



GBT Reports Recent Business Progress and Fourth Quarter and Full Year 2019 Financial Results

February 26, 2020

Obtained FDA approval of Oxbryta[®] (voxelotor), the first treatment that specifically targets the root cause of sickle cell disease (SCD), three months ahead of schedule

Made Oxbryta available to adults and children 12 years of age and older with SCD within days of FDA approval

Maintained strong balance sheet with \$695 million in cash as of year-end 2019

Conference Call today at 1:30 p.m. PT / 4:30 p.m. ET

SOUTH SAN FRANCISCO, Calif., Feb. 26, 2020 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) today reported recent business progress and financial results for the fourth quarter and year ended December 31, 2019.

"2019 was a momentous year for both the sickle cell community and GBT as we saw the FDA approval of Oxbryta, the first medicine that directly inhibits sickle hemoglobin polymerization, the root cause of this devastating, lifelong genetic disease, three months ahead of schedule," said Ted W. Love, M.D., president and chief executive officer of GBT. "With the early investment we made in building out our commercial infrastructure, we were able to make Oxbryta available to patients and physicians within days of its approval. Patient access to this needed therapy is a top priority for us. We will continue to work closely with payers throughout 2020 to meet our goal of obtaining broad coverage by the end of the year. Most importantly, we are encouraged by the positive feedback we have received to date from physicians and patients who have started Oxbryta therapy. We are optimistic about achieving our vision of making sickle cell disease a well-managed chronic condition by establishing Oxbryta as a standard of care, expanding its approved label and availability around the world, and continuing to research new pathways and develop innovative new therapies."

Recent Business Progress

Commercial

- Received U.S. Food and Drug Administration (FDA) approval of Oxbryta for the treatment of SCD in adults and children 12 years of age and older. The accelerated approval came three months before the Prescription Drug User Fee Act (PDUFA) target action date of February 26, 2020.
- On the day of FDA approval, launched GBT Source™, a comprehensive Oxbryta support program for patients and physicians. GBT Source provides a wide range of practical, educational and financial support customized to each patient's needs and eligibility. GBT Source also provides educational and support resources for physician practices and streamlined administrative processes for payers.
- Made Oxbryta available to patients and physicians through GBT's specialty pharmacy partner network within days after approval.
- Began accepting patient enrollments into GBT Source and facilitating reimbursement coverage, primarily through medical exceptions, within days after approval.
- Completed the build-out and launch training of the company's commercial organization.
- On track to complete most payer meetings to discuss Oxbryta's approved label and price during the first half of 2020, to meet the company's goal of obtaining broad coverage by the end of the year.

Clinical

- Initiated the HOPE-KIDS 2 Study, a post-approval confirmatory study using transcranial Doppler (TCD) flow velocity to assess the ability of Oxbryta to decrease the risk of stroke in children 2 to 15 years of age.
- Initiated a dose-optimization study designed to explore the potential safety and tolerability of Oxbryta at doses higher than 1500 mg.
- Presented eight abstracts at the 61st American Society of Hematology (ASH) Annual Meeting & Exposition. Key abstracts included:
 - Three post-hoc analyses of the Phase 3 HOPE Study that provide greater insight into the safety and efficacy of Oxbryta for treating SCD:
 - Oxbryta safely raised hemoglobin without evidence of viscosity-related complications. Rates of vaso-occlusive crises (VOCs) were inversely related to attained hemoglobin levels, and there was no evidence of an increased risk of VOC within 28 days of discontinuing treatment with Oxbryta as compared to placebo.
 - Oxbryta-treated patients with >1 g/dL increase in hemoglobin showed the greatest reduction in markers of hemolysis, consistent with the mechanism of inhibition of sickle hemoglobin polymerization.
 - Hemoglobin increases were comparable in Oxbryta-treated patients regardless of concomitant hydroxyurea use, providing reassurance that the increases seen with Oxbryta were not due to increased hydroxyurea

compliance during the study.

- A longitudinal analysis of children treated with hydroxyurea showed that a therapeutic rise in hemoglobin levels was significantly associated with a reduction in TCD levels.
- An analysis demonstrating the economic burden of end organ damage among U.S. patients with SCD, with predicted mean annual costs as high as \$286,000 for patients in the first year after a stroke.

