Rhythm Pharmaceuticals Receives European Medicines Agency PRIME Designation for Setmelanotide in Rare Genetic Disorders of Obesity

July 23, 2018

-- Program Offers Enhanced Support for Development of Medicines that Target Unmet Medical Needs --

BOSTON, July 23, 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 the European Medicines Agency (EMA) has granted PRIority MEdicines (PRIME) designation for setmelanotide, the Company's first-in-class melanocortin-4 receptor (MC4R) agonist, for the treatment of obesity and the control of hunger associated with deficiency disorders of the MC4R pathway.

The PRIME program was launched by the EMA in 2016 to provide early and enhanced support to optimize the development of eligible medicines, speed up their evaluation, and contribute to timely patient access. To be eligible for PRIME, medicines must address an unmet medical need and preliminary data must be available showing the potential to address this need and bring a major therapeutic advantage to patients.

"We are pleased to receive PRIME designation, which allows us the opportunity to engage more closely with the EMA and potentially accelerate our clinical development of setmelanotide in the European Union," said Keith Gottesdiener, M.D., Chief Executive Officer of Rhythm Pharmaceuticals. "The receipt of PRIME designation for setmelanotide further validates the fact that people living with rare genetic disorders of obesity are severely underserved by current treatments, which fail to address underlying disease biology. We look forward to working closely with the EMA through our pivotal-stage clinical research to deliver a potentially transformative treatment option to patients."

Rhythm has completed pivotal enrollment in two ongoing Phase 3 clinical trials evaluating setmelanotide in pro-opiomelanocortin (POMC) and leptin receptor (LEPR) deficiency obesity and expects to report initial data in the third quarter of 2019. The Company is also evaluating setmelanotide in four additional rare genetic disorders of obesity, including Bardet-Biedl Syndrome (BBS), Alström Syndrome, POMC and other MC4R pathway heterozygous deficiency obesities, and POMC epigenetic disorders. Rhythm plans to initiate a Phase 3 trial of setmelanotide in BBS and Alström Syndrome by the end of 2018, and intends to continue enrolling patients with POMC and other MC4R pathway heterozygous deficiency obesities, as well as POMC epigenetic disorders, in its ongoing Phase 2 basket studies, in order to identify those patients most likely to benefit from setmelanotide treatment.

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 Pharmaceuticals

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 also supports The Genetic Obesity Project (www.GeneticObesity.com), which is dedicated to improving the understanding of severe obesity that results from specific genetic disorders. 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 plans and timing regarding the design of patient enrollment and announcement of data from clinical trials, and potential regulatory filings, and related statements. Statements using words such as "expect", "anticipate", "believe", "may", "will", "plan", "goal" 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, the impact of changes in the financial markets and global economic conditions, risks associated with data analysis and reporting, our use of cash 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.