Global Blood Therapeutics, Inc.
Form 424B4
June 21, 2016
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Filed Pursuant to Rule 424(b)(4)
Registration File No. 333-211976

Prospectus

6,400,000 Shares

COMMON STOCK

Global Blood Therapeutics, Inc. is offering 6,400,000 shares of common stock. Our common stock is listed on The NASDAQ Global Select Market under the symbol GBT. The last reported sale price of our common stock on The NASDAQ Global Select Market on June 20, 2016 was $18.94 per share.

We are an emerging growth company as that term is used in the Jumpstart Our Business Startups Act of 2012, as amended, and, as such, have elected to comply with certain reduced public company reporting requirements for this prospectus and future filings.

Investing in our common stock involves risks. See Risk Factors beginning on page 11.

PRICE $18.75 A SHARE

	Price to Public	Underwriting Discounts and Commissions [1]	Proceeds, before expenses, to Global Blood Therapeutics, Inc.
Per Share	$ 18.75	$ 1.1250	$ 17.6250
Total	$ 120,000,000	$ 7,200,000	$ 112,800,000

(1) The underwriters will receive compensation in addition to underwriting discounts and commissions. See Underwriting beginning on page 100 for additional information regarding underwriting compensation.

We have granted the underwriters an option to purchase up to 960,000 additional shares of our common stock from us at the public offering price, less underwriting discounts and commissions. The underwriters can exercise this option at any time within 30 days after the date of this prospectus.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or determined if this prospectus is truthful or complete. Any representation to the contrary is a criminal offense.

The underwriters expect to deliver the shares of common stock to purchasers on or about June 24, 2016.

J.P. Morgan **Morgan Stanley**

Cowen and Company **Wedbush PacGrow**

June 20, 2016

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We and the underwriters have not authorized anyone to provide any information other than that contained or incorporated by reference in this prospectus or in any free writing prospectus prepared by or on behalf of us or to which we have referred you. We take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may give you. We and the underwriters are not making an offer to sell these securities in any jurisdiction where the offer or sale is not permitted. You should assume that the information appearing or incorporated by reference in this prospectus is accurate only as of its date. Our business, financial condition, results of operations and prospects may have changed since the respective dates.

For investors outside of the United States: We have not, and the underwriters have not, done anything that would permit this offering or possession or distribution of this prospectus in any jurisdiction where action for that purpose is required, other than the United States. Persons outside of the United States who come into possession of this prospectus must inform themselves about, and observe any restrictions relating to, the offering of the shares of common stock and the distribution of this prospectus outside of the United States.

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PROSPECTUS SUMMARY

This summary highlights information contained elsewhere and incorporated by reference in this prospectus and does not contain all of the information that you should consider in making your investment decision. Before investing in our common stock, you should carefully read this entire prospectus, including the section titled Risk Factors and the information in our filings with the U.S. Securities and Exchange Commission, or the SEC, incorporated by reference in this prospectus. Unless the context suggests otherwise, all references to us, our, GBT, we, the Company and similar designations refer to Global Blood Therapeutics, Inc. and, where appropriate, our subsidiaries.

Global Blood Therapeutics, Inc.

Our Company

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. In June 2016, we initiated clinical sites which began screening for a Phase 2a clinical trial of GBT440 in idiopathic pulmonary fibrosis, or IPF, and we expect to initiate a Phase 2b clinical trial of GBT440 in a hypoxemic pulmonary disorder in the second half of 2016. We are also engaged in other research and development activities targeted towards hereditary angioedema, or HAE. In 2015, we nominated GBT018713, a proprietary, small molecule kallikrein inhibitor, for development as an orally administered therapy intended for the prevention of HAE attacks. We plan to complete toxicology studies to enable the filing of an Investigational New Drug (IND) application, and subject to submission and clearance of the IND, we expect to initiate a Phase 1 clinical trial for GBT018713 in early 2017. We own or jointly own and have exclusively licensed rights to our portfolio of product candidates in the United States, Europe and other major markets. We own two issued U.S. patents that cover the composition of matter and method of use for GBT440, which are due to expire in 2032 and 2034, respectively (absent any applicable patent term extensions), and we own or co-own additional pending patent applications in the United States and selected foreign countries.

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.

