



GBT Reports Recent Business Progress and Fourth Quarter and Full Year 2020 Financial Results

February 24, 2021

Achieved Oxbryta[®] (voxelotor) net revenues of \$41.3 million in the fourth quarter and \$123.8 million for full year 2020

Further established leadership in sickle cell disease (SCD) with data presentations on pipeline programs inclacumab and GBT021601 (GBT601)

Conference call today at 4:30 p.m. ET

SOUTH SAN FRANCISCO, Calif., Feb. 24, 2021 (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, 2020.

"In 2020, GBT adapted to the challenging conditions of the global COVID-19 pandemic to safely and successfully execute the launch of Oxbryta. It is a testament to our team's dedication and the need for innovative therapies for sickle cell disease that we ended the year with thousands of patients on Oxbryta, an established base of prescribers, broad payer coverage, and a growing body of real-world evidence demonstrating statistically significant decreases in VOCs and transfusions and improved overall health-status. In addition, we announced robust progress for our pipeline with plans to advance inclacumab and GBT601 in 2021. We believe both these programs have the potential to become best-in-class SCD therapies, further expanding our leadership in SCD," said Ted W. Love, M.D., president and CEO of GBT.

"We continue to be confident in the long-term potential of Oxbryta and are well positioned to build on our strong fundamentals to enhance our commercialization efforts in the U.S. and accelerate adoption. Furthermore, we continue to make important progress on our goal to provide access to Oxbryta to more SCD patients. We will also continue to support the SCD community and efforts to improve access to care, which has become even more important because of the impact of the COVID-19 pandemic that has dramatically magnified long-standing health inequities," added Dr. Love.

Recent Business Progress

Commercial

- Achieved Oxbryta[®] (voxelotor) tablets net sales of \$41.3 million and \$123.8 million in the three and twelve months ended December 31, 2020, respectively.
- Recorded approximately 950 new prescriptions of Oxbryta in the fourth quarter of 2020, despite a dramatic increase in COVID-19 cases in the U.S. during this period.
- New prescriptions in the fourth quarter of 2020 reflect ongoing increases in the use of telemedicine by healthcare providers, partially offset by fewer healthcare provider and patient interactions due to the increase in COVID-19 cases in the United States, and the Thanksgiving and Christmas holidays. GBT continues to believe that when the pandemic subsides, the number of new prescriptions will further improve and surpass pre-COVID-19 levels over time.
- Continued to secure Oxbryta reimbursement coverage, with more than 90% of lives covered by payers either through published policies or verified patient adjudication by the end of the fourth quarter. GBT has secured fee-for-service Medicaid coverage in 47 states, including all 17 priority states where most SCD patients live.
- In February 2021, GBT launched patient starter kits and new sales materials in the U.S. to provide deeper education on Oxbryta and launched www.gbtsource.com, which provides information for patients, caregivers and health professionals on GBT Source Solutions[®] -- the company's dedicated SCD patient support program that helps eligible patients start and stay on Oxbryta.

Clinical

- [Presented](#) nine posters at the American Society of Hematology (ASH) Annual Meeting & Exposition in December 2020, including the 72-week analysis of the completed Phase 3 HOPE Study and data on real-world experience with Oxbryta. The ASH presentations also included preclinical data on the company's promising SCD pipeline, including inclacumab, a P-selectin inhibitor with potential to deliver more potency and once-quarterly dosing for a best-in-class profile, and GBT601, a next generation hemoglobin S polymerization inhibitor with increased potency and the potential to provide a functional cure for SCD.
- Announced plans to initiate two global, randomized, placebo-controlled pivotal Phase 3 trials evaluating the safety and efficacy of inclacumab in the first half of 2021. One trial will focus on the reduction in VOCs and the second will focus on reducing 90-day hospital readmission following an initial VOC hospitalization.
- In December 2020, initiated a first-in-human Phase 1 study on the safety and tolerability of GBT601 in healthy volunteers. In 2021, GBT plans to begin studying GBT601 in SCD patients, with a goal of providing proof-of-concept data by the end of the year.
- In January 2021, a post hoc analysis that evaluated the incidence of leg ulcers and outcomes in patients enrolled in the HOPE trial across the 72-week treatment period was published in the [American Journal of Hematology](#). The study findings add to the growing body of evidence of the potential clinical benefits of Oxbryta.

- In January 2021, [enrolled](#) the first patient in an expanded access protocol (EAP) in the U.S. for Oxbryta in pediatric patients with SCD. The EAP provides access for children ages 4 to 11 years who have no alternative treatment options and are ineligible to participate in clinical trials of Oxbryta.
- [Initiated](#) an early access program for voxelotor in Europe and other regions outside the United States in December 2020, allowing physicians in countries with an early access regulatory and legal pathway the option to request voxelotor for eligible SCD patients who do not have access to the medicine as part of a clinical trial.

