

PRESS RELEASE

FDA grants Orphan Drug Designation in the US for CAM2029 for the treatment of polycystic liver disease

Lund, Sweden — 16 September 2021 — Camurus (NASDAQ STO: CAMX) today announced that the US Food and Drug Administration (FDA) has granted orphan drug designation (ODD) for the company's investigational medicinal product, octreotide subcutaneous depot (CAM2029), for the treatment of autosomal dominant polycystic liver disease.

Polycystic liver disease (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 an impaired quality of life of patients. There is today no approved pharmacological treatment available for PLD.

“The orphan drug designation for CAM2029 is a significant milestone in our efforts to develop an effective pharmacological treatment for people with polycystic liver disease and a recognition of the importance of our overall development program for CAM2029,” says Dr. Fredrik Tiberg, CEO and Head of R&D at Camurus. “CAM2029 is designed to address a significant unmet medical need in this population and could potentially become the first approved pharmacological treatment for PLD in the US market.”

Camurus has also recently received a Safe-to-Proceed letter from the FDA for the start of a randomized placebo-controlled Phase 2/3 study designed to assess efficacy and safety of CAM2029 in patients with symptomatic PLD.

Orphan drug designation qualifies the drug developer for a variety of development incentives and the prospect of seven years of market exclusivity, if approved. The FDA's ODD program is designed to advance the development of drugs for the treatment of rare diseases affecting fewer than 200,000 people in the US. More information about FDA rare diseases and the ODD program is available on www.fda.org.

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 polycystic liver disease

Polycystic liver disease (PLD) is a rare genetic and chronic disorder characterized by progressive growth of multiple (> 10) 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, infection or rupture.¹⁻³ 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.⁴⁻⁶ Most patients with PLD are diagnosed in their 30s after reporting a sudden and accelerated increase of abdominal girth, together with other PLD-related symptoms.⁵ There is currently no approved pharmacological treatment for PLD, but growing scientific evidence has shown that somatostatin analogues, such as octreotide, are effective in slowing cyst growth and fluid secretion in the liver and that they may also help reduce liver volume.⁷⁻⁹

About CAM2029

CAM2029 is a ready-to-use, long-acting subcutaneous depot of octreotide under development for treatment of three rare diseases; acromegaly, gastroenteropancreatic neuroendocrine tumors (GEP-NET), and polycystic liver disease (PLD). CAM2029 has been successfully evaluated in four

completed clinical Phase 1 and 2 studies and is being assessed in two ongoing pivotal Phase 3 studies in acromegaly and a pivotal Phase 3 study in GEP-NET. A Phase 2/3 study in patients with PLD is under preparation. CAM2029 is developed for enhanced octreotide exposure and easy self-administration by patients using a prefilled pen injector. CAM2029 has been granted orphan designation in the EU for the treatment of acromegaly and in the US for the treatment of PLD.

About Camurus

Camurus is a Swedish science-led biopharmaceutical company committed to developing and commercializing 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 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.

References

1. Abu-Wasel, B., et al., Pathophysiology, epidemiology, classification and treatment options for polycystic liver diseases. *World J Gastroenterol*, 2013. 19(35): p. 5775-86.
2. Perugorria, M.J., et al., Polycystic liver diseases: advanced insights into the molecular mechanisms. *Nat Rev Gastroenterol Hepatol*, 2014. 11(12): p. 750-61.
3. Neijenhuis, M.K., et al., Impact of liver volume on polycystic liver disease-related symptoms and quality of life. *United European Gastroenterol J*, 2018. 6(1): p. 81-88.
4. Van Keimpema L., et al. . Patients with isolated polycystic liver disease referred to liver centres: clinical characterization of 137 cases. *Liver international : official journal of the International Association for the Study of the Liver*. 2011;31(1):92-8.
5. van Aerts RMM, et al. . Clinical management of polycystic liver disease. *J Hepatol*. 2018;68(4):827-37.50. van Aerts RMM, Kievit W, de Jong ME, Ahn C, Bañales JM, Reiterová J, et al. Severity in polycystic liver disease is associated with aetiology and female gender: Results of the International PLD Registry. *Liver international : official journal of the International Association for the Study of the Liver*. 2019;39(3):575-82.
6. Gevers T. J. G., et al. *Liver Int*. 2015 May;35(5):1607-14.. [doi: 10.1111/liv.12726](https://doi.org/10.1111/liv.12726);
7. isani A., et al. *Clin Gastroenterol Hepatol*. 2016 Jul;14(7):1022-1030. [doi: 10.1016/j.cgh.2015](https://doi.org/10.1016/j.cgh.2015);
8. van Aerts R., et al., *Gastroenterology*. 2019 Aug;157(2):481-491. [doi:10.1053/j.gastro.2019.04.018](https://doi.org/10.1053/j.gastro.2019.04.018)

This information is information that Camurus AB is obliged to make public pursuant to the EU Market Abuse Regulation. The information was submitted for publication, through the agency of the managing director, at 11:00 am CET on 16 September 2021.