Corporate

- Entered into a collaboration with Syros Pharmaceuticals, Inc., to discover, develop and commercialize novel therapies for SCD and beta thalassemia. Syros will use its leading gene control platform to identify therapeutic targets and discover small-molecule drugs that potentially induce fetal hemoglobin. GBT has the option to obtain an exclusive worldwide license to develop, manufacture and commercialize therapies resulting from the collaboration.
- Entered into a \$150 million loan agreement with funds managed by Pharmakon Advisors LP, and drew down on the first \$75 million of the non-dilutive loan. The proceeds will be used to advance the discovery and development of potential novel treatments for SCD and other grievous blood-based conditions without diverting financial resources from the launch of Oxbritya. GBT believes that the proceeds from this loan, in conjunction with existing cash and investments, have the potential to provide the necessary runway for the company to achieve positive cash flow while enabling the continued advancement of clinical development programs and other earlier-stage product candidates.
- Strengthened the company's leadership team with the appointment of Steven Immergut as senior vice president, head of corporate communications.

Financial Results for the Fourth Quarter and Year-End 2019

Total product sales, net for the fourth quarter of 2019 was \$2.1 million, driven by initial sales of Oxbritya. The company did not generate product sales in the fourth quarter of 2018.

Cost of sales for the three months ended December 31, 2019 was \$48,000. Manufacturing costs incurred prior to FDA approval of Oxbritya in November 2019 have been recorded as research and development expense in the company's consolidated statement of operations. The company expects that the cost of Oxbritya sales as a percentage of revenue will increase in future periods as product manufactured prior to FDA approval, and therefore fully expensed, is utilized. The company did not incur cost of sales for Oxbritya in 2018 as no product sales were generated.

Research and development (R&D) expenses for the three months ended December 31, 2019, were \$65.0 million compared with \$36.8 million for the same period in 2018. The increase in R&D expenses for this comparative period was primarily attributable to increased costs related to the company's SCD program for Oxbritya, increased employee-related costs, including non-cash stock compensation expense, increased costs related to preclinical research and manufacturing activities for inlacumab, and a \$20 million upfront payment incurred in December 2019 related to the company's Syros collaboration agreement. R&D expenses for the year ended December 31, 2019, were \$174.6 million compared with \$131.3 million for the same period in 2018. The increase in expenses for the full year were largely driven by the same factors as noted above for the fourth quarter. Total R&D non-cash stock compensation expense incurred for the three months ended December 31, 2019, was \$5.3 million compared with \$3.2 million for the same period in 2018. Total R&D non-cash stock compensation expense incurred for the year ended December 31, 2019, was \$19.1 million compared with \$12.7 million for the same period in 2018.

Sales, general and administrative (SG&A) expenses for the three months ended December 31, 2019, were \$44.6 million compared with \$15.3 million for the same period in 2018. SG&A expenses for the year ended December 31, 2019, were \$117.1 million compared with \$51.4 million for the same period in 2018. The increase in SG&A expenses for both comparative periods is primarily attributable to increased employee-related costs, including non-cash stock compensation expense, and increased professional and consulting services associated with the build-out of the company's commercial operations and launch of Oxbritya. Total SG&A non-cash stock compensation expense incurred in the three months ended December 31, 2019, was \$7.5 million compared with \$4.4 million for the same period in 2018. Total SG&A non-cash stock compensation expense incurred in the year ended December 31, 2019, was \$26.5 million compared with \$17.3 million for the same period in 2018.

A non-cash gain on the company's lease modification for the three months ended December 31, 2019, was \$8.3 million. This is a non-recurring item related to the upcoming move to the company's new location and related termination of its existing lease.

Net loss for the three months ended December 31, 2019, was \$96.0 million compared with \$49.2 million for the same period in 2018. Basic and diluted net loss per share for the three months ended December 31, 2019, was \$1.59 compared with \$0.93 for the same period in 2018. Net loss for the year ended December 31, 2019, was \$266.8 million compared with \$174.2 million for the same period in 2018. Basic and diluted net loss per share for the year ended December 31, 2019, was \$4.57 compared with \$3.41 for the same period in 2018. The company expects its operating costs to increase during 2020 due to hiring that occurred in the fourth quarter as well as costs associated with commercialization activities.