Corporate

- Strengthened the company's senior leadership team with the appointment of [Kim Smith-Whitley, M.D.](#), as executive vice president and head of research and development. Dr. Smith-Whitley is a board-certified pediatric hematologist and clinical director of hematology and director of the Comprehensive Sickle Cell Center at Children's Hospital of Philadelphia. Dr. Smith-Whitley will officially join GBT in May 2021, and in the interim, is serving in an advisory capacity.
- In January 2021, GBT [announced](#) that the European Medicines Agency (EMA) completed the validation of the company's Marketing Authorization Application (MAA) for Oxbryta and started its standard review process. GBT is seeking full marketing approval for Oxbryta from the EMA to treat hemolytic anemia in patients with SCD 12 years of age and older.
- GBT was included in the [2021 Bloomberg Gender-Equality Index](#) (GEI). The GEI measures gender equality across five pillars: female leadership and talent pipeline, equal pay and gender pay parity, inclusive culture, sexual harassment policies, and pro-women brand. GBT was one of only 380 companies across 11 sectors, including one of 22 in healthcare, that were included in this index.
- Supported the launch of Life Science Cares (LSC) Bay Area, a nonprofit that empowers corporations within the Life Sciences Industry to help end poverty. Dr. Love is a co-founder of LSC Bay Area and serves as a member of its Board of Managers.

Financial Results for the Fourth Quarter and Year-End 2020

Total net product sales for the fourth quarter of 2020 was \$41.3 million, resulting from sales of Oxbryta, compared with \$2.1 million for the same period in 2019. Net product sales resulted from sales of Oxbryta, which was launched in the United States in December 2019. Total net product sales for the full year 2020 was \$123.8 million, compared with \$2.1 million for the same period in 2019.

Cost of sales for the three months ended December 31, 2020, was \$1.0 million compared with \$48,000 for the same period in 2019. Total cost of sales for the full year 2020 was \$2.0 million, compared with \$48,000 for the same period in 2019. Manufacturing costs incurred prior to FDA approval of Oxbryta in November 2019 were previously recorded as research and development expense in the company's consolidated statement of operations. GBT expects that the cost of Oxbryta sales as a percentage of revenue will increase in future periods as product manufactured prior to FDA approval, and therefore fully expensed, is utilized.

Research and development (R&D) expenses for the three months ended December 31, 2020, were \$41.1 million compared with \$65.0 million for the same period in 2019. R&D expense in the fourth quarter 2019 included a \$20 million upfront expense incurred in December 2019 related to the company's Syros collaboration agreement. Excluding this expense, the decrease in R&D expense compared to the prior year was primarily due to lower expense as a result of capitalization of Oxbryta manufacturing costs to inventory, which was partially offset by higher pre-clinical development activities under the Syros collaboration and other clinical programs. Following FDA approval of Oxbryta in November 2019, GBT capitalizes manufacturing of Oxbryta to inventory. R&D expenses for the year ended December 31, 2020, were \$155.1 million compared with \$174.6 million for the same period in 2019. The year-over-year decrease was primarily due to a \$33.9 million decrease in Oxbryta related manufacturing costs and medical affairs costs that were previously expensed to R&D and a \$1.7 million decrease in other preclinical programs. The decrease was partially offset by a \$16.3 million increase in external costs associated with inlacumab and driven by the manufacturing activities. Total R&D non-cash stock compensation expense incurred for the three months ended December 31, 2020, was \$3.7 million compared with \$5.3 million for the same period in 2019. Total R&D non-cash stock compensation expense incurred for the year ended December 31, 2020, was \$18.1 million compared with \$19.1 million for the same period in 2019.

Selling, general and administrative (SG&A) expenses for the three months ended December 31, 2020, were \$58.6 million compared with \$44.6 million for the same period in 2019. SG&A expenses for the year ended December 31, 2020, were \$210.9 million compared with \$117.1 million for the same period in 2019. The increase in SG&A expenses for both comparative periods was 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 Oxbryta. Total SG&A non-cash stock compensation expense incurred in the three months ended December 31, 2020, was \$14.3 million compared with \$7.5 million for the same period in 2019. Total SG&A non-cash stock compensation expense incurred in the year ended December 31, 2020, was \$53.4 million compared with \$26.5 million for the same period in 2019.

A non-cash gain on the company's lease modification for the year ended December 31, 2020, was \$1.0 million compared with \$8.3 million for the same period in 2019. This is a non-recurring item related to the move to the company's new headquarters in 2020 and related termination of its previous lease in 2019.

