GBT Appoints Heidi L. Wagner as Senior Vice President, Government Affairs and Policy

August 21, 2018

SOUTH SAN FRANCISCO, Calif., Aug. 21, 2018 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) today announced the appointment of Heidi L. Wagner as senior vice president, government affairs and policy. Ms. Wagner joins GBT from Alexion Pharmaceuticals, where she was senior vice president, global government affairs. At GBT, Ms. Wagner will create and lead the Company's government affairs and public policy function, initially focused on concerns related to the sickle cell disease (SCD) community. GBT is currently developing voxelotor, an oral, once-daily therapy, as a potentially disease-modifying therapy for SCD.

"As we continue to advance the clinical development of our voxelotor program, we are thrilled to bring Heidi on board to build our government affairs and public policy function from the ground up," said Ted W. Love, M.D., president and chief executive officer of GBT. "Heidi is an experienced biopharmaceutical leader with long-term legal and government policy expertise, as well as deep experience with policymakers and stakeholders in the U.S. and globally. Her addition to the team will ensure that GBT has an active and effective voice in the governmental processes and policies that impact not only the company but also the SCD community."

"GBT is at an important stage in the development of voxelotor, having just announced key Phase 3 clinical data that could potentially lead to an accelerated approval in the U.S.," said Ms. Wagner. "I look forward to bringing my experience in public policy, reimbursement, patient access and patient advocacy to GBT, and to working with its visionary management team to seek to move closer toward its goal of commercializing voxelotor and serving SCD patients in need."

Ms. Wagner spent more than eight years at Alexion Pharmaceuticals, where she held roles of increasing responsibility on the company's management team. Before Alexion, she had an 11-year tenure at Genentech, where she was responsible for managing a broad portfolio of U.S. federal legislative and regulatory policy issues. She also was involved in pricing, reimbursement and the commercial launches for Herceptin, Rituxan, Avastin, Lucentis and Tarceva. Earlier in her career, Ms. Wagner was a health policy director and consultant to the Healthcare Leadership Council, a trade association. She received a B.S. in journalism and mass communication from the University of Colorado and a J.D. from George Mason University School of Law.

About Voxelotor in Sickle Cell Disease
Voxelotor (previously called GBT440) is being developed as an oral, once-daily therapy for patients with SCD. Voxelotor works by increasing hemoglobin's affinity for oxygen. Since oxygenated sickle hemoglobin does not polymerize, GBT believes voxelotor blocks polymerization and the resultant sickling of red blood cells. With the potential to improve hemolytic anemia and oxygen delivery, GBT believes that voxelotor may potentially modify the course of SCD. In recognition of the critical need for new SCD treatments, the U.S. Food and Drug Administration (FDA) has granted voxelotor 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.

GBT is currently evaluating voxelotor in the HOPE (Hemoglobin Oxygen Affinity Modulation to Inhibit HbS PolymErization) Study, a Phase 3 clinical study in patients age 12 and older with SCD. Additionally, voxelotor is being studied in the ongoing Phase 2a HOPE-KIDS 1 Study, an open-label, single- and multiple-dose study in pediatric patients (age 6 to 17) with SCD. HOPE-KIDS 1 is assessing the safety, tolerability, pharmacokinetics and exploratory treatment effect of voxelotor.

About Global Blood Therapeutics
GBT is a clinical-stage biopharmaceutical company determined to discover, develop and deliver innovative treatments that provide hope to underserved patient communities. GBT is developing its lead product candidate, voxelotor, as an oral, once-daily therapy for sickle cell disease. To learn more, please visit www.gbt.com and follow the company on Twitter @GBT_news.

Forward-Looking Statements
Statements we make 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. We intend these forward-looking statements, including statements regarding the therapeutic potential and safety profile of voxelotor, our ability to implement and complete our clinical development plans for voxelotor, the availability of accelerated approval, our ability to build a government affairs and public policy function, our ability to have an active and effective voice in the governmental processes and policies that impact our company and the SCD community, our ability to generate and report data from our ongoing and potential future studies of voxelotor (including our ability to generate additional data from patients enrolled in our ongoing Phase 3 HOPE Study), the sufficiency of our data to support an application for regulatory approval, regulatory review and actions relating to voxelotor, and the timing of these events, 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 are making this statement for purposes of complying with those safe harbor provisions. These forward-looking statements reflect our current views about our plans, intentions, expectations, strategies and prospects, which are based on the information currently available to us and on assumptions we have made. We 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 our control including, without limitation, the risks that our clinical and preclinical development activities may be delayed or terminated for a variety of reasons, that results of clinical trials may be subject to differing interpretations, that regulatory authorities may disagree with our clinical development plans, including the sufficiency of our clinical data and of our primary and other key endpoints in our Phase 3 HOPE Study of voxelotor to support approval, or require additional studies or data to support approval or further clinical investigation of voxelotor, that drug-related adverse events may be observed in clinical development, that data and results may not meet regulatory requirements or otherwise be sufficient for further development, regulatory review or approval, that we may be unable to build a government affairs and public policy function, that we may be unable to have an active and effective voice in the governmental processes and policies that impact our company and the SCD community, and that we may need to devote additional time and resources to meet these regulatory requirements, along with those risks set forth in our Annual Report on Form 10-K for the fiscal year ended December 31, 2017, and our Quarterly Report on Form 10-Q for the quarter ended June 30, 2018, as well as discussions of potential risks, uncertainties and other important factors in our subsequent filings with the U.S. Securities and Exchange Commission. Except as required by law, we assume no obligation to update publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

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