



Rhythm Pharmaceuticals Announces Publication of Children's Book for Patients and Families Living with Bardet-Biedl Syndrome (BBS)

September 23, 2022

-- Book developed in collaboration with the Bardet-Biedl Syndrome Foundation --

-- BBS Global Day on Sept. 24 coordinated by BBS International --

BOSTON, Sept. 23, 2022 (GLOBE NEWSWIRE) -- Rhythm Pharmaceuticals, Inc. (Nasdaq: RYTM), a commercial-stage biopharmaceutical company focused on transforming the lives of patients and their families living with hyperphagia and severe obesity caused by rare melanocortin-4 receptor (MC4R) pathway diseases, today announced the publication of a children's book developed in collaboration with the Bardet-Biedl Syndrome (BBS) Foundation. The publication announcement of the book, titled, "*Understanding Hunger & Bardet-Biedl Syndrome (BBS): Gabe's Story*," comes ahead of the second annual BBS Global Day on September 24.

"This new children's book is a valuable educational resource for BBS families in the United States faced with the challenges of constant hunger, known as hyperphagia," said Timothy Ogden, President, BBS Foundation. "It's often difficult for affected children, their siblings, parents and loved ones to understand and explain how managing hunger affects every part of their day. The book is an important new tool for us as we seek to improve the lives of individuals and families affected by BBS."

"*Gabe's Story*" is designed to help children living with BBS make sense of their feelings, provide education on why they are experiencing insatiable hunger, and recognize they are not alone.

"The hyperphagia experienced by many patients with BBS and other rare MC4R pathway diseases can have a significant impact on their quality of life and that of their family," said Jennifer Chien, Executive Vice President and Head of North America for Rhythm. "We hope that '*Gabe's Story*' illustrates the unique challenges that an unrelenting hunger poses for families who are simply trying to go about their daily routine, and we are proud to join the effort to raise awareness and appreciation for what BBS families experience."

"*Gabe's Story*" is now available in the United States through Rhythm InTune, a support program designed for caregivers and people living with rare genetic diseases, including BBS. Rhythm plans to release an audiobook version in the coming months. You can download a PDF copy of the book [here](#). To request a hard copy of this book, please call 1-855-206-0815 or email patientsupport@rhythmtx.com.

BBS Global Day is coordinated by BBS International with assistance from BBS support organizations in France, Germany, Italy, the Netherlands, the United Kingdom (UK) and North America. BBS is an ultra-rare genetic disease that affects multiple organ systems.

"BBS Global Day is an opportunity to bring together international researchers and doctors for collaboration and to influence a BBS focused research agenda," said Véronique Héloir, President of Bardet-Biedl Syndrome International (BBSI). "Our goal is to host resources and provide a contact point to facilitate countries with or without a BBS association."

Clinical features of BBS may include visual impairment, obesity, development delays or cognitive impairment, polydactyly, and renal dysfunction. Hyperphagia and severe obesity beginning early in life may be common in people living with BBS. Hyperphagia can lead to extreme food seeking behavior. The estimated prevalence of BBS is 1,500 to 2,500 individuals in the U.S., and 2,500 individuals in the European Union (EU) and UK.

About Rhythm Pharmaceuticals

Rhythm is a commercial-stage biopharmaceutical company committed to transforming the lives of patients and their families living with hyperphagia and severe obesity caused by rare melanocortin-4 receptor (MC4R) pathway diseases. Rhythm's precision medicine, setmelanotide, is approved by the U.S. Food and Drug Administration (FDA) for chronic weight management in adult and pediatric patients 6 years of age and older with monogenic or syndromic obesity due to pro-opiomelanocortin (POMC), proprotein convertase subtilisin/kexin type 1 (PCSK1) or leptin receptor (LEPR) deficiency confirmed by genetic testing, or patients with a clinical diagnosis of Bardet-Biedl syndrome (BBS). The European Commission (EC) has authorized setmelanotide for the treatment of obesity and the control of hunger associated with genetically confirmed BBS or genetically confirmed loss-of-function biallelic POMC, including PCSK1, deficiency or biallelic LEPR deficiency in adults and children 6 years of age and above.

The UK's Medicines & Healthcare Products Regulatory Agency (MHRA) authorized setmelanotide for the treatment of obesity and the control of hunger associated with genetically confirmed loss-of-function biallelic POMC, including PCSK1, deficiency or biallelic LEPR deficiency in adults and children 6 years of age and above. Additionally, Rhythm is advancing a broad clinical development program for setmelanotide in other rare genetic diseases of obesity and is leveraging the Rhythm Engine and the largest known obesity DNA database -- now with approximately 45,000 sequencing samples -- to improve the understanding, diagnosis and care of people living with severe obesity due to certain genetic deficiencies. Rhythm's headquarters is in Boston, MA.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the U.S. Private Securities Litigation Reform Act of 1995. All statements contained in this press release that do not relate to matters of historical fact should be considered forward-looking statements, including without limitation statements regarding the potential, safety, efficacy, and regulatory and clinical progress of setmelanotide, the potential benefits of setmelanotide for patients with BBS, and our expectations surrounding potential regulatory submissions, approvals and timing thereof, and our business strategy and plans, including regarding commercialization of setmelanotide in Europe, Canada, the United States and other international

regions. Statements using word such as “expect”, “anticipate”, “believe”, “may”, “will” and similar terms are also forward-looking statements. Such statements are subject to numerous risks and uncertainties, including, but not limited to, our ability to enroll patients in clinical trials, the design and outcome of clinical trials, the impact of competition, the ability to achieve or obtain necessary regulatory approvals, risks associated with data analysis and reporting, our liquidity and expenses, the impact of the COVID-19 pandemic on our business and operations, including our preclinical studies, clinical trials and commercialization prospects, and general economic conditions, and the other important factors discussed under the caption “Risk Factors” in our Quarterly Report on Form 10-Q for the quarter ended June 30, 2022 and our other filings with the U.S. Securities and Exchange Commission. Except as required by law, we undertake no obligations to make any revisions to the forward-looking statements contained in this release or to update them to reflect events or circumstances occurring after the date of this release, whether as a result of new information, future developments or otherwise.

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A photo accompanying this announcement is available at <https://www.globenewswire.com/NewsRoom/AttachmentNg/558ac9b9-e3fd-4dbe-ad07-a3f14b30de0e>.



Source: Rhythm Pharmaceuticals, Inc.

Understanding Hunger & Bardet-Biedl Syndrome (BBS): Gabe's Story



Developed in collaboration with the Bardet-Biedl Syndrome (BBS) Foundation