Net loss for the three months ended December 31, 2020, was \$61.8 million compared with \$96.0 million for the same period in 2019. Basic and diluted net loss per share for the three months ended December 31, 2020, was \$1.00 compared with \$1.59 for the same period in 2019. Net loss for the year ended December 31, 2020, was \$247.6 million compared with \$266.8 million for the same period in 2019. Basic and diluted net loss per share for the year ended December 31, 2020, was \$4.04 compared with \$4.57 for the same period in 2019. The company expects its operating costs to increase during 2021 due to costs associated with expanding commercialization activities related to Oxbryta and the advancement of its clinical pipeline.

Cash, cash equivalents, and marketable securities totaled \$560.9 million at December 31, 2020, compared with \$695.0 million at December 31, 2019.

Conference Call Details

GBT will host a conference call today, Wednesday, February 24, 2021, at 4:30 p.m. ET to provide a general business update and discuss the financial

results for the fourth quarter and full year 2020. 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,¹ an estimated 52,000 people in Europe,² and millions of people throughout the world, particularly among those whose ancestors are from sub-Saharan Africa.¹ It also affects people of Hispanic, South Asian, Southern European, and Middle Eastern ancestry.¹ SCD is a lifelong inherited rare blood disorder that impacts hemoglobin, a protein carried by red blood cells that delivers oxygen to tissues and organs throughout the body.³ Due to a genetic mutation, individuals 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.³⁻⁵ 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.⁴⁻⁷

About Oxbrya® (voxelotor) tablets

Oxbrya (voxelotor) is an oral, once-daily therapy for patients with sickle cell disease (SCD). Oxbrya works by increasing hemoglobin's affinity for oxygen. Since oxygenated sickle hemoglobin does not polymerize, GBT believes Oxbrya blocks polymerization and the resultant sickling and destruction of red blood cells, which are primary pathologies faced by every single person living with SCD. With the potential to improve hemolytic anemia and oxygen delivery, GBT believes that Oxbrya has the potential to modify the course of SCD. On November 25, 2019, Oxbrya 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.⁸ As a condition of accelerated approval, GBT will continue to study Oxbrya in the HOPE-KIDS 2 Study, a post-approval confirmatory study using transcranial Doppler (TCD) flow velocity to assess the ability of the therapy 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 Oxbrya Breakthrough Therapy, Fast Track, Orphan Drug, and Rare Pediatric Disease designations for the treatment of patients with SCD. Additionally, Oxbrya has been granted Priority Medicines (PRIME) designation from the European Medicines Agency (EMA), and the European Commission (EC) has designated Oxbrya as an orphan medicinal product for the treatment of patients with SCD.

The EMA has accepted for review GBT's MAA seeking full marketing authorization of Oxbrya in the European Union to treat hemolytic anemia in SCD patients ages 12 years and older. GBT also plans to seek regulatory approval to expand the potential use of Oxbrya in the United States for the treatment of SCD in children as young as 4 years old.

Important Safety Information

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

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

Before taking Oxbrya, 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 Oxbrya can harm an unborn baby; or if they are breastfeeding or plan to breastfeed as it is not known if Oxbrya can pass into breastmilk or if it can harm a baby. Patients should not breastfeed during treatment with Oxbrya 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 Oxbrya works. Oxbrya 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 Oxbrya is available at Oxbrya.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 Oxbrya® (voxelotor), the first FDA-approved treatment that directly inhibits sickle hemoglobin polymerization, the root cause of red blood cell sickling in 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, and GBT021601 (GBT601), the company's next generation hemoglobin S polymerization inhibitor. In addition, GBT's drug discovery teams are working on new targets to develop the next wave 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, commitment, dedication, focus, goals, and vision; the safety, efficacy, and mechanism of action of Oxbrya, and other product characteristics; the commercialization, delivery, availability, use, adoption, and commercial and medical potential of Oxbrya; building on

fundamentals and enhancing commercialization efforts; use of telemedicine and provider and patient interaction; payer coverage for Oxbryta; ongoing and planned studies and related protocols, activities, and expectations; GBT's financial position, outlook, guidance, and expectations; the COVID-19 pandemic and related expectations; the expanded access protocol and early access program for Oxbryta, including the potential availability, use and impact; providing access to Oxbryta to more patients; regulatory submissions, review and approval to potentially expand the approved use of Oxbryta for more patients in the U.S. and to treat patients in Europe; supporting the SCD community and impacting the treatment, care and course of SCD; expanding GBT's leadership in SCD; the potential and advancement of GBT's pipeline, including inclacumab and other product candidates; working on new targets, and discovering, developing, and delivering 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, risks and uncertainties relating to the COVID-19 pandemic, including the extent and duration of the impact on GBT's business, including commercialization activities, regulatory efforts, research and development, corporate development activities, and operating results, which will depend on future developments that are highly uncertain and cannot be accurately predicted, such as the ultimate duration of the pandemic, travel restrictions, quarantines, social distancing, and business closure requirements in the U.S. and in other countries, and the effectiveness of actions taken globally to contain and treat the disease; the risks that GBT is continuing to establish its commercialization capabilities and may not be able to successfully commercialize Oxbryta; risks associated with GBT's dependence on third parties for development, manufacture, distribution and commercialization activities related to Oxbryta; government and third-party payer 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; data and results may not meet regulatory requirements or otherwise be sufficient for further development, regulatory review, or approval; compliance with obligations under the Pharmakon loan; and the timing and progress of GBT's and Syros' research and development activities under their collaboration; along with those risks set forth in GBT's Annual Report on Form 10-K for the fiscal year ended December 31, 2019, 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