Cash, cash equivalents and marketable securities totaled \$695.0 million at December 31, 2019, compared with \$591.8 million at December 31, 2018.

Conference Call Details

GBT will host a conference call and webcast today, Wednesday, February 26, 2020, at 4:30 p.m. ET (1:30 p.m. PT), during which time management will provide a general business update and discuss the financial results for the quarter and year ended December 31, 2019. To participate in the conference call, please dial 877-407-3982 (domestic) or 201-493-6780 (international). A live audio webcast of the conference call can be accessed on GBT's website at www.gbt.com under the Investors section. An archived audio webcast will be available for one month following the event.

About Sickle Cell Disease

Sickle cell disease (SCD) affects an estimated 100,000 people in the United States and millions of people throughout the world, particularly among those whose ancestors are from sub-Saharan Africa. It also affects people of Hispanic, South Asian, Southern European and Middle Eastern ancestry.¹ SCD is a lifelong inherited blood disorder that impacts hemoglobin, a protein carried by red blood cells that delivers oxygen to tissues and organs throughout the body.² Due to a genetic mutation, people with SCD form abnormal hemoglobin known as sickle hemoglobin. Through a process

called hemoglobin polymerization, red blood cells become sickled – deoxygenated, crescent-shaped and rigid.²⁻⁴ The sickling process causes hemolytic anemia (low hemoglobin due to red blood cell destruction) and blockages in capillaries and small blood vessels, which impede the flow of blood and oxygen throughout the body. The diminished oxygen delivery to tissues and organs can lead to life-threatening complications, including stroke and irreversible organ damage.⁴⁻⁶

About Oxbryta® (voxelotor) tablets

Oxbryta (voxelotor), previously called GBT440, is an oral, once-daily therapy for patients with sickle cell disease (SCD). Oxbryta works by increasing hemoglobin's affinity for oxygen. Since oxygenated sickle hemoglobin does not polymerize, GBT believes Oxbryta blocks polymerization and the resultant sickling and destruction of red blood cells. With the potential to improve hemolytic anemia and oxygen delivery, GBT believes that Oxbryta has the potential to modify the course of SCD. On November 25, 2019, Oxbryta received U.S. Food and Drug Administration (FDA) accelerated approval for the treatment of SCD in adults and children 12 years of age and older.⁷ As a condition of accelerated approval, GBT will continue to study voxelotor in the HOPE-KIDS 2 Study, a post-approval confirmatory study using transcranial Doppler (TCD) flow velocity to assess the ability of Oxbryta to decrease stroke risk in children 2 to 15 years of age.

In recognition of the critical need for new SCD treatments, the FDA granted Oxbryta Breakthrough Therapy, Fast Track, Orphan Drug and Rare Pediatric Disease designations for the treatment of patients with SCD. The European Medicines Agency (EMA) has included voxelotor in its Priority Medicines (PRIME) program, and the European Commission (EC) has designated voxelotor as an orphan medicinal product for the treatment of patients with SCD.

Indication

Oxbryta is a prescription medicine used for the treatment of sickle cell disease in adults and children 12 years of age and older. It is not known if Oxbryta is safe and effective in children below 12 years of age.

This indication is approved under accelerated approval based on increase in hemoglobin (Hb). Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trial(s).

Important Safety Information

Oxbryta should not be taken if the patient has had an allergic reaction to voxelotor or any of the ingredients in Oxbryta. See the end of the patient leaflet for a list of the ingredients in Oxbryta.

Oxbryta can cause serious side effects, including serious allergic reactions. Patients should tell their healthcare provider or get emergency medical help right away if they get rash, hives, shortness of breath or swelling of the face.

Patients receiving exchange transfusions should talk to their healthcare provider about possible difficulties with the interpretation of certain blood tests when taking Oxbryta.

The most common side effects of Oxbryta include headache, diarrhea, stomach (abdominal) pain, nausea, tiredness, rash and fever. These are not all the possible side effects of Oxbryta.

