Rhythm Pharmaceuticals Announces Positive Opinion by the European Medicines Agency on Orphan Drug Designation for Setmelanotide for the Treatment of Bardet-Biedl Syndrome

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BOSTON, July 29, 2019 (GLOBE NEWSWIRE) -- Rhythm Pharmaceuticals, Inc. (Nasdaq:RYTM), a biopharmaceutical company focused on the development and commercialization of therapeutics for the treatment of rare genetic disorders of obesity, today announced that the European Medicines Agency's (EMA's) Committee for Orphan Medicinal Products (COMP) has adopted a positive opinion recommending setmelanotide for designation as an orphan medicinal product for the treatment of patients with Bardet-Biedl syndrome (BBS). People living with BBS may experience an insatiable hunger, also known as hyperphagia, and severe obesity beginning early in life. Rhythm is currently evaluating setmelanotide in an ongoing pivotal Phase 3 trial in patients with BBS and Alström syndrome.

"This positive opinion on orphan medicinal product designation for setmelanotide in BBS, as well as our PRIority MEdicines (PRIME) designation, reflects our commitment to working closely with the EMA to advance setmelanotide for patients with BBS who have limited treatment options for their hunger or obesity," said Murray Stewart, M.D., Chief Medical Officer of Rhythm. "We believe setmelanotide has the potential to transform the treatment of BBS and other rare genetic disorders of obesity by addressing underlying defects in the melanocortin-4 receptor (MC4R) pathway. This designation reflects the significant need for new therapies for BBS and we look forward to working with the EMA to potentially deliver setmelanotide to patients in Europe."

The orphan medicinal product designation by the European Commission is granted to medicines being developed for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition that affects fewer than five in 10,000 people in the EU. This designation could allow for a number of incentives, including protocol assistance, access to the centralized authorization procedure, reduced regulatory fees, and a ten-year period of market exclusivity in the EU after product approval.

About Bardet-Biedl Syndrome
BBS is an ultra-rare, genetic disorder that affects multiple organ systems. Clinical features may include and are not limited to severe obesity, insatiable hunger, retinal degeneration, polydactyly, kidney abnormalities, and developmental delays. There is great variability in presentation and severity of these features across individuals with BBS. Currently there are no approved therapies for regulating hunger in BBS.

About Setmelanotide
Setmelanotide is a potent MC4R agonist in development for the treatment of rare genetic disorders of obesity. Setmelanotide activates MC4R, part of the key biological pathway that independently regulates energy expenditure and appetite. Variants in genes within the MC4R pathway are associated with unrelenting hunger and severe, early-onset obesity. Rhythm is currently developing setmelanotide as a replacement therapy for patients with monogenic defects upstream of MC4R, for whom there are no effective or approved therapies. The FDA has granted Breakthrough Therapy designation to setmelanotide for the treatment of obesity associated with genetic defects upstream of the MC4 receptor in the leptin-melanocortin pathway, which includes POMC deficiency obesity, LEPR deficiency obesity, BBS and Alström Syndrome. The European Medicines Agency has also granted PRIority MEdicines (PRIME) designation for setmelanotide for the treatment of obesity and the control of hunger associated with deficiency disorders of the MC4R pathway.

About Rhythm
Rhythm is a biopharmaceutical company focused on the development and commercialization of therapies for the treatment of rare genetic disorders of obesity. Rhythm is currently evaluating the efficacy and safety of setmelanotide, the company's first-in-class MC4R agonist, in Phase 3 studies in patients with Pro-opiomelanocortin (POMC) deficiency obesity, Leptin receptor (LEPR) deficiency obesity, Bardet-Biedl syndrome, and Alström syndrome. The company is leveraging the Rhythm Engine -- comprised of its Phase 2 basket study, TEMPO Registry, GO-ID genotyping study and Uncovering Rare Obesity program -- to improve the understanding, diagnosis and potentially the treatment of rare genetic disorders of obesity. For healthcare professionals, visit www.UNcommonObesity.com for more information. For patients and caregivers, visit www.LEADforRareObesity.com for more information. The company is based in Boston, MA.

Forward-Looking Statements

This press release contains certain statements that are 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, and that involve risks and uncertainties, including statements regarding Rhythm's business strategy and goals, possible benefits from receipt of orphan

medical product designation, and its expectations regarding setmelanotide. Statements using words such as “expect”, “goal”, “anticipate”, “believe”, “may”, “will”, “plan” 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, and expenses, and other risks as may be detailed from time to time in our Annual Reports on Form 10-K and quarterly reports on Form 10-Q and other reports we file with the 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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