials. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier studies, and we cannot be certain that we will not face similar setbacks. 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 GBT440 or any other product candidate we may choose to develop in any ongoing or future clinical trials would substantially harm our business, prospects, financial condition and results of operations.

Before we are able to submit GBT440 for marketing approval, the FDA and comparable foreign regulatory authorities will require that we conduct additional clinical trials and may impose additional requirements, the scope of which are

not known at this time.

Before we can submit an NDA to the FDA for GBT440, we must successfully complete our ongoing clinical trial and one or more additional larger clinical trials. The FDA typically requires at least two pivotal, well-controlled clinical trials as a condition to the submission of an NDA and does not consider a single clinical trial to be adequate to support product approval. The FDA will typically only consider relying on one pivotal trial if, in addition, other well-controlled studies of the drug exist (for example, for other dosage forms or in other populations) or if the pivotal trial is a multi-center trial that provides highly

Table Of Contents

reliable and statistically strong evidence of an important clinical benefit, such as effect on survival, organ function or patient reported outcomes and a confirmatory study would have been difficult to conduct on ethical grounds. Although we characterize our current clinical trial of GBT440 as a Phase 1/2 clinical trial because it is designed to evaluate exploratory endpoints that we believe may be clinically relevant to SCD patients, it is possible that, even if we achieve favorable results in our first clinical trial of GBT440, the FDA may require us to conduct one or more additional clinical trials, possibly involving a larger sample size or a different clinical trial design, before we can initiate a pivotal trial. The FDA may also require that we conduct additional toxicology studies before evaluating GBT440 in longer term clinical trials or impose a longer follow-up period for subjects treated with GBT440 prior to accepting an NDA submission.

It is possible that the FDA or the comparable foreign authorities may not consider the results of our ongoing and planned clinical trials to be sufficient for approval of GBT440 for SCD or IPF. If the FDA or comparable foreign regulatory authorities require additional clinical trials or data beyond that which we currently anticipate, we would incur increased costs and delays in the clinical development and marketing approval process, which may require us to expend more resources than are available to us. In addition, it is possible that the FDA and the comparable foreign authorities may have divergent opinions on the elements necessary for a successful NDA and Marketing Authorization Application, or MAA, respectively, which may cause us to alter our development, regulatory and/or commercialization strategies.

We may encounter substantial delays in completing our clinical trials, which in turn will result in additional costs and may ultimately prevent successful or timely completion of the clinical development and commercialization of our product candidates.

Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of the product candidates in humans. Clinical testing is expensive, time-consuming and uncertain as to the outcome. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. A failure of one or more clinical trials can occur at any stage of testing. Events that may prevent successful or timely completion of clinical development include:

- delays in reaching, or any failure to reach, a consensus with regulatory agencies on study design;
- delays in reaching, or any failure to reach, agreement on acceptable terms with a sufficient number of prospective contract research organizations, or CROs, and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- delays in obtaining required Institutional Review Board, or IRB, approval at each clinical trial site;
- delays in recruiting a sufficient number of suitable patients to participate in our clinical trials;
- imposition of a clinical hold by regulatory agencies, after an inspection of our clinical trial operations or study sites;
- failure by our CROs, other third parties or us to adhere to clinical trial, regulatory or legal requirements;
- failure to perform in accordance with the FDA's good clinical practices, or GCP, or applicable regulatory guidelines in other countries;
- delays in the testing, validation, manufacturing and delivery of sufficient quantities of our product candidates to the clinical sites;
- delays in having patients complete participation in a study or return for post-treatment follow-up;
- clinical trial sites or patients dropping out of a trial;
- failure to address in an adequate or timely manner any patient safety concerns that arise during the course of a trial;
- unanticipated costs or increases in costs of clinical trials of our product candidates;
- occurrence of serious adverse events or other safety concerns associated with the product candidate that are viewed to outweigh its potential benefits; or
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols.

We could also encounter delays if a clinical trial is suspended or terminated by us, by the IRBs of the institutions in which such trials are being conducted, by an independent Safety Review Board for such trial or by the FDA or other regulatory authorities. Such authorities may suspend or terminate a clinical trial due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of

the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, and failure to demonstrate a benefit from using a drug. In addition, if we make manufacturing or formulation changes to our product candidates, we may need to conduct additional studies to bridge our modified product candidates to earlier versions.

