PRESS RELEASE

EMA positive opinion for orphan drug designation to Camurus' octreotide SC depot for the treatment of polycystic liver disease

Lund, Sweden — 20 September 2024 — Camurus (NASDAQ STO: CAMX) today announced that the Committee for Orphan Medicinal Products (COMP) at the European Medicines Agency (EMA) has adopted a positive opinion for orphan drug designation (ODD) to the company's investigational medicinal product, octreotide subcutaneous (SC) depot (CAM2029), for the treatment of autosomal dominant polycystic liver disease (PLD). The European Commission is now assessing the opinion and is expected to grant the designation within 30 days.

PLD is a rare, genetic, and chronic disorder characterized by progressive growth of cysts in the liver, which can cause severe symptoms and result in impaired quality of life for patients. An estimated 37,000 patients in the US and EU are today living with PLD, of which a majority are women.[1]

"The positive opinion by the EMA underpins the need of a treatment for patients living with polycystic liver disease, for which there today is no approved pharmaceutical treatment in the EU", says Dr Fredrik Tiberg, President & CEO of Camurus. "CAM2029 is currently being assessed for efficacy and safety in the POSITANO clinical study in patients with symptomatic PLD, with topline results expected in the first half of 2025."

CAM2029 has previously been granted ODD for the treatment of autosomal dominant PLD by the US Food and Drug Administration (FDA)[2] and ODD for the treatment of acromegaly in the EU.

For more information about EMA's ODD program, visit: https://www.ema.europa.eu/en/human-regulatory/research-development/orphan-designation-research-development

For more information
Fredrik Tiberg, President & CEO
Tel. +46 (0)46 286 46 92
fredrik.tiberg@camurus.com

Fredrik Joabsson, Chief Business Development Officer
Tel. +46 (0)70 776 17 37
ir@camurus.com

About PLD
Polycystic liver disease (PLD) is a rare genetic and chronic disorder characterized by progressive growth of fluid-filled cysts in the liver, which can cause severe symptoms such as abdominal pain and discomfort, shortness of breath (dyspnea), indigestion (dyspepsia), gastro-esophageal reflux, and limited mobility. Rare complications are hepatic cyst hemorrhage and infection or rupture.[3-6] Age and gender contribute to disease severity; increasing age is positively associated with both cyst sizes and numbers, and women are highly overrepresented among symptomatic patients.[7-9] Most patients with PLD are diagnosed in their 30s after reporting a sudden and accelerated increase of abdominal breadth together with PLD-related symptoms.[8] There is currently no approved pharmacological treatment for PLD. Clinical studies indicate that somatostatin receptor ligands, e.g., octreotide, can slow down cyst growth, decrease fluid secretion, and reduce the liver volume.[10-12]

About octreotide SC depot (CAM2029)
CAM2029 is a ready-to-use, long-acting subcutaneous depot of octreotide under development for treatment of three rare disease indications: acromegaly, gastroenteropancreatic neuroendocrine tumors (GEP-NET), and polycystic liver disease (PLD). CAM2029 has been evaluated in a comprehensive clinical program, including five Phase 1 and 2 studies, two Phase 3 studies in acromegaly (ACROINNOVA 1 and 2), an ongoing Phase 3 study in patients with GEP-NET (SORENTO), and an ongoing Phase 2/3 study in patients with PLD (POSITANO). CAM2029 is

designed for enhanced octreotide exposure and convenient, once-monthly administration with a prefilled autoinjector pen to facilitate easy self-administration by patients.

About the POSITANO study
POSITANO is a randomized, double-blind, placebo-controlled Phase 2/3 study evaluating efficacy and safety of CAM2029 in patients with symptomatic PLD. Primary endpoint is change in height-adjusted total liver volume (htTLV) and first secondary endpoint is change in self-reported disease symptoms. For more information, visit www.clinicaltrials.gov (NCT05281328).

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 dependence, pain, cancer and endocrine diseases, 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.

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