GBT Reports Recent Business Progress and Second Quarter 2020 Financial Results

August 5, 2020

Achieved Oxbryta® (voxelotor) net revenues of $31.5 million, with approximately 1,000 new patient prescriptions

Announced plans to seek approval of Oxbryta for younger pediatric patients in the United States and seek approval of Oxbryta in Europe

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

SOUTH SAN FRANCISCO, Calif., Aug. 05, 2020 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) today reported recent business progress and financial results for the second quarter ended June 30, 2020.

“In the second quarter of 2020, we continued to make important progress on GBT’s vision of being a leading biopharmaceutical company and the leader in sickle cell disease. Despite the impact of COVID-19, we continued to successfully execute the launch of Oxbryta and make significant progress on new prescriptions, new prescribers, and payer coverage. During the quarter, we were pleased to see increased use of virtual engagements and telemedicine by healthcare professionals and continued growth of Oxbryta prescriptions. In addition, we made significant progress in our efforts to expand the approved use of Oxbryta for more patients in the United States and in Europe, with regulatory submissions planned by mid-2021,” said Ted W. Love, M.D., president and CEO of GBT.

“Last quarter was also marked by a magnification of health disparities driven by COVID-19, which is having an outsized impact on African Americans, and a renewed focus on racial injustice sparked by deaths due to police brutality. The sickle cell disease community lives and breathes these issues, and GBT is working to address them, as evidenced by our cohort of ACCEL grant recipients. We are committed to enacting real and lasting change for communities of color, especially when it comes to ensuring patients have access to the care they need and deserve,” added Dr. Love.

Recent Business Progress

Commercial

- Achieved Oxbryta® (voxelotor) net sales of $31.5 million in the second quarter of 2020.
- Despite the impact of COVID-19, recorded approximately 1,000 new prescriptions of Oxbryta during the second quarter.
- The decrease in new prescriptions stabilized following an initial impact of COVID-19 at the end of the first quarter. The stabilization was primarily due to the increased use of telemedicine by healthcare providers and increased comfort with virtual engagements with GBT field teams. When the pandemic subsides, GBT expects that, over time, the number of new prescriptions will improve from current levels and surpass pre-COVID-19 levels.
- Conducted a review of patient charts, claims, and lab data that showed Oxbryta is being prescribed to a broad range of patients irrespective of hemoglobin (Hb) level, history of vaso-occlusive crisis (VOC), and current therapy for sickle cell disease (SCD). Almost half of patients started therapy with baseline Hb greater than 8 g/dL, and more than half take Oxbryta in combination with another SCD therapy.
- Continued to secure Oxbryta reimbursement coverage, with 53% of lives covered by payers either through published policies or medical exceptions. GBT has secured fee-for-service Medicaid coverage in 42 states, including all 17 priority states where most SCD patients live. GBT believes it is on track to meet its goal of obtaining broad coverage by the end of the year.

Clinical

- Collaborated with clinical trial sites to implement appropriate protocols that would, over time, enable the resumption of patient enrollment and participation in GBT-sponsored clinical trials. The company believes it remains on track with the long-term timelines for its clinical trials.
- Presented four abstracts at the virtual edition of the 25th Annual European Hematology Association Congress:
  - A retrospective analysis of data from the landmark STOP 2 study linking higher Hb levels to lower transcranial Doppler flow velocity, a predictor of stroke risk in children with SCD.
  - Three encore presentations of the pivotal Phase 3 HOPE Study that reinforced key attributes of Oxbryta, including that the lowest incidence of VOCs occurred in patients who achieved the highest Hb levels; that biomarkers of hemolysis decrease with increasing dose; and that the treatment effect seen with Oxbryta was independent of hydroxyurea use.

Corporate

- Announced plans to expand the potential use of Oxbryta for the treatment of SCD in children as young as 4 years old. GBT plans to submit a New Drug Application, which will include a new age-appropriate formulation, to the U.S. Food and Drug Administration by mid-2021.

- Announced plans to seek full marketing authorization in Europe for Oxbryta to treat hemolytic anemia in SCD patients ages 12 years and older. GBT plans to submit a Marketing Authorization Application to the European Medicines Agency by mid-2021.
- Awarded $250,000 in grants to five nonprofit organizations to advance access to care for people living with SCD through the company's Access to Excellent Care for Sickle Cell Patients (ACCEL) program. The ACCEL program was established by GBT in 2019 to help fund programs that have the potential to deliver high-quality healthcare to people living with SCD.

Financial Results for the Second Quarter 2020
Total net product sales for the second quarter of 2020 was $31.5 million, resulting from sales of Oxbryta. The company did not generate product sales in the second quarter of 2019.

Cost of sales for the three months ended June 30, 2020, was $0.4 million. 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. GBT did not incur cost of sales for Oxbryta in the second quarter of 2019 as no product sales were generated.