Overview of Sickle Cell Disease

SCD is a genetic blood disorder caused by a single point mutation in the beta-chain of hemoglobin, which results in the formation of abnormal hemoglobin known as sickle hemoglobin, or HbS. Normally, oxygenated

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RBCs travel from the lung through blood vessels. Hemoglobin, the oxygen-carrying protein inside RBCs, releases oxygen at the tissues. In SCD, when oxygen is released at the tissues, HbS becomes sticky and aggregates into polymers, or long, rigid rods within an RBC, much like a sword within a balloon. The RBC assumes a sickled shape and becomes inflexible, which can cause blockage in small blood vessels. These polymers destroy RBCs and block blood flow, resulting in decreased oxygen delivery to tissues. Beginning in early childhood, SCD patients suffer many clinical consequences, including unpredictable and recurrent episodes, or crises, of severe chronic and acute pain, anemia, stroke, spleen failure, pulmonary hypertension, acute chest syndrome, liver disease, kidney failure, other morbidities, and premature death. These consequences are directly related to reduced blood flow and insufficient oxygen delivery. A 2014 publication noted that in the United States, SCD resulted in a shortened patient life expectancy by approximately 25 to 30 years even with available therapies.

Current treatment options for SCD are limited to hydroxyurea, or HU, blood transfusions and bone marrow transplantation. The utilization of these treatments is significantly limited due to their suboptimal efficacy and significant toxicity. As a result, patients with SCD continue to suffer serious morbidity and premature mortality.

We believe there is a significant unmet medical need for a novel SCD therapy that:

- inhibits abnormal hemoglobin polymer formation, the underlying mechanism of RBC sickling;
- stops inappropriate RBC destruction and improves blood flow and oxygen delivery to tissues;
- prevents or reduces the episodes or crises of severe pain associated with SCD;
- modifies the long-term course of the disease;
- is effective in all SCD genotypes, and in both children and adults;
- has a more favorable side effect profile than currently available therapies; and
- is available as a convenient, oral therapy.

Our Product Candidate

GBT440 s therapeutic approach was inspired by the natural activity of fetal hemoglobin, or HbF. HbF, which is present during fetal development and in early infancy until it is replaced with adult hemoglobin, has an inherently increased oxygen affinity that allows a fetus to extract oxygen from the mother s blood. Typically, newborns with SCD do not experience RBC sickling until approximately six to nine months of age, after which HbF is usually no longer expressed. Additionally, it has been observed that rare individuals who have inherited both the HbS mutation as well as a gene deletion that allows them to continue to express 10 to 30% HbF in their RBCs into adulthood do not exhibit the clinical manifestations of SCD, despite expressing up to 90% HbS in their blood. HbF dilutes the concentration of deoxygenated HbS that can participate in polymerization, and thereby prevents hemoglobin polymers from forming.

GBT440 is a novel, investigational drug that increases hemoglobin s affinity for oxygen by binding to the alpha-chain of hemoglobin. GBT440 has been observed to keep a proportion of sickle hemoglobin in its oxygenated state, which cannot participate in polymerization. Similar to HbF, by diluting total HbS with a proportion of GBT440-bound hemoglobin, GBT440 prevents hemoglobin polymer formation.

In December 2014, we initiated our randomized, placebo-controlled, double-blind, single and multiple ascending dose Phase 1/2 clinical trial of GBT440 in healthy subjects and subjects with SCD. The study is being conducted in three parts: Part A (single dose administration), Part B (multiple dose administration, daily for 15 days in healthy subjects and 28 days in SCD subjects), and Part C (multiple dose administration, daily for 90

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days in SCD subjects). We are evaluating the safety, tolerability, pharmacokinetics, or PK, and pharmacodynamics, or PD, of GBT440, as well as exploratory markers of SCD activity, including anti-hemolytic effects and SCD-related clinical effects. We reported initial results from our Phase 1/2 clinical trial at the American Society of Hematology meeting in December 2015, and additional results from this trial at the European Hematological Association (EHA) meeting in June 2016. We believe the observations from the trial to date demonstrate a favorable safety profile and pharmaceutical properties, and the potential for GBT440 to serve as a disease-modifying therapy for SCD. In the third quarter of 2016, we intend to engage in discussions with U.S. and European regulatory authorities to define the future development plan for GBT440. In 2015, the FDA granted Fast Track Designation and Orphan Drug Designation for GBT440 for the treatment of SCD.

