

Press release

Rhythm and Camurus Announce Positive Initial Data for Extended-Release Delivery of Setmelanotide for Treatment of Rare Genetic Disorders of Obesity

Once-weekly formulation of setmelanotide for the treatment of rare genetic obesity disorders using FluidCrystal® drug delivery technology

Boston, U.S. and Lund, Sweden — 27 June 2017 — Rhythm and Camurus (NASDAQ STO:CAMX) today announced positive initial results from an ongoing Phase 1A clinical trial evaluating the pharmacokinetics and tolerability of an extended-release formulation of setmelanotide (RM-493), Rhythm's novel melanocortin-4 receptor (MC4R) agonist in development for the treatment of rare genetic disorders of obesity. The new setmelanotide formulation uses Camurus drug delivery technology FluidCrystal® injection depot. Camurus has granted Rhythm a worldwide license to the FluidCrystal® technology to formulate setmelanotide and to develop, manufacture and commercialize this new formulation that has the potential for once-weekly dosing administered as a subcutaneous injection.

Rhythm is developing setmelanotide for the treatment of obesity caused by genetic deficiencies in the MC4 pathway, a key biological pathway in humans that regulates weight by increasing energy expenditure and reducing appetite. This ongoing Phase 1A clinical trial is a double-blind, placebo-controlled, single-rising dose study to evaluate the pharmacokinetics and tolerability of three doses of setmelanotide extended-release formulation in healthy obese patients (BMI \geq 30 kg/m²), with setmelanotide administered by subcutaneous injection.

"The initial results from this first trial are impressive and meet our pharmacokinetics and tolerability criteria for a once-weekly formulation of setmelanotide," said Dr. Keith Gottesdiener, Chief Executive Officer and Chairman of the Board of Directors of Rhythm. "We intend to develop this once-weekly formulation as a product line extension, to improve convenience for patients with genetic deficiencies in the MC4 pathway."

"Reducing the burden of daily injections and contributing to keeping the individual at a healthy weight, is an important step forward that may potentially provide patients with enhanced quality of life," said Dr. Fredrik Tiberg, President and Chief Executive Officer of Camurus.

The U.S. Food and Drug Administration (FDA) granted setmelanotide Breakthrough Therapy Designation (BTD) for the treatment of pro-opiomelanocortin (POMC) and leptin receptor (LepR) deficiency obesity. Results from Phase 2 clinical trials of setmelanotide demonstrated significant weight loss and substantial reductions in hunger for patients with POMC and LepR deficiency obesity. Rhythm recently initiated Phase 3 clinical trials for each of these indications.

About Setmelanotide

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 weight by increasing energy expenditure and reducing 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.

Rhythm recently initiated Phase 3 clinical trials of setmelanotide in POMC deficiency obesity and in LepR deficiency obesity. These are rare genetic disorders associated with severe, early-onset obesity and unrelenting hyperphagia caused by defects in the MC4 pathway that are upstream of MC4R. Initial efficacy data with setmelanotide demonstrate that it has the potential to restore lost function by activating the intact MC4 pathway below the genetic defect in these disorders. Rhythm is currently evaluating setmelanotide for the treatment of the following genetic disorders of obesity: POMC deficiency obesity, LepR deficiency obesity, Prader-Willi syndrome, Bardet-Biedl syndrome, Alström syndrome, POMC heterozygous deficiency obesity and POMC epigenetic disorders.

About FluidCrystal® injection depot

The FluidCrystal® injection depot delivers therapeutic levels of drug substance over selected extended periods – from days to months – from a single injection. While traditional depot therapeutics comprise complicated microsphere technology, the FluidCrystal® injection depot offers a liquid solution that transforms into a controlled release liquid crystal gel matrix in situ on contact with minute quantities of aqueous fluid at the injection site. The FluidCrystal® delivery system overcomes traditional side effects associated with high initial drug release on injection (drug burst) and poor drug stability by effectively encapsulating the drug compound in the nanopores of the depot matrix throughout the entire process from injection until final degradation. This, together with the ready-to-use product design, makes the system highly suitable for sustained parenteral delivery of peptides and proteins. FluidCrystal® is a registered trademark of Camurus AB.

About Rhythm

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 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. For more information, visit www.rhythmtx.com.

About Camurus

Camurus is a Swedish research-based pharmaceutical company committed to developing and commercialising innovative and differentiated medicines for the treatment of severe and chronic conditions. New drug products with best-in-class potential are conceived based on the proprietary FluidCrystal® drug delivery technologies and an extensive R&D expertise. Camurus' clinical pipeline includes products for treatment of cancer, endocrine diseases, pain and addiction, developed in-house and in collaboration with international pharmaceutical companies. The company's shares are listed on Nasdaq Stockholm under the ticker "CAMX". For more information, visit www.camurus.com.

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