Research and development (R&D) expenses for the three months ended June 30, 2020, were $34.1 million compared with $36.0 million for the same period in 2019. The decrease in R&D expenses for this comparative period was primarily attributable to a decrease in manufacturing costs for Oxbryta. Following FDA approval of Oxbryta in November 2019, GBT now capitalizes manufacturing of the product to inventory. The decrease in R&D expenses was partially offset by increased costs related to preclinical research and manufacturing activities for inclacumab, increased employee-related costs, and increased costs for other preclinical research activities related to the collaboration with Syros Pharmaceuticals, Inc. Total R&D non-cash stock compensation expense incurred for the three months ended June 30, 2020, was $3.4 million compared with $4.7 million for the same period in 2019.

Sales, general, and administrative (SG&A) expenses for the three months ended June 30, 2020, were $49.1 million compared with $24.8 million for the same period in 2019. The increase in SG&A expenses for this comparative period 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 June 30, 2020, was $13.1 million compared with $6.2 million for the same period in 2019.

Net loss for the three months ended June 30, 2020, was $52.8 million compared with $57.3 million for the same period in 2019. Basic and diluted net loss per share for the three months ended June 30, 2020, was $0.86 compared with $1.01 for the same period in 2019. The company expects its operating costs to increase in subsequent quarters due to costs associated with expanding commercialization activities as well as costs associated with the advancement of its clinical pipeline.

Cash, cash equivalents, and marketable securities totaled $574.2 million at June 30, 2020, compared with $695.0 million at December 31, 2019.

Conference Call Details
GBT will host a conference call today, Wednesday, August 5, 2020, at 4:30 p.m. ET to provide a general business update and discuss the financial results for the second quarter 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 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, 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.[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 Oxbryta® (voxelotor) tablets
Oxbryta (voxelotor) 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, Oxbryta inhibits sickle hemoglobin polymerization and the resultant sickling and destruction of red blood cells. Through addressing hemolytic anemia and improving oxygen delivery throughout the body, 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.[8] 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 Oxbryta in its Priority Medicines (PRIME) program, and the European Commission (EC) has designated Oxbryta as an orphan medicinal product for the treatment of patients with SCD.

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 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. 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.

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 Oxbryta, and other product characteristics; the commercialization, delivery, availability, and commercial and medical potential of Oxbryta; use of Oxbryta, including new prescriptions and related expectations; payer coverage for Oxbryta; ongoing and planned studies of Oxbryta and related protocols, activities, and expectations, including with respect to resumption of enrollment and participation in clinical trials and the impact on related timelines; GBT's financial position, outlook, guidance, and expectations; the COVID-19 pandemic and related expectations; the potential expansion of the approved use of Oxbryta for more patients in the U.S. and potential approval of Oxbryta to treat patients in Europe, including the submission of regulatory filings, the type and contents of such filings, and related timing; impacting the treatment, care and course of SCD; GBT's efforts with respect to health disparities, racial injustice, and enacting change; the significance and potential impact of the ACCEL program and the grant recipients; the potential of inclacumab; and advancing GBT's pipeline, 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 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; data and results may not meet regulatory requirements or otherwise be sufficient for further development, regulatory review, or approval; compliance with the funding and other 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

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5. Kato GJ, et al. *Nat Rev Dis Primers*. 2018;4:18010.
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8. 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 June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
	(Unaudited)	(Unaudited)	(Unaudited)	(Unaudited)
Product sales, net	$ 31,501	$ —	$ 45,619	$ —
Costs and operating expenses:				
Cost of sales	377	—	512	—
Research and development	34,085	36,010	73,858	70,476
Selling, general and administrative	49,075	24,794	96,736	42,849
Total costs and operating expenses	83,537	60,804	171,106	113,325
Loss from operations	(52,036)	(60,804)	(125,487)	(113,325)
Other income (expense):				
Interest income	1,514	3,706	4,370	7,537
Interest expense	(2,282)	(160)	(4,596)	(341)
Other expenses, net	(36)	(63)	(153)	(115)
Total other income, net	(804)	3,483	(379)	7,081
Net loss	$ (52,840)	$ (57,321)	$ (125,866)	$ (106,244)
Basic and diluted net loss per common share	$ (0.86)	$ (1.01)	$ (2.06)	$ (1.88)
Weighted-average number of shares used in computing basic and diluted net loss per common share	61,116,707	56,539,760	60,952,269	56,386,560

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

	June 30, 2020	December 31, 2019
Assets	(Unaudited)	
Current assets:		
Cash and cash equivalents	$ 384,716	$ 302,237
Short-term marketable securities	156,014	307,732
Other current assets	53,303	18,028
Total current assets	594,033	627,997
Property and equipment, net	39,668	27,113
Long-term marketable securities	33,479	85,030
Operating lease right-of-use assets	51,580	52,775
Other assets	2,907	3,184
Total assets	$ 721,667	$ 796,099
Liabilities and Stockholders' Equity		
Current liabilities	$ 69,402	$ 71,453
Long-term debt	73,775	73,559
Operating lease liabilities, noncurrent	81,903	72,359

Other noncurrent liabilities	771	34
Total liabilities	225,851	217,405
Total stockholders' equity	495,816	578,694
Total liabilities and stockholders' equity	$ 721,667	$ 796,099

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