Rhythm Pharmaceuticals Receives Orphan Drug Designation from U.S. FDA for Setmelanotide for the Treatment of Alström Syndrome

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BOSTON, March 18, 2020 (GLOBE NEWSWIRE) -- Rhythm Pharmaceuticals, Inc. (Nasdaq: RYTM), a late-stage biopharmaceutical company aimed at developing and commercializing therapies for the treatment of rare genetic disorders of obesity, today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to setmelanotide for the treatment Alström syndrome. People living with Alström syndrome may experience an insatiable hunger, also known as hyperphagia, and severe obesity beginning early in life. Rhythm is currently evaluating setmelanotide's ability to reduce hunger and affect weight loss in an ongoing pivotal Phase 3 trial in patients living with Alström and Bardet-Biedl syndromes.

"Individuals and families living with Alström syndrome face a high disease burden, which often adversely affects their daily lives, and yet there are currently no treatment options available to address this serious unmet need," said Murray Stewart, M.D., Chief Medical Officer of Rhythm. "Orphan drug designation from the FDA reinforces the urgency of our work with setmelanotide in Alström syndrome, as we advance our pivotal Phase 3 trial to topline data expected by the end of this year or early next year."

Orphan drug designation is granted by the FDA to drugs and biologics that are intended for the safe and effective treatment, diagnosis or prevention of rare diseases or conditions that affect fewer than 200,000 people in the U.S. Orphan drug designation provides certain incentives, which may include tax credits towards the cost of clinical trials and prescription drug user fee waivers.

About Alström Syndrome
Alström syndrome is an ultra-rare genetic disorder that affects multiple organ systems. Insatiable hunger, also known as hyperphagia, and severe obesity beginning early in life may be seen in individuals living with Alström syndrome. Clinical features of Alström syndrome may include progressive visual and auditory impairments, insulin resistance and Type 2 diabetes, hyperlipidemia, progressive kidney dysfunction, cardiomyopathy and short stature in adulthood. There is great variability in presentation and severity of these symptoms across individuals with Alström syndrome. In the United States, the Company estimates that Alström syndrome affects approximately 500 people. Currently, there are no approved therapies that restore the impaired function of the melanocortin-4 receptor (MC4R) pathway, a component of the central melanocortin pathway, for reducing body weight and hunger in Alström syndrome.

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 insatiable hunger and early-onset, severe obesity. Rhythm is currently developing setmelanotide as a targeted therapy that restores function of an impaired MC4R pathway to reduce weight and hunger in patients 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 central melanocortin pathway, which includes POMC deficiency obesity, LEPR deficiency obesity, Bardet-Biedl syndrome 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 late-stage biopharmaceutical company focused on the development and commercialization of therapies for the treatment of rare genetic disorders of obesity. The company recently announced positive topline results from pivotal Phase 3 clinical trials of setmelanotide, its MC4R agonist, in people living with POMC deficiency obesity and LEPR deficiency obesity and plans to complete its first rolling NDA submission to the FDA in the first quarter of 2020. Rhythm is also evaluating setmelanotide in a pivotal Phase 3 trial in people living with Bardet-Biedl and Alström syndromes, with topline data from this trial expected in the fourth quarter of 2020 or early in the first quarter of 2021. Rhythm 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 anticipated timing for filing of an NDA and the release of results of clinical trials. 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, 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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