Clinical trial delays could shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates. In addition, any delays in completing our clinical trials will increase our

Table Of Contents

costs, slow down our product candidate development and approval process and jeopardize our ability to obtain regulatory approvals, commence product sales and generate revenues. Any of these occurrences may significantly harm our business, prospects, financial condition and results of operations.

Difficulty in enrolling patients or maintaining patient compliance with dosing requirements in our clinical trials could delay or prevent clinical trials of our product candidates, which in turn could delay or prevent our ability to obtain the regulatory approvals necessary to commercialize our product candidates.

Identifying and qualifying patients to participate in our ongoing and planned clinical trials of GBT440 and any other product candidates that we may develop are critical to our success. Our clinical development efforts are initially focused on rare chronic blood diseases. Accordingly, there are limited patient pools from which to draw for clinical trials. For example, according to CDC estimates, the prevalence of SCD, for which GBT440 is being studied, is 90,000 to 100,000 individuals in the United States. Although genetic screening for SCD is mandatory for newborns in the United States, we may not be able to identify, recruit, and enroll a sufficient number of subjects to complete our clinical trials of GBT440 because of the perceived risks and benefits of GBT440, the availability of competing therapies and clinical trials, the proximity and availability of clinical trial sites for prospective subjects and the subject referral practices of physicians. Further, if subjects in our clinical trials fail to comply with our dosing regimens, we may not be able to generate clinical data acceptable to the FDA in our trials. If patients are unwilling to participate in our studies for any reason, the timeline for recruiting subjects, conducting studies and obtaining regulatory approval of potential products may be delayed.

If we experience difficulties or delays in enrollment or are otherwise unable to successfully complete any clinical trial of GBT440 or our other product candidates, our costs may increase, and our ability to obtain regulatory approval and generate product revenue from any of these product candidates will be impaired. Any of these occurrences would harm our business, prospects, financial condition and results of operations.

If serious adverse events or unacceptable side effects are identified during the development of our product candidates, we may need to delay, limit or terminate our clinical development activities.

Clinical trials by their nature utilize a sample of the potential patient population. Our Phase 1/2 clinical trial of GBT440 is designed to enroll between 96 and 128 subjects. Any rare and severe side effects of GBT440 may be uncovered only in later stages of our Phase 1/2 trial or only in any larger, subsequent trials that we may conduct. Many product candidates that initially showed promise in early stage testing have later been found to cause side effects that prevented their further development. Moreover, a preclinical toxicology study with GBT440 in non-humans and clinical trials involving other hemoglobin modifiers have shown a decrease in oxygen delivery to tissue when the percentage of modified hemoglobin is significant. Hemoglobin modifiers, by increasing HbS's affinity for oxygen, can cause a shift in oxygen levels, potentially resulting in tissue hypoxia. If GBT440 or any product candidates that we may develop are associated with tissue hypoxia or undesirable side effects in clinical trials or have characteristics that are unexpected, we may need to abandon their development or limit their development to more narrow uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective, which could adversely affect our business, prospects, financial condition and results of operations.

Although we intend to pursue expedited regulatory approval pathways for GBT440, it may not qualify for expedited development or, if it does qualify for expedited development, it may not actually lead to a faster development or regulatory review or approval process.

Although we believe there may be an opportunity to accelerate the development of GBT440 through one or more of the FDA's expedited programs, such as fast track, breakthrough therapy, accelerated approval or priority review, and we intend to pursue one or more of these expedited programs, we cannot be assured that GBT440 or any other product candidates that we may develop will qualify for such programs.

In October 2015, the FDA designated our investigation of GBT440 for the treatment of SCD as a Fast Track development program. Fast Track is a process designated to facilitate the development and expedite the review of drugs to treat serious conditions and that demonstrate the potential to address an unmet medical need. While Fast Track designation may provide more frequent access and communication with the FDA, it does not ensure that regulatory approval for GBT440 will occur on an expedited basis.

