Rhythm Announces Preliminary Data from Phase 2 Study of Setmelanotide for Treatment of Bardet-Biedl Syndrome

October 31, 2017

-- Substantial Weight Loss Observed in 4 of 5 Patients --
-- Hunger Scores Improved in All 5 Treated Patients --
-- Results presented at ObesityWeek 2017 Meeting --

BOSTON, Oct. 31, 2017 (GLOBE NEWSWIRE) -- Rhythm Pharmaceuticals, Inc. (NASDAQ:RYTM), a biopharmaceutical company developing peptide therapeutics for rare genetic deficiencies that result in life-threatening metabolic disorders, today announced the presentation of preliminary data from an ongoing Phase 2 proof-of-concept study evaluating the safety and efficacy of setmelanotide, the company's novel melanocortin-4 receptor (MC4R) agonist, for the treatment of Bardet-Biedl syndrome (BBS). Results are being presented at the ObesityWeek 2017 meeting held October 29 – November 2, 2017, at the Gaylord National Resort & Convention Center in Washington, D.C.

Rhythm is advancing clinical research programs evaluating setmelanotide as a first-in-class treatment for a number of rare genetic forms of obesity caused by deficiencies in the MC4 pathway, a key biological pathway in humans that regulates weight by increasing energy expenditure and reducing appetite. Mutations affecting the MC4 pathway are a potential cause of early onset obesity and hyperphagia often associated with BBS, a rare genetic disorder that is also characterized by vision loss, polydactyly, kidney abnormalities, and other symptoms. BBS is estimated to have a prevalence of approximately one in 100,000 in North America.

"There are currently no approved treatment options to manage obesity in people living with BBS," said Keith Gottesdiener, CEO of Rhythm. "We are committed to addressing the unmet needs in the BBS community as well as other patient populations impacted by rare genetic forms of obesity that result from deficiencies along the MC4 pathway and are not treatable with lifestyle modifications or currently available treatment options."

The Phase 2 study includes five BBS patients who presented with morbid obesity and hyperphagia at initiation. Setmelanotide is being administered daily by subcutaneous injection for 52 weeks. Within 6-19 weeks of initiation, four patients experienced cumulative weight loss of 12.1% (17.8 kg), 7.9% (7.9 kg), 9.7% (11.8 kg), and 9.7% (9.5 kg) respectively. One patient showed no weight loss; however, achieved apparent weight stabilization. Hunger scores improved in all patients. Treatment has been well tolerated with adverse effects including mild injection site reactions and increased skin pigmentation.

The preliminary data provide support for continued evaluation of setmelanotide in BBS patients. Rhythm has also demonstrated proof-of-concept in Phase 2 clinical trials that evaluated setmelanotide for the treatment of two additional MC4 pathway deficiencies: pro-opiomelanocortin (POMC) deficiency obesity and leptin receptor (LepR) deficiency obesity. The U.S. Food and Drug Administration (FDA) granted setmelanotide Breakthrough Therapy Designation for the treatment of POMC deficiency obesity and LepR deficiency obesity, and setmelanotide is currently in Phase 3 development for both conditions.

About Rhythm

Rhythm is a biopharmaceutical company focused on the development and commercialization of peptide therapeutics for the treatment of rare genetic deficiencies that result in life-threatening metabolic disorders. Rhythm's lead peptide product candidate is setmelanotide, a first-in-class melanocortin-4 receptor (MC4R) agonist for the treatment of rare genetic disorders of obesity. Rhythm supports The Genetic Obesity Project (www.GeneticObesity.com), which is dedicated to improving the understanding of severe obesity that is caused by specific genetic defects. 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 clinical research programs and its commitment to addressing unmet needs in patients with certain forms of genetic obesity. 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, the impact of changes in the financial markets and global economic conditions, risks associated with data analysis and reporting, 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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