



## Rhythm Announces Completion of \$41 Million Mezzanine Financing

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— Proceeds to finance significant expansion of genetic obesity clinical trials —

*BOSTON, February 16, 2017*—Rhythm, a biopharmaceutical company developing peptide therapeutics for rare genetic deficiencies that result in life-threatening metabolic disorders, today announced the completion of a \$41 million mezzanine round of financing with existing investors including Deerfield Management, Ipsen, OrbiMed, MPM Capital, New Enterprise Associates, Pfizer Venture Investments, Third Rock Ventures, and an undisclosed public healthcare investment fund. Rhythm will use the proceeds to advance setmelanotide (RM-493), the company's novel melanocortin-4 receptor (MC4R) agonist that targets the MC4 pathway, into Phase 3 clinical trials for pro-opiomelanocortin (POMC) and leptin receptor (LEPR) deficiency obesity. The company will also expand Phase 2 clinical trials for the treatment of obesity caused by several additional rare genetic deficiencies in the MC4 pathway, a key biological pathway in humans that regulates energy expenditure, homeostasis, and appetite.

"This mezzanine financing enables a significant expansion of the setmelanotide clinical development program, for which the FDA has granted Breakthrough Therapy Designation status for POMC deficiency obesity," said Bart Henderson, President and Founder of Rhythm. "In parallel, we are advancing setmelanotide into initial proof-of-concept studies for several additional rare genetic disorders of obesity that are life-threatening and for which there are no effective treatments."

During 2017, Rhythm plans to initiate Phase 3 registration studies for both POMC and LEPR deficiency obesity, along with Phase 2 proof-of-concept clinical studies for Bardet-Biedl syndrome, Alström syndrome, and POMC heterozygous deficiency obesity. Rhythm is also evaluating setmelanotide for the treatment of Prader-Willi syndrome in a Phase 2 study. The company previously presented clinical data for setmelanotide demonstrating substantial efficacy in reducing weight and hyperphagia in both POMC and LEPR deficiency obesity, rare diseases caused by genetic defects that are upstream of the MC4 receptor.

"We are extremely pleased for the continued support of our investors in this financing," said Keith Gottesdiener, CEO of Rhythm. "The initial proof-of-concept data for setmelanotide in both POMC and LEPR deficiency obesity is compelling and is the foundation for our MC4 genetic obesity program. This mezzanine financing enables both a significant expansion of the clinical program and the build-out of Rhythm's capabilities to support Phase 3 development."

### **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 biological 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. In 2016, *The New England Journal of Medicine* reported results from a setmelanotide Phase 2 trial in pro-opiomelanocortin (POMC) deficiency obesity that demonstrated substantial weight loss in two adult patients. At ObesityWeek 2016, investigators presented initial data for the first patient enrolled in a Phase 2 non-randomized, open-label clinical trial of setmelanotide for the treatment of leptin receptor (LEPR) deficiency obesity. Both POMC and LEPR deficiency obesity are rare genetic disorders associated with severe, early-onset obesity and unrelenting hyperphagia. The initial efficacy data with setmelanotide in these disorders demonstrate that setmelanotide has the potential to provide meaningful efficacy in genetic forms of obesity due to MC4 pathway deficiency by restoring absent LEPR-POMC signaling. The company is currently evaluating setmelanotide for the treatment of six genetic disorders of obesity: POMC and LEPR deficiency obesity, Prader-Willi syndrome, Bardet-Biedl syndrome, Alström syndrome, and POMC heterozygous deficiency obesity.

### **About Rhythm ([www.rhythmtx.com](http://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. Rhythm supports The Genetic Obesity Project ([www.GeneticObesity.com](http://www.GeneticObesity.com)), which is dedicated to improving the understanding of severe obesity that is caused by specific genetic defects. The company is based in Boston, Massachusetts.