Before taking Oxbryta, patients should tell their healthcare provider about all medical conditions, including if they have liver problems; if they are pregnant or plan to become pregnant as it is not known if Oxbryta can harm an unborn baby; or if they are breastfeeding or plan to breastfeed as it is not known if Oxbryta can pass into breastmilk or if it can harm a baby. Patients should not breastfeed during treatment with Oxbryta and for at least 2 weeks after the last dose.

Patients should tell their healthcare provider about all the medicines they take, including prescription and over-the-counter medicines, vitamins and herbal supplements. Some medicines may affect how Oxbryta works. Oxbryta may also affect how other medicines work.

Patients are advised to call their doctor for medical advice about side effects. Side effects can be reported to FDA at 1-800-FDA-1088. Side effects can also be reported to Global Blood Therapeutics at 1-833-428-4968 (1-833-GBT-4YOU).

Full Prescribing Information for Oxbryta is available at Oxbryta.com.

About Global Blood Therapeutics

Global Blood Therapeutics (GBT) is a biopharmaceutical company dedicated to the discovery, development and delivery of life-changing treatments that provide hope to underserved patient communities. Founded in 2011, GBT is delivering on its goal to transform the treatment and care of sickle cell disease (SCD), a lifelong, devastating inherited blood disorder. The company has introduced Oxbryta® (voxelotor), the first FDA-approved treatment that directly inhibits sickle hemoglobin polymerization, the root cause of SCD. GBT is also advancing its pipeline program in SCD with inclacumab, a p-selectin inhibitor in development to address pain crises associated with the disease. In addition, GBT's drug discovery teams are working on new targets to develop the next generation of treatments for SCD. To learn more, please visit www.gbt.com and follow the company on Twitter [@GBT_news](https://twitter.com/GBT_news).

Forward-Looking Statements

Certain statements in this press release are forward-looking within the meaning of the Private Securities Litigation Reform Act of 1995, including statements containing the words "will," "anticipates," "plans," "believes," "forecast," "estimates," "expects" and "intends," or similar expressions. These forward-looking statements are based on GBT's current expectations and actual results could differ materially. Statements in this press release may include statements that are not historical facts and are considered forward-looking 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. GBT intends these forward-looking statements, including statements regarding GBT's priorities, goals and vision, the significance of 2019 for the sickle cell community and GBT, the safety, efficacy and mechanism of action of Oxbryta and other product characteristics, the availability, use, commercialization and commercial and medical potential of Oxbryta, the need for Oxbryta and other SCD treatments, making SCD a well-managed condition, establishing Oxbryta as a standard of care, expanding its approved label and availability around the world, and researching new pathways and developing new therapies, working and meeting with payers and obtaining reimbursement for Oxbryta, transforming the treatment and care of SCD and establishing GBT as a leader in addressing blood disorders, ongoing studies of Oxbryta and related protocols, activities and expectations, GBT's collaboration with Syros and related rights, obligations, activities and expectations, the Pharmakon loan, including its significance, the use of its proceeds, the impact on GBT's use of its financial resources, and the

availability of additional funds under the loan, GBT's financial position, outlook and expectations, including its financial runway and potential to achieve positive cash flow while continuing research and development, and advancing GBT's pipeline and discovering, developing and delivering innovative treatments, to be covered by the safe harbor provisions for forward-looking statements contained in Section 27A of the Securities Act and Section 21E of the Securities Exchange Act, and GBT makes this statement for purposes of complying with those safe harbor provisions. These forward-looking statements reflect GBT's current views about its plans, intentions, expectations, strategies and prospects, which are based on the information currently available to the company and on assumptions the company has made. GBT can give no assurance that the plans, intentions, expectations or strategies will be attained or achieved, and, furthermore, actual results may differ materially from those described in the forward-looking statements and will be affected by a variety of risks and factors that are beyond GBT's control including, without limitation, compliance with the funding and other obligations under the Pharmakon loan, the timing and progress of GBT's and Syros' research and development activities under their collaboration, the amount and timing of resources devoted by each of such parties to activities under the collaboration, the risks that GBT has only recently established its commercialization capabilities and may not be able to successfully commercialize Oxbryta, risks associated with GBT's dependence on third parties for development, manufacture and commercialization activities related to Oxbryta, government and third-party payor actions, including those relating to reimbursement and pricing, risks and uncertainties relating to competitive products and other changes that may limit demand for Oxbryta, the risks regulatory authorities may require additional studies or data to support continued commercialization of Oxbryta, the risks that drug-related adverse events may be observed during commercialization or clinical development, and data and results may not meet regulatory requirements or otherwise be sufficient for further development, regulatory review or approval, along with those risks set forth in GBT's Annual Report on Form 10-K for the fiscal year ended December 31, 2018, and in GBT's most recent Quarterly Report on Form 10-Q filed with the U.S. Securities and Exchange Commission, as well as discussions of potential risks, uncertainties and other important factors in GBT's subsequent filings with the U.S. Securities and Exchange Commission. Except as required by law, GBT assumes no obligation to update publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

