

**PRESS RELEASE**

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## **Positive results from Phase 2 study of once-weekly FluidCrystal® formulation of setmelanotide in healthy volunteers with obesity**

- *Once-weekly formulation of setmelanotide achieved weight loss efficacy comparable to daily-dosing formulation*
- *Both weekly and daily formulations of setmelanotide were observed to be safe and well-tolerated*

**Lund, Sweden — 24 June 2020** — Camurus' partner Rhythm Pharmaceuticals, Inc. today announced interim data from a Phase 2 study evaluating once-weekly setmelanotide, CAM4072, an investigational melanocortin-4 receptor (MC4R) agonist. Healthy people with obesity treated with the weekly formulation of setmelanotide achieved comparable weight loss to those treated with the daily formulation, and both weekly and daily setmelanotide were observed to be safe and well-tolerated. Pharmacokinetic analyses showed similar trough drug concentrations for the daily and weekly formulations over the duration of therapy.

"The Phase 2 results are promising and show that our weekly FluidCrystal® formulation of setmelanotide delivers similar clinical benefits in terms of weight loss as the daily formulation, but with a more convenient dosing regimen for patients and potential for improved treatment adherence," said Fredrik Tiberg, President & CEO of Camurus. "We look forward to Rhythm's next steps towards registering weekly setmelanotide for the treatment of patients with rare genetic disorders of obesity for whom there is a high unmet medical need."

The Phase 2 study was designed to assess the pharmacokinetics, safety and tolerability of the weekly formulation of setmelanotide and its effect on reducing body weight in healthy individuals with a body mass index (BMI) of 40kg/m<sup>2</sup> or greater. A total of 75 individuals were included in this interim analysis: 42 individuals were treated with weekly setmelanotide (10mg, 20mg, or 30mg doses) for 12 weeks; 23 individuals were treated with placebo for 12 weeks; and ten individuals were treated with daily setmelanotide (2mg daily for 1 week, followed by 3mg daily for 11 weeks).

The interim data analysis demonstrated that individuals treated with weekly setmelanotide achieved similar weight loss to those treated with the daily formulation over 12 weeks of therapy. The mean difference in change from baseline on weight between each weekly dose of 10mg, 20mg or 30mg and daily dose of 2mg/3mg was -0.69kg (p=0.659), -0.02kg (p=0.990), and -1.71kg (p=0.296), respectively.

The weekly setmelanotide formulation was well tolerated with no serious adverse events, and the safety profile was similar to the daily formulation and consistent with prior clinical experience. Incidents of injection site reactions, hyperpigmentation and nausea or vomiting were classified as mild by investigators.

In addition, an analysis of pharmacokinetic data measuring mean trough drug concentrations in plasma samples taken weekly for the duration of treatment showed that 20mg and 30mg doses of the weekly setmelanotide formulation were very similar to the 3mg daily dose of setmelanotide with greater trough drug concentrations seen in the 30mg weekly dose.

Rhythm is continuing to analyze the efficacy, safety and pharmacokinetic data for weekly setmelanotide, and plans to share these data at an upcoming medical meeting. Rhythm also plans to discuss next steps towards registration of the weekly formulation with the FDA.

The weekly formulation of setmelanotide, CAM4072, is based on Camurus' FluidCrystal® injection depot technology which Rhythm licensed to develop with setmelanotide in 2016.

### **About setmelanotide**

*Setmelanotide is an investigational, melanocortin-4 receptor (MC4R) agonist. The MC4R is part of the key biological pathway that independently regulates energy expenditure and appetite. Variants*

*in genes may impair the function of the MC4R pathway, potentially leading to insatiable hunger and early-onset, severe obesity. Rhythm is currently developing setmelanotide as a targeted therapy to restore the function of an impaired MC4R pathway and, in so doing, reduce hunger and weight in patients with rare genetic disorders of obesity. Currently, no pharmacologic therapies exist to treat these conditions. The FDA has granted Breakthrough Therapy designation to setmelanotide for the treatment of obesity associated with genetic defects upstream of the MC4R in the central melanocortin pathway, which includes pro-opiomelanocortin (POMC) deficiency obesity and leptin receptor (LEPR) deficiency obesity. The European Medicines Agency (EMA) has also granted PRiority MEDicines (PRIME) designation for setmelanotide for the treatment of obesity and the control of hunger associated with deficiency disorders of the MC4R pathway. Both the FDA and EMA have granted orphan drug status to setmelanotide for POMC and LEPR deficiency obesities. The FDA has accepted Rhythm's New Drug Application (NDA) for daily administered setmelanotide for the treatment of POMC and LEPR deficiency obesities, granted Priority Review of the NDA and assigned a Prescription Drug User Fee Act (PDUFA) goal date of November 27, 2020. Rhythm expects to complete submission of a Marketing Authorization Application (MAA) for daily administered setmelanotide to treat individuals living with POMC deficiency obesity or LEPR deficiency obesity to the EMA in the second quarter of 2020.*

#### **About Rhythm Pharmaceuticals**

*Rhythm is a late-stage biopharmaceutical company focused on the development and commercialization of therapies for the treatment of rare genetic disorders of obesity. In August 2019, the company announced positive topline results from pivotal Phase 3 clinical trials of setmelanotide, its MC4R agonist, in people living with POMC deficiency obesity or LEPR deficiency obesity and, in March 2020, completed its first rolling NDA submission to the FDA. Rhythm is also evaluating setmelanotide for reduction in hunger and body weight in a pivotal Phase 3 trial in people living with Bardet-Biedl and Alström syndromes, with topline data from this trial expected in the fourth quarter of 2020 or early in the first quarter of 2021. Rhythm is based in Boston, MA, US.*

#### **About Camurus**

*Camurus is a Swedish, science-led biopharmaceutical company committed to developing and commercializing innovative, long-acting medicines for the treatment of severe and chronic conditions. New drug products with best-in-class potential are conceived based on the company's proprietary FluidCrystal® drug delivery technologies and its extensive R&D expertise. Camurus' clinical pipeline includes products for the treatment of cancer, endocrine diseases, pain and addiction, which are 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](http://www.camurus.com).*

#### **For more information**

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