Rhythm Announces “The Genetic Obesity Project” to Expand Understanding of Rare Genetic Disorders of Obesity

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BOSTON, July 27, 2016— Rhythm today announced "The Genetic Obesity Project" to improve the medical and scientific understanding of severe life-threatening obesity that is caused by specific genetic defects. The Project has initiated a genotyping study and a patient registry, both focusing on identifying people with pro-opiomelanocortin (POMC) deficiency obesity and LEPR (leptin receptor) deficiency obesity. POMC and LEPR deficiency obesity are two rare but important genetic disorders associated with unrelenting hyperphagia that begins in infancy and severe, early-onset obesity.

"These genetic disorders of obesity are debilitating, life-threatening conditions that are likely underdiagnosed," said Jennifer Miller, MD, Associate Professor, Pediatric Endocrinology, University of Florida. "It is important to know when obesity is due to a genetic cause because it is easier to find possible solutions once you know the problem. There can also be an important positive psychological impact for families once they know there is a genetic cause, and that it is not a failure of willpower. If you have a child who has severe early-onset obesity and a significantly increased appetite, they should be considered for the genotyping study."

Details for the registry and genotyping study follow:

— The goal of the registry—**Genetic Obesity ID | Registry**—is to gain a more accurate and reliable assessment of the worldwide prevalence of obesity resulting from POMC and LEPR deficiency, and to increase understanding of disease impact and progression. Rhythm is collaborating with healthcare providers to develop this registry as an international database that best supports the needs of patients and their healthcare providers. Healthcare providers can enroll patients with a confirmed diagnosis of POMC or LEPR deficiency at GeneticObesity.com.

— The goal of the genotyping study—**Genetic Obesity ID | Genotyping Study**—is to develop a screening algorithm for selecting patients to be genotyped and diagnosed with POMC and LEPR deficiency. Study investigators are located in both the US and EU. Healthcare providers can register patients to participate in the study at GeneticObesity.com.

"We are potentially on the verge of important advances in the treatment of patients with POMC and LEPR deficiencies," said Karine Clément, MD PhD, Professor of Nutrition and Director of ICAN, Pitié-Salpêtrière Hospital in Paris, France. "This is the time to improve awareness and understanding of these genetic disorders of obesity. Both the genotyping study and registry will help patients with these conditions, and also further our understanding of the biology and pathogenesis of obesity more broadly."

About The Genetic Obesity Project

Obesity is epidemic in the U.S., and current treatment approaches have demonstrated limited long-term success for most obese patients. Leveraging new understanding of severe obesity caused by specific genetic defects has the potential to improve both diagnosis and treatment for specific types of life-threatening obesity. The Genetic Obesity Project is dedicated to improving the understanding of severe obesity that is caused by specific genetic defects—particularly rare genetic disorders that result in life-threatening obesity.

About POMC Deficiency Obesity

POMC deficiency obesity is a life-threatening ultra-rare orphan disease, with approximately 50 patients reported to date, and we estimate that actual prevalence of this disorder could be between 100 and 500 patients worldwide. Patients with POMC deficiency have unrelenting hyperphagia that begins in infancy, and they develop severe, early-onset obesity. POMC deficiency obesity results from two different homozygous genetic defects, both upstream (which refers to the relative position of the defect earlier in the pathway) of the MC4 receptor. Currently, there is no approved treatment for the obesity and hyperphagia associated with POMC deficiency obesity.

About LEPR Deficiency Obesity

LEPR deficiency obesity is an ultra-rare orphan disease resulting in extreme hyperphagia and severe early-onset obesity with an estimated prevalence of 1% of subjects with severe, early-onset obesity. We estimate actual prevalence could be between 500 and 2,000 patients in the United States. Like other deficiencies upstream in the MC4 pathway, LepR deficiency results in loss of function in the MC4 pathway. Therefore, patients with this indication also manifest intense hyperphagia and severe obesity from early childhood. Currently, there is no approved treatment for the obesity and hyperphagia associated with LEPR deficiency 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. Rhythm supports The Genetic Obesity Project (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.