Market Opportunity in SCD

We believe there is a significant market opportunity in SCD. The U.S. Centers for Disease Control, or CDC, estimates the prevalence of SCD at 90,000 to 100,000 individuals in the United States, where newborn screening is mandatory. It is estimated that the prevalence of SCD in Europe is approximately 60,000. The global incidence of SCD is estimated to be 250,000 to 300,000 births annually. One study estimated that in the United States, the average annual cost for the care of an adult patient with the most common genotype of SCD exceeds $200,000, and the cumulative lifetime cost exceeds $8.0 million over an assumed 50-year lifespan, driven primarily by hospital admissions, physician fees, clinic and emergency department visits, and the costs of diagnostic procedures and outpatient consultations.

Given a concentrated prescriber base for SCD and the small number of key opinion leaders who significantly influence the treatments for this patient population, we intend to promote GBT440 with a specialty sales force in the United States and Europe. We are also evaluating options for commercializing GBT440 in other significant markets due to the concentration of SCD patient populations in sub-Saharan Africa, the Middle East, South Asia and Latin America.

Additional Opportunities

Beyond SCD, building on data from preclinical models of hypoxemia, we initiated clinical sites which began screening for a Phase 2a clinical trial in IPF in June 2016, and we expect to initiate a Phase 2b clinical trial of GBT440 in a hypoxemic pulmonary disorder in the second half of 2016. Results from these clinical trials will guide further clinical development in IPF, as well as other chronic and acute hypoxemic pulmonary disorders. A 2012 publication estimated that there are approximately 90,000 patients with IPF in the United States.

Additionally, in 2015, we nominated GBT018713, a proprietary, small molecule kallikrein inhibitor, for development as an orally administered therapy intended for the prevention of HAE attacks. All currently marketed therapeutics for HAE must be administered intravenously or by subcutaneous injection. As a result, we believe that the availability of a safe and effective oral agent targeting a validated mechanism that prevents HAE attacks would have the potential to transform the treatment paradigm for this disease. We plan to complete toxicology studies to enable the filing of an IND application, and subject to submission and clearance of the IND, we expect to initiate a Phase 1 study for GBT018713 in early 2017.

Management

We have assembled a team of employees, directors and scientific founders rich in scientific experience and capabilities in drug discovery, development and commercialization. Our management has a successful track record in developing and commercializing drug candidates. In aggregate, our team has contributed to 18 drug approvals,

including Avastin, CellCept, Herceptin, INTEGRILIN, Kaletra, Kyprolis and Rituxan. We intend to leverage this expertise and experience to rapidly pursue the development of GBT440 and any other product candidates we may identify and develop.

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Our Strategy

Our strategy is to use our expertise in blood biology to build a multi-product company leading in the discovery, development and commercialization of novel medicines for grievous blood-based disorders. Key elements of our strategy include to:

- rapidly advance GBT440 for the treatment of SCD;
- explore the clinical development of GBT440 in IPF patients with hypoxemia, as well as in other chronic and acute hypoxemic pulmonary disorders;
- submit an Investigational New Drug application, or IND, and initiate clinical development for an oral kallikrein inhibitor in HAE; and
- evaluate opportunities to expand the scope of our product offerings.

Financial Overview

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. Our operating expenses increased during the first quarter of 2016 and we expect that they will increase during the remainder of 2016 and beyond, particularly as we continue the development of GBT440 in SCD, including the possible initiation of clinical trials in pediatric patients and pivotal clinical trials in adults and adolescents in the second half of 2016, and as we initiate clinical trials of GBT440 in IPF in the second quarter of 2016.

Our Development Pipeline

The following table summarizes our development programs, potential indications and their current stages of development:

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Risks Associated with Our Business

Our ability to implement our business strategy is subject to numerous risks that you should be aware of before making an investment decision. These risks are described more fully in the section entitled Risk Factors in this prospectus. These risks include, among others:

- We are a clinical development-stage company with a limited operating history, have incurred significant losses since our inception, and anticipate that we will continue to incur significant losses for the foreseeable future;
- Even if this offering is successful, we will need to raise additional funding before we can expect to generate any revenues from product sales;
- If we are unable to obtain regulatory approval for GBT440 or any other product candidates that we may identify or develop, our business will be substantially harmed;
- We are heavily dependent upon the success of GBT440, which is in the early stages of clinical development;
- Results of earlier studies may not be predictive of future clinical trial results, and we may fail to establish an adequate safety or efficacy profile to conduct advanced clinical trials or obtain regulatory approval for GBT440 or any other product candidates that we may pursue;
- If we are unable to obtain and maintain sufficient intellectual property protection for GBT440, our technologies, or any future product candidates, we may not be able to compete effectively in our markets; and
- Our future success depends in part upon our ability to retain our key employees, consultants and advisors and to attract, retain and motivate other qualified personnel.

