Rhythm Receives FDA Breakthrough Therapy Designation for Setmelanotide for Treatment of POMC Deficiency Obesity

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— First Breakthrough Designation by FDA's Division of Metabolism and Endocrinology Products —

BOSTON, January 7, 2016— Rhythm, a biopharmaceutical company developing peptide therapeutics for rare genetic deficiencies that result in life-threatening metabolic disorders, announced today that the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy designation (BTD) to setmelanotide, the company's novel melanocortin-4 receptor (MC4R) agonist, for the treatment of pro-opiomelanocortin (POMC) deficiency obesity. Setmelanotide is in Phase 2 clinical trials for the treatment of rare genetic disorders of obesity caused by MC4 pathway deficiencies.

"Patients with POMC deficiency obesity have extreme and unrelenting appetite and obesity because of impaired function in the MC4 pathway," said Keith Gottesdiener, CEO of Rhythm. "We are pleased to receive this breakthrough designation and look forward to working closely with the FDA as we continue to advance the setmelanotide program."

Breakthrough Therapy designation is granted by the FDA to expedite the development and review of therapeutics to treat serious or life-threatening conditions for which preliminary clinical evidence indicates that the therapy may demonstrate substantial improvement on at least one clinically significant endpoint over existing therapies. This designation conveys all FDA fast track program features, such as eligibility for rolling NDA submissions and priority review (if supported by clinical data at the time of NDA). Additionally, this designation provides more intensive involvement of FDA staff in a proactive, collaborative, cross-disciplinary review process. Setmelanotide is the first BTD to be awarded by FDA's Division of Metabolism and Endocrinology Products, which is responsible for obesity and diabetes indications.

About Rhythm's Setmelanotide Clinical Programs

Study	Phase	Indication	Status
Setmelanotide NCT02311673	2 (N = 36)	Prader-Willi Syndrome (PWS)	Phase 2 enrolling
Setmelanotide NCT02507492	2 (N = 4-6)	POMC-deficiency obesity	Phase 2 enrolling

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 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 (MC4R) agonist for the treatment of rare genetic disorders of obesity. The company is based in Boston, Massachusetts.