Rhythm Pharmaceuticals Announces First Patient Enrolled in Pivotal Phase 3 Clinical Trial Evaluating Setmelanotide in Bardet-Biedl and Alström Syndromes

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Company to host KOL Symposium focused on Bardet-Biedl Syndrome in New York City; live webcast beginning at 8:30am ET

BOSTON, Dec. 13, 2018 (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 the enrollment of the first patient in a pivotal Phase 3 clinical trial evaluating setmelanotide, the company's first-in-class melanocortin-4 (MC4R) agonist, in Bardet-Biedl Syndrome (BBS) and Alström Syndrome. The company made the announcement at its Key Opinion Leader (KOL) Symposium held today in New York City.

"We are encouraged by the continued progress across our development program for setmelanotide, including the enrollment of the first patient in our combined pivotal Phase 3 trial in BBS and Alström Syndrome," said Murray Stewart, M.D., Chief Medical Officer of Rhythm Pharmaceuticals. "This accomplishment reflects our commitment to the efficient development of setmelanotide as a potential first-in-class therapy for people living with MC4R pathway disorders. We are pleased to be working with leading clinicians, including those presenting at our symposium this morning, as we continue our work to improve patient identification and deepen our understanding of the burden of rare genetic disorders of obesity."

BBS and Alström Syndrome are rare genetic disorders that have many biological similarities. People living with these disorders may experience an insatiable hunger, also known as hyperphagia, and severe obesity beginning early in life. BBS affects approximately 1,500-2,500 people in the U.S. and may also be associated with cognitive impairment, polydactyly, renal dysfunction, hypogonadism, and visual impairment. Alström Syndrome is estimated to affect 500-1,000 people worldwide and can lead to short stature in adulthood, progressive visual and auditory impairment, insulin resistance and Type 2 diabetes, hyperlipidemia, and progressive kidney dysfunction. Currently there are no approved therapies for reducing body weight and hunger in BBS or Alström Syndrome.

"Many of our BBS patients struggle with insatiable hunger and severe obesity, which can be a challenging burden while also managing the disorder's potential effects on vision, kidney function, and other organs," said Robert M. Haws, M.D., Director of Clinical Research Center at the Marshfield Clinic Research Institute and Director of the Center of Excellence for Bardet-Biedl Syndrome. "We are eager to begin the pivotal Phase 3 trial as we continue to study setmelanotide's potential to reduce body weight and hunger in these patients." Dr. Haws serves as principal investigator for Rhythm's Phase 3 trial.

The combined pivotal Phase 3 trial is a multinational, open-label, single-arm study designed to enroll 30 patients, including at least 20 patients with BBS and at least six patients with Alström Syndrome, aged six years and older. The trial is expected to consist of 52 weeks of treatment with setmelanotide administered once daily by subcutaneous injection, including a 14-week placebo-controlled period. The primary endpoint of the trial is a responder analysis after approximately 52 weeks of therapy. The company plans to continue enrolling supplemental patients following enrollment of the last pivotal patient in order to generate additional data regarding the safety and efficacy in people living with BBS and Alström Syndrome.

Webcast Information:

The live audio webcast of today's event can be accessed under "Events & Presentations" in the Investors & Media section of the Company's website at www.rhythmtx.com. A replay of the webcast will be available on the Rhythm website for 30 days following the event.

About Setmelanotide

Setmelanotide is a potent, first-in-class, melanocortin-4 receptor (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 U.S. Food and Drug Administration 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, Bardet-Biedl Syndrome and Alström Syndrome.

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, BBS, and Alström Syndrome. Rhythm is dedicated to improving the understanding of severe obesity that results from specific genetic disorders. For healthcare professionals, visit www.uncommonobesity.com for more information. For patients and caregivers, visit www.geneticobesity.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 expectations regarding its anticipated timing for enrollment and design of clinical trials, the understanding of rare genetic disorders, and the development of a potentially transformative therapy. 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 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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