- Centers for Disease Control and Prevention website. Sickle Cell Disease (SCD). <https://www.cdc.gov/ncbddd/sicklecell/data.html>. Accessed June 3, 2019.
- European Medicines Agency. <https://www.ema.europa.eu/en/medicines/human/orphan-designations/eu3182125>. Accessed June 12, 2020.
- National Heart, Lung, and Blood Institute website. Sickle Cell Disease. <https://www.nhlbi.nih.gov/health-topics/sickle-cell-disease>. Accessed August 5, 2019.
- Rees DC, et al. Lancet. 2010;376(9757):2018-2031.
- Kato GJ, et al. Nat Rev Dis Primers. 2018;4:18010.
- Kato GJ, et al. J Clin Invest. 2017;127(3):750-760.
- Caboot JB, et al. Paediatr Respir Rev. 2014;15(1):17-23.
- 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 | | Year Ended December 31, | |
|-------------------------------------|--------------------|-------------|-------------------------|----------|
| | December 31, | | December 31, | |
| | 2020 | 2019 | 2020 | 2019 |
| | (Unaudited) | (Unaudited) | (Unaudited) | |
| Product sales, net | \$ 41,295 | \$ 2,108 | \$ 123,803 | \$ 2,108 |
| Costs and operating expenses: | | | | |
| Cost of sales | 960 | 48 | 1,986 | 48 |
| Research and development | 41,068 | 64,990 | 155,122 | 174,556 |
| Selling, general and administrative | 58,640 | 44,585 | 210,851 | 117,088 |
| Gain on lease modification | — | (8,301) | (984) | (8,301) |
| Total costs and operating expenses | 100,668 | 101,322 | 366,975 | 283,391 |

| | | | | |
|---|--------------------|--------------------|---------------------|---------------------|
| Loss from operations | (59,373) | (99,214) | (243,172) | (281,283) |
| Other income (expense): | | | | |
| Interest income (expense), net | (2,340) | 3,275 | (3,975) | 14,697 |
| Other income (expenses), net | (93) | (36) | (406) | (180) |
| Total other income (expense), net | (2,433) | 3,239 | (4,381) | 14,517 |
| Net loss | <u>\$ (61,806)</u> | <u>\$ (95,975)</u> | <u>\$ (247,553)</u> | <u>\$ (266,766)</u> |
| Basic and diluted net loss per common share | <u>\$ (1.00)</u> | <u>\$ (1.59)</u> | <u>\$ (4.04)</u> | <u>\$ (4.57)</u> |
| Weighted-average number of shares used in computing basic and diluted net loss per common share | <u>61,849,456</u> | 60,352,124 | 61,334,037 | 58,321,612 |

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

| | <u>December 31, 2020</u> | <u>December 31, 2019</u> |
|---|--------------------------|--------------------------|
| | (Unaudited) | |
| Assets | | |
| Current assets: | | |
| Cash and cash equivalents | \$ 494,766 | \$ 302,237 |
| Short-term marketable securities | 66,126 | 307,732 |
| Other current assets | 71,271 | 18,028 |
| Total current assets | 632,163 | 627,997 |
| Property and equipment, net | 37,882 | 27,113 |
| Long-term marketable securities | — | 85,030 |
| Operating lease right-of-use assets | 50,722 | 52,775 |
| Other assets | 3,235 | 3,184 |
| Total assets | <u>\$ 724,002</u> | <u>\$ 796,099</u> |
| Liabilities and Stockholders' Equity | | |
| Current liabilities | \$ 79,032 | \$ 71,453 |
| Long-term debt | 148,815 | 73,559 |
| Operating lease liabilities, noncurrent | 79,176 | 72,359 |
| Other noncurrent liabilities | 822 | 34 |
| Total liabilities | 307,845 | 217,405 |
| Total stockholders' equity | 416,157 | 578,694 |
| Total liabilities and stockholders' equity | <u>\$ 724,002</u> | <u>\$ 796,099</u> |

Contact:

Steven Immergut (media)
650.410.3258
simmergut@gbt.com

Courtney Roberts (investors)
650.351.7881
croberts@gbt.com



Source: Global Blood Therapeutics, Inc.