Implications of Being an Emerging Growth Company

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

- reduced disclosure about our executive compensation arrangements;

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

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

We may take advantage of these exemptions for up to five years from our initial public offering or such earlier time that we are no longer an emerging growth company. We would cease to be an emerging growth company on the date that is the earliest of (i) the last day of the fiscal year in which we have total annual gross revenues of $1.0 billion or more; (ii) December 31, 2020; (iii) the date on which we have issued more than $1.0 billion in nonconvertible debt during the previous three years; or (iv) the last day of the fiscal year in which we are deemed to be a large accelerated filer under the rules of the Securities and Exchange Commission, or SEC, which means the market value of our common stock that is held by non-affiliates exceeds $700 million as of the prior June 30th. We may choose to take advantage of some but not all of these exemptions. We have taken advantage of reduced reporting requirements in this prospectus. Accordingly, the information contained herein may be different from the information you receive from other public companies in which you hold stock. Also, we have irrevocably elected to opt out of the exemption for the delayed adoption of certain accounting standards and, therefore, will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

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Corporate History and Information

We were incorporated under the laws of the State of Delaware in February 2011. Our principal executive office is located at 400 East Jamie Court, Suite 101, South San Francisco, California, and our telephone number is (650) 741-7700. Our website address is *www.globalbloodtx.com*. We do not incorporate the information on or accessible through our website into this prospectus, and you should not consider any information on, or that can be accessed through, our website as part of this prospectus.

We use various trademarks and trade names in our business, including without limitation our corporate name and logo. All other trademarks or trade names referred to in this prospectus are the property of their respective owners. Solely for convenience, the trademarks and trade names in this prospectus may be referred to without the ® and symbols, but such references should not be construed as any indicator that their respective owners will not assert, to the fullest extent under applicable law, their rights thereto.

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THE OFFERING

Common stock offered by us	6,400,000 shares
Common stock to be outstanding after this offering	35,936,449 shares
Underwriters option	We have granted the underwriters an option to purchase a maximum of 960,000 additional shares of common stock from us. The underwriters can exercise this option at any time within 30 days from the date of this prospectus.
Use of Proceeds	We estimate that we will receive net proceeds from the sale of shares of our common stock in this offering of approximately $112.3 million, or $129.2 million if the underwriters fully exercise their option to purchase additional shares, and after deducting underwriting discounts and commissions and estimated offering expenses payable by us. We intend to use the net proceeds from this offering and our existing cash and cash equivalents to fund our clinical development of GBT440 for the treatment of SCD, including the completion of our ongoing Phase 1/2 clinical trial, planned clinical pharmacology studies and through the initiation of a pivotal clinical trial, our planned clinical trials of GBT440 for the treatment of IPF and other hypoxemic pulmonary disorders, our completion and filing of an IND and commencement of clinical development of GBT018713 for the treatment of HAE, our other research and development activities, and for working capital and general corporate purposes. See Use of Proceeds for additional information.
Risk Factors	You should read carefully Risk Factors beginning on page 11 and other information included in, or incorporated by reference into, this prospectus for a discussion of factors that you should consider before deciding to invest in shares of our common stock.
NASDAQ Global Select Market symbol	GBT

The number of shares of common stock to be outstanding after this offering is based on 29,536,449 shares of common stock outstanding as of March 31, 2016 and excludes:

- 2,487,374 shares of common stock issuable upon exercise of outstanding options as of March 31, 2016 at a weighted average exercise price of $9.67 per share;

992,176 shares of restricted common stock which were subject to our right of repurchase as of March 31, 2016;

115,900 shares of common stock issuable upon exercise of options granted subsequent to March 31, 2016 at a weighted-average exercise price of $19.38 per share;

2,379,284 shares of common stock reserved for future issuance under our 2015 Stock Option and Incentive Plan, or the 2015 Plan, as of March 31, 2016; and

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112,100 shares of common stock reserved for future issuance under our 2015 Employee Stock Purchase Plan, or the 2015 ESPP, as of March 31, 2016.

