Rhythm Pharmaceuticals Announces Presentation of Updated Clinical Data from Phase 2 Basket Studies Evaluating Setmelanotide in Alström Syndrome at ObesityWeek 2018

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Company continues on track to initiate combined Phase 3 clinical trial in Alström Syndrome and Bardet-Biedl Syndrome before end of 2018

BOSTON, Nov. 15, 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 that interim, updated data from the company's ongoing Phase 2 basket studies evaluating setmelanotide in people with rare genetic disorders of obesity are being presented at ObesityWeek 2018 held November 11-15, 2018 in Nashville, TN. As previously reported, these results continue to show that treatment with setmelanotide reduced body weight and decreased appetite in multiple patients with Alström Syndrome. Safety data were consistent with previous clinical studies.

"There are currently no available treatment options for reducing body weight and regulating hunger in people living with Alström Syndrome or the closely-related Bardet-Biedl Syndrome (BBS)," said Keith Gottesdiener, M.D., Chief Executive Officer of Rhythm. "As we work to address the critical unmet needs in these rare genetic disorders, we are encouraged by these results from our basket studies that continue to support the development of setmelanotide. We look forward to advancing setmelanotide into a combined pivotal Phase 3 clinical trial in Alström Syndrome and BBS before year-end."

Setmelanotide is a potent, first-in-class melanocortin-4 receptor (MC4R) agonist that is being developed by Rhythm for the treatment of rare genetic disorders of obesity. The U.S. Food & Drug Administration (FDA) has included Alström Syndrome under a Breakthrough Therapy designation for setmelanotide for the treatment of obesity associated with genetic defects upstream of the MC4R in the central melanocortin pathway. The European Medicines Agency (EMA) 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.

Alström Syndrome is a rare genetic disorder caused by deleterious variants in the ALMS1 gene. People living with Alström Syndrome may experience severe obesity beginning early in life. Features might also include short stature in adulthood, progressive visual and auditory impairment, insulin resistance and Type 2 diabetes, hyperlipidemia, and progressive kidney dysfunction. Alström Syndrome is estimated to affect 500-1,000 people worldwide. Currently there are no approved therapies for reducing body weight and regulating hunger in Alström Syndrome.

Rhythm previously announced preliminary clinical data in one patient with Alström Syndrome from the ongoing Phase 2 basket studies evaluating setmelanotide, with only limited clinical data in two other patients in ongoing treatment. Now, in the poster presentation at ObesityWeek 2018, Joan Han, M.D., director of the Pediatric Obesity Program at Le Bonheur Children's Hospital and associate professor in the division of pediatric endocrinology at the University of Tennessee Health Science Center, provided updated data from treatment in multiple Alström Syndrome patients enrolled in the studies:

- The first, previously reported pediatric patient continued to maintain weight loss of 16.4 kg (decrease of 20.9%) and a reduction in baseline hunger score (decrease of 45.5%) following 56 weeks of observation, including 50 weeks of treatment at a therapeutic dose.
- Two additional pediatric patients demonstrated weight loss of 3.8 kg (decrease of 5.4%) and 5 kg (decrease of 5.5%) following 46 weeks and 20 weeks of observation, including 36 weeks and 13 weeks on therapeutic doses, respectively, with one of these patients also demonstrating a reduction in baseline hunger score (decrease of 66.7%).
- As previously reported, a fourth adult patient did not show compelling weight loss or hunger score improvement, and discontinued after withdrawing consent before completing 12 weeks of treatment on a therapeutic dose of setmelanotide.
- Treatment with setmelanotide was well tolerated. Adverse events reported by investigators included increased pigmentation of the skin/nevi and injection-site reactions. No patients discontinued the study due to adverse events and no serious adverse events were reported.

In addition, Ashley Shoemaker, M.D., assistant professor of pediatrics at Vanderbilt University Medical Center, presented a poster at ObesityWeek 2018 providing an overview of ongoing efforts to identify MC4R pathway variants in rare genetic disorders of obesity in Rhythm's Genetic Obesity ID (GO-ID) Genotyping Study.

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 melanocortin-4 receptor (MC4R) agonist, in Phase 3 studies in patients with pro-opiomelanocortin (POMC) deficiency obesity (which includes deficiencies in both the POMC and PCSK1 genes) and leptin receptor (LEPR) deficiency obesity. 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 initiation and enrollment for clinical trials, 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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