Rhythm Pharmaceuticals to Outline Expected 2019 Milestones and Review Updated Clinical Data in Bardet-Biedl Syndrome at 37th Annual J.P. Morgan Healthcare Conference

January 4, 2019

- Updated clinical data from Phase 2 basket studies evaluating setmelanotide in Bardet-Biedl Syndrome (BBS) show continued weight loss for additional two patients at longer-term follow-up –

- Pivotal Phase 3 clinical trials in pro-opiomelanocortin (POMC) and leptin receptor (LEPR) deficiency obesity fully enrolled and on track for initial data anticipated in third quarter of 2019 followed by New Drug Application (NDA) filings -

- Combined pivotal Phase 3 clinical trial in BBS and Alström Syndrome on target and expected to complete enrollment in second half of 2019 -

BOSTON, Jan. 04, 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 company will outline expected 2019 milestones and review updated clinical data from its Phase 2 basket studies of setmelanotide in patients with BBS in a presentation at the 37th Annual J.P. Morgan Healthcare Conference on Thursday, January 10, 2019. Setmelanotide is a first-in-class melanocortin-4 receptor (MC4R) agonist.

“We expect 2019 to be an important year for Rhythm, marked by key advancements across our clinical development program for setmelanotide and culminating in preparations for the submission of our first NDA for POMC and LEPR deficiency obesity,” said Keith Gottesdiener, M.D., Chief Executive Officer of Rhythm. “Our significant achievements in 2018, including completing pivotal enrollment in our Phase 3 trials in POMC and LEPR deficiency obesity, initiating an additional pivotal trial in BBS and Alström Syndrome, and beginning to build an integrated patient community, have brought us closer to providing a first-in-class therapeutic option for people living with rare genetic disorders of obesity who have no approved treatments for reducing body weight and hunger.”

Updated BBS Data from Phase 2 Basket Studies and Pivotal Phase 3 Trial Design

Today, Rhythm announced updated clinical data from two adolescent patients in its Phase 2 basket studies evaluating setmelanotide for the treatment of BBS who previously had only short-term results (18 and 15 weeks of total treatment). Data is now available for 47 and 41 weeks of treatment. These two patients have lost 11.2% (-13.7 kg) and 15.5% (-13.7 kg) of their body weight and experienced hunger score reductions of 66% and 21%, respectively. Treatment with setmelanotide continues to be well tolerated and safety data were consistent with previous clinical studies. In total, six out of nine BBS patients enrolled in the Phase 2 basket studies have now achieved a clinically meaningful weight loss of 10% change from baseline, which is the primary endpoint for the company’s pivotal Phase 3 clinical trial, and, as previously reported, one additional patient with Type-1 diabetes responded with marked improvements in hunger score and blood sugar levels.

In December 2018, Rhythm began treating patients in a combined pivotal Phase 3 clinical trial evaluating setmelanotide in BBS and Alström Syndrome and expects to complete pivotal enrollment of at least 20 patients with BBS and at least six patients with Alström Syndrome in the second half of 2019.

Progress in Phase 3 Clinical Trials in POMC and LEPR Deficiency Obesity

Rhythm’s ongoing, separate pivotal Phase 3 clinical trials evaluating setmelanotide in POMC and LEPR deficiency obesity continue to progress, with topline data expected in the third quarter of 2019. Pending positive results, the company expects to submit an NDA filing for each of POMC and LEPR deficiency obesity to the U.S. Food and Drug Administration (FDA) in late 2019 or early 2020.

“In 2019, Rhythm expects to make significant strides toward our vision of becoming a fully-integrated biotechnology company, as we approach the anticipated submissions of concurrent NDAs for setmelanotide in POMC and LEPR deficiency obesity,” said Murray Stewart, M.D., Chief Medical Officer of Rhythm. “As we advance toward this milestone, we are working hard to build an integrated community of patients, physicians, payors and caregivers, in order to better understand the burden of these disorders and ensure that people living with these conditions are diagnosed and readily able to access treatment.”

Additional Development Pipeline Updates

Rhythm continues to evaluate setmelanotide for the treatment of additional rare genetic disorders of obesity in its Phase 2 basket studies, including POMC and other MC4R pathway deficiency heterozygous obesities, as well as POMC epigenetic disorders. The company expects to announce updated interim data from these disorders in the first quarter of 2019. In addition, Rhythm is continuing to expand the evaluation of setmelanotide into additional MC4R pathway disorders in its Phase 2 basket studies.

Webcast Information

Rhythm will webcast its corporate presentation from the 37th Annual J.P. Morgan Healthcare Conference in San Francisco on Thursday, January 10, 2019 at 10:00 a.m. PST (1:00 p.m. EST). Live webcasts of the presentation and subsequent breakout session can be accessed under “Events & Presentations” in the Investors & Media section of the company’s website at www.rhythmtx.com. A replay of the webcasts will be available on the Rhythm website for 30 days.

About Setmelanotide

Setmelanotide is a potent, first-in-class, 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 POMC deficiency obesity, 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.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 expectations regarding its expectations for 2019, anticipated timing for enrollment and design of clinical trials, the 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 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.

Investor Contact:
Hannah Deresiewicz
Stern Investor Relations, Inc.
212-362-1200
hannahd@sternir.com

Media Contact:
Adam Daley
Berry & Company Public Relations
212-253-8881
adaley@berrypr.com

Rhythm Pharmaceuticals, Inc.