Except as otherwise indicated, all information in this prospectus assumes no exercise by the underwriters of their option to purchase an additional 960,000 shares of our common stock in this offering.

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SUMMARY FINANCIAL DATA

The following tables present summary financial data for our business. We derived the following statements of operations data for the years ended December 31, 2015, 2014 and 2013 from our audited financial statements. We derived the statements of operations data for the three months ended March 31, 2016 and 2015 and the balance sheet data as of March 31, 2016 from our unaudited interim condensed financial statements. We have prepared the unaudited interim condensed financial statements on the same basis as our audited financial statements and, in the opinion of management, they reflect all adjustments, consisting only of normal recurring adjustments, that are necessary for the fair statement of our unaudited interim condensed financial statements. Our historical results are not necessarily indicative of the results that may be expected in the future, and our interim results are not necessarily indicative of the results to be expected for the full year or any other period. You should read this data together with the section titled Selected Quarterly Financial Information (unaudited) in our Annual Report on Form 10-K for the year ended December 31, 2015, the sections titled Management s Discussion and Analysis of Financial Condition and Results of Operations in our Annual Report on Form 10-K for the year ended December 31, 2015 and our Quarterly Report on Form 10-Q for the quarter ended March 31, 2016, and our financial statements and related notes included therein, each of which is incorporated by reference in this prospectus.

	Year Ended December 31,			Three Months Ended March 31,	
	2015	2014	2013	2016	2015
				(unaudited)	
	(in thousands, except share and per share data)				
Summary of Operations Data:					
Operating expenses:					
Research and development	$ 36,657	$ 16,324	$ 12,855	$ 12,415	$ 6,069
General and administrative	9,671	3,855	2,309	4,302	1,298
Related party expenses	65	332	499		53
Total operating expenses	46,393	20,511	15,663	16,717	7,420
Loss from operations	(46,393)	(20,511)	(15,663)	(16,717)	(7,420)
Change in fair value of Series A redeemable convertible preferred stock liability		(297)	(2,455)		
Interest income	33	1	2	117	3
Net loss	$ (46,360)	$ (20,807)	$ (18,116)	$ (16,600)	$ (7,417)
Net loss attributable to common stockholders	$ (50,540)	$ (23,772)	$ (19,851)	$ (16,600)	$ (8,657)
Net loss per share attributable to common stockholders, basic and diluted [1]	$ (3.95)	$ (14.20)	$ (16.14)	$ (0.56)	$ (4.22)
	12,806,697	1,673,919	1,230,241	29,441,404	2,052,874

Weighted-average number of shares used in computing net loss per share attributable to common stockholders, basic and diluted [(1)]	

(1) See Notes 2 and 13 to our audited financial statements and Notes 2 and 7 to our unaudited interim condensed financial statements incorporated by reference in this prospectus from our Annual Report on Form 10-K for the year ended December 31, 2015 and our Quarterly Report on Form 10-Q for the quarter ended March 31, 2016, respectively, for an explanation of the calculations of our basic and diluted net loss per share attributable to common stockholders and the weighted-average number of shares used in the computation of the per share amounts.

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	As of March 31, 2016	
	Actual	As Adjusted [1]
	(unaudited) (in thousands)	
Balance Sheet Data:		
Cash and cash equivalents	$ 133,984	$ 246,234
Working capital	125,043	237,293
Total assets	138,971	251,221
Additional paid-in capital	241,157	353,401
Accumulated deficit	(115,065)	(115,065)
Total stockholders equity	126,122	238,372

(1) The as adjusted column reflects the sale by us of 6,400,000 shares of our common stock in this offering at the public offering price of $18.75 per share, after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

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RISK FACTORS

Investing in our common stock involves a high degree of risk. You should consider carefully the following risks, together with all the other information included and incorporated by reference in this prospectus, including our financial statements and notes thereto, before you invest in our common stock. If any of the following risks actually materializes, our operating results, financial condition and liquidity could be materially adversely affected. As a result, the trading price of our common stock could decline and you could lose all or part of your investment.

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 years ended December 31, 2015, 2014 and 2013 were $46.4 million, $20.8 million and $18.1 million, respectively. 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

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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 and other product candidates that we may identify and pursue in clinical trials. As of March 31, 2016, we had working capital of $125.0 million and capital resources consisting of cash and cash equivalents of $134.0 million. 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 initial public offering, or 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;

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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 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;

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- 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 ad