Rhythm Receives Orphan Drug Designation for Setmelanotide for the Treatment of Prader-Willi Syndrome

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BOSTON, January 7, 2016— Rhythm announced today that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation for setmelanotide (RM-493), the company's novel melanocortin-4 receptor (MC4R) agonist, for the treatment of Prader-Willi syndrome (PWS).

"The orphan drug designation is an important regulatory milestone as we advance development of setmelanotide for the treatment of Prader-Willi syndrome," said Keith Gottesdiener, CEO of Rhythm. "Phase 2 trials are now under way for both PWS and POMC deficiency obesity, and we expect to complete these trials in the first half of 2016."

FDA's Orphan Drug Designation program is designed to facilitate drug development for rare diseases, providing substantial benefits to the sponsor including the potential for grants to fund clinical studies, study-design assistance, and several years of market exclusivity for the product upon regulatory approval.

About Setmelanotide (RM-493)
Setmelanotide is a potent, first-in-class MC4R agonist in development for the treatment of obesity caused by genetic deficiencies in the MC4 pathway, a key pathway in humans that regulates energy expenditure, homeostasis, and appetite. The critical role of the MC4 pathway in weight regulation was validated with the discovery that single genetic defects along this pathway result in early onset and severe obesity. A Phase 2 setmelanotide trial is ongoing for the treatment of Prader-Willi syndrome (PWS), a rare genetic disorder that causes life-threatening obesity. Recent scientific evidence implicates defects in the MC4 pathway as the likely cause of the weight and appetite abnormalities in PWS. A second Phase 2 trial is ongoing for the treatment of pro-opiomelanocortin (POMC) deficiency obesity, a very rare, life-threatening genetic disorder of the MC4 pathway associated with unrelenting appetite and obesity.

About Prader-Willi Syndrome
PWS is a life-threatening orphan disease with prevalence estimates ranging from approximately one in 8,000 to one in 52,000, and with at least 8,000 diagnosed patients in the United States. A hallmark of PWS is severe hyperphagia, an overriding physiological drive to eat, leading to severe obesity and other complications. For PWS patients, hyperphagia and obesity are the greatest threats to their health, and these patients are likely to die prematurely as a result of choking, stomach rupture, or from complications caused by morbid obesity. There is currently no approved treatment for the obesity and hyperphagia associated with PWS. Recent scientific studies identify deficiencies affecting the MC4 pathway as the likely cause of the obesity and hyperphagia associated with PWS, and demonstrate that an MC4R agonist can directly impact these symptoms.

About Rhythm (www.rhythmtx.com)
Rhythm is a biopharmaceutical company focused on developing 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, or MC4R, agonist for the treatment of rare genetic disorders of obesity. The company is based in Boston, Massachusetts.