Rhythm Pharmaceuticals Completes Enrollment of Pivotal Cohort in Phase 3 Clinical Trial Evaluating Setmelanotide in Bardet-Biedl and Alström Syndromes

December 5, 2019

-- Topline data expected in the fourth quarter of 2020 or early in the first quarter of 2021 --

-- Supplemental cohort continues enrollment to meet patient demand --

BOSTON, Dec. 05, 2019 (GLOBE NEWSWIRE) -- Rhythm Pharmaceuticals, Inc. (Nasdaq:RYTM), a biopharmaceutical company aimed at developing and commercializing therapies for the treatment of rare genetic disorders of obesity, today announced it completed enrollment of the pivotal cohort in its Phase 3 clinical trial evaluating setmelanotide for the treatment of insatiable hunger and severe obesity in individuals living with Bardet-Biedl syndrome (BBS) or Alström syndrome. The Company enrolled 32 individuals with BBS and six individuals with Alström syndrome in the pivotal cohort, and it will continue to enroll patients in a supplemental cohort to meet demand and provide further data on the use of setmelanotide in people living with these conditions.

"With no approved treatment options available, there is a pressing need for a new therapy that addresses the insatiable hunger and severe obesity that people living with BBS or Alström syndrome may experience," said Murray Stewart, M.D., Chief Medical Officer of Rhythm. "We believe the significant demand among patients, families, caregivers and healthcare providers in the BBS community reflects this need for a therapy to address these conditions. We look forward to continuing to partner with the BBS and Alström syndrome communities as we work to identify patients, better understand the long-term burden of these disorders and advance this pivotal trial toward topline data in the fourth quarter of 2020 or early in the first quarter of 2021."

About Bardet-Biedl and Alström Syndromes
BBS and Alström syndrome are ultra-rare genetic disorders that affect multiple organ systems. Clinical features of BBS may include cognitive impairment, polydactyly, renal dysfunction, hypogonadism and visual impairment. Clinical features of Alström syndrome may include progressive visual and auditory impairment, insulin resistance and Type 2 diabetes, hyperlipidemia, progressive kidney dysfunction, cardiomyopathy and short stature in adulthood. Insatiable hunger, also known as hyperphagia, and severe obesity beginning early in life may be common in people living with either BBS or Alström syndrome. There is great variability in presentation and severity of these symptoms across individuals with BBS or Alström syndrome. In the United States, the Company estimates that BBS affects approximately 2,500 people and that Alström syndrome affects approximately 500 people. Currently, there are no approved therapies targeting the melanocortin-4 receptor (MC4R) pathway for reducing body weight and hunger in BBS or Alström syndrome.

About the Pivotal Phase 3 Trial
The pivotal Phase 3 trial is designed to evaluate setmelanotide, a MC4R agonist, for the treatment of obesity and hyperphagia in people with BBS or Alström syndrome (NCT03746522). The trial is 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. Participants were randomized to placebo or setmelanotide for 14 weeks followed by an open-label period on setmelanotide for 52 weeks. The primary endpoint of the trial is the proportion of participants (≥12 years of age) who achieve at least 10 percent reduction in body weight from baseline at approximately 52 weeks of therapy. Rhythm expects to report topline data from this trial in the fourth quarter of 2020 or early in the first quarter of 2021.

About Rhythm
Rhythm is a 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 pro-opiomelanocortin (POMC) deficiency obesity and leptin receptor (LEPR) deficiency obesity, and plans to complete its first rolling New Drug Application (NDA) submission to the U.S. Food and Drug Administration (FDA) in the fourth quarter of 2019 or the first quarter of 2020. Rhythm is also evaluating setmelanotide in a pivotal Phase 3 trial in people living with BBS and Alström syndrome, 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 enrollment of patients in clinical trials and submission of an NDA, its expectations regarding addressable patient populations, its expectations regarding the timing of topline data from its clinical trials, and its ongoing efforts related the efficacy of setmelanotide in patients with POMC deficiency obesity, LEPR deficiency obesity, BBS syndrome and Alström syndrome. 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, our sufficiency of cash and our 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.

Corporate Contact:
David Connolly
Head of Investor Relations and Corporate Communications
Rhythm Pharmaceuticals, Inc.
857-254-4489
dconnolly@rhythmtx.com

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

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

Source: Rhythm Pharmaceuticals, Inc.