In addition, a drug may be eligible for designation as a breakthrough therapy if the drug is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening condition and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant

Table Of Contents

endpoints. Although breakthrough designation or access to any other expedited program may expedite the development or approval process, it does not change the standards for approval. If we apply for breakthrough therapy designation or any other expedited program for GBT440, the FDA may determine that GBT440, our proposed target indication or other aspects of our clinical development plans do not qualify for such expedited program. Even if we are successful in obtaining a breakthrough therapy designation or access to any other expedited program, we may not experience faster development timelines or achieve faster review or approval compared to conventional FDA procedures.

Furthermore, access to an expedited program may also be withdrawn by the FDA if it believes that the designation is no longer supported by data from our clinical development program. Additionally, qualification for Fast Track or any other expedited review procedure does not ensure that we will ultimately obtain regulatory approval for GBT440 or any other product candidate that we may develop in a timely manner, or at all.

Although the FDA has granted orphan drug designation to GBT440 for the treatment of SCD, we may not receive orphan drug designation for GBT440 in other jurisdictions or for other indications that we may pursue, or for any other product candidates for which we may submit new applications for orphan drug designation, and any orphan drug designations that we have received or may receive may not confer marketing exclusivity or other expected commercial benefits.

Our business strategy focuses on the development of product candidates for the treatment of rare, chronic blood disorders that may be eligible for FDA or European Union, or EU, orphan drug designation. Regulatory authorities in some jurisdictions, including the United States and the EU, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a drug as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals annually in the United States. In the EU, the Committee for Orphan Medicinal Products of the European Medicines Agency, or EMA, grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than five in 10,000 persons in the EU (or where it is unlikely that the development of the medicine would generate sufficient return to justify the investment) and for which no satisfactory method of diagnosis, prevention, or treatment is authorized or, if a method exists, the product would be of significant benefit to those affected by the condition. In December 2015, the FDA granted orphan drug designation for GBT440 for the treatment of patients with SCD. Generally, if a drug with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the drug is entitled to a period of marketing exclusivity, which precludes the FDA or the EMA from approving another marketing application for the same drug for that time period. The applicable period is seven years in the United States and 10 years in the EU. The EU exclusivity period can be reduced to six years if a drug no longer meets the criteria for orphan drug designation or if the drug is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be lost if the FDA or EMA determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition.

Although the FDA has granted orphan drug designation to GBT440 for the treatment of SCD, we may apply for orphan drug designation for GBT440 in other jurisdictions or for other indications, or for other product candidates we may develop, and applicable regulatory authorities may not grant us these additional designations. In addition, the exclusivity granted under any orphan drug designations that we have received or may receive may not effectively protect the product candidate from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved, the FDA can subsequently approve another drug for the same condition if the FDA concludes that the later drug is clinically superior, in that it is shown to be safer, more effective or makes a major contribution to patient care. Any inability to secure or maintain orphan drug designation or the exclusivity benefits of this designation would have an adverse impact on our ability to develop and commercialize our product candidates.

Even if we receive regulatory approval for GBT440 or any other product candidate that we may develop, we will be subject to ongoing regulatory obligations and scrutiny and may be subject to product labeling and other post-marketing restrictions.

Even if a product candidate is approved, regulatory authorities may still impose significant restrictions on its indicated uses or marketing or impose ongoing requirements for potentially costly post-marketing studies. Furthermore, any new legislation addressing drug safety issues could result in delays or increased costs to assure compliance. If GBT440 or any other product candidates that we may develop are approved, they will each be subject to ongoing regulatory requirements for labeling, packaging, storage, advertising, promotion, sampling, record-keeping and submission of safety and other post-market information, including both federal and state requirements in the United States. In addition, regulatory agencies may not approve the labeling claims that are necessary or desirable for the successful commercialization of our product candidates. For

Table Of Contents

example, the development of GBT440 for the prophylactic treatment of SCD in pediatric patients is an important part of our current business strategy, and if we are unable to obtain regulatory approval for the desired age ranges, our business may suffer.

In addition, manufacturers and manufacturers' facilities are required to comply with extensive FDA requirements, including ensuring that quality control and manufacturing procedures conform to