References

1. Centers for Disease Control and Prevention website. Sickle Cell Disease (SCD). <https://www.cdc.gov/ncbddd/sicklecell/data.html>. Accessed June 3, 2019.
2. National Heart, Lung, and Blood Institute website. Sickle Cell Disease. <https://www.nhlbi.nih.gov/health-topics/sickle-cell-disease>. Accessed August 5, 2019.
3. Rees DC, et al. *Lancet*. 2010;376(9757):2018-2031.
4. Kato GJ, et al. *Nat Rev Dis Primers*. 2018;4:18010.
5. Kato GJ, et al. *J Clin Invest*. 2017;127(3):750-760.
6. Caboot JB, et al. *Paediatr Respir Rev*. 2014;15(1):17-23.
7. Oxbryta (voxelotor) tablets prescribing information. South San Francisco, Calif. Global Blood Therapeutics, Inc.; November 2019.

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

	Three Months Ended December		Year Ended December 31,	
	31,		31,	
	2019	2018	2019	2018
	(Unaudited)	(Unaudited)	(Unaudited)	
Product sales, net	\$ 2,108	\$ —	\$ 2,108	\$ —
Costs and operating expenses:				
Cost of sales	48	—	48	—
Research and development	64,990	36,765	174,556	131,307
Selling, general and administrative	44,585	15,319	117,088	51,435
Gain on lease modification	(8,301)	—	(8,301)	—
Total costs and operating expenses	101,322	52,084	283,391	182,742
Loss from operations	(99,214)	(52,084)	(281,283)	(182,742)
Other income (expense):				
Interest income, net	3,275	2,850	14,697	8,618
Other income (expenses), net	(36)	33	(180)	(69)
Total other income, net	3,239	2,883	14,517	8,549
Net loss	\$ (95,975)	\$ (49,201)	\$ (266,766)	\$ (174,193)
Basic and diluted net loss per common share	\$ (1.59)	\$ (0.93)	\$ (4.57)	\$ (3.41)
Weighted-average number of shares used in computing basic and diluted net loss per common share	60,352,124	52,972,225	58,321,612	51,150,728

GLOBAL BLOOD THERAPEUTICS, INC.
Condensed Consolidated Balance Sheets
(In thousands)

	<u>December 31, 2019</u>	<u>December 31, 2018</u>
	(Unaudited)	
Assets		
Current assets:		
Cash and cash equivalents	\$ 302,237	\$ 275,357
Short-term marketable securities	307,732	202,177
Other current assets	18,028	8,246
Total current assets	<u>627,997</u>	<u>485,780</u>
Property and equipment, net	27,113	14,981
Long-term marketable securities	85,030	114,281
Operating lease right-of-use assets	52,775	—
Other assets	3,184	2,601
Total assets	<u>\$ 796,099</u>	<u>\$ 617,643</u>
Liabilities and Stockholders' Equity		
Current liabilities	\$ 71,453	\$ 33,773
Long-term debt	73,559	—
Operating lease liabilities, noncurrent	72,359	—
Other noncurrent liabilities	34	11,071
Total liabilities	<u>217,405</u>	<u>44,844</u>
Total stockholders' equity	<u>578,694</u>	<u>572,799</u>
Total liabilities and stockholders' equity	<u>\$ 796,099</u>	<u>\$ 617,643</u>

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Source: Global Blood Therapeutics, Inc.