PRESS RELEASE
PledPharma AB
Stockholm, December 07, 2020

First patient dosed in Phase IIb/III study with Emcitate®

Stockholm, December 07, 2020. PledPharma AB (publ) (STO: PLED) announced today that the first patient has been dosed in the pivotal Phase IIb/III early intervention study in young patients with the drug candidate Emcitate®, a novel therapy developed to treat MCT8 deficiency, a genetic disturbance in thyroid hormone signalling with life-long severe disability.

Emcitate is developed for the treatment of MCT8 deficiency, a genetic disturbance in thyroid hormone trafficking with detrimental natural history and no currently available therapy. The pivotal Phase IIb/III study is an international, open label, multi-centre study in very young children with MCT8 deficiency, run at 10 centres in both Europe and North America. 12-22 children 0-30 months of age is planned to be included in the study, the design of which has been discussed and anchored with the regulatory authorities European Medicines Agency (EMA) and the U.S. Food and Drug Administration (FDA).

The efficacy endpoints in the study are improvement of neurocognitive development, achievement of motor milestones, and a normalisation of thyroid hormone function tests and markers of thyrotoxicosis. The recruitment of patients is projected to be completed in H2 2021, with interim results in 2022 to pave the way for regulatory submission, market approvals and commercial launch.

“We see a great potential for Emcitate to be beneficial to patients with MCT8 deficiency where there is no available treatment today. The start of this study confirms our commitment to build a specialized late-stage orphan drug development company, which will be renamed Egetis Therapeutics, that was formed when PledPharma recently acquired Rare Thyroid Therapeutics.,” said Nicklas Westerholm, CEO of PledPharma.

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About PledPharma

PledPharma is an innovative, unique and integrated pharmaceutical drug development company, focusing on improving treatments for diseases with substantial unmet medical need. The drug candidate Aladote® is a first in class drug candidate developed to reduce the risk of acute liver injury associated with paracetamol poisoning. A proof of principle study has been successfully completed and the design of the upcoming pivotal Phase IIb/III study for Aladote has been finalized after completed interactions with FDA, EMA and MHRA. Aladote® has been granted Orphan Drug Designation in the US. Through the acquisition of Rare Thyroid Therapeutics (RTT), the clinical portfolio also includes Emcitate®, for the treatment of MCT8 deficiency, a rare disease with high unmet medical need and no available treatment. A pivotal Phase IIb/III early intervention study was initiated in Q4 2020. Emcitate has been granted Orphan Drug Designation in the US and EU. The Phase III POLAR program for the drug candidate PledOx® was prematurely stopped in Q2 2020. Results from POLAR program will determine if further development of PledOx is warranted via strategic partnerships and is expected to be announced in Q4 2020. The company is planning for a name change to Egetis Therapeutics pending a resolution at the EGM on December 11, 2020

PledPharma

About Emcitate®

Emcitate is one of PledPharma's lead candidate drugs in clinical development. It addresses MCT8 deficiency, which is a rare disease with high unmet medical need and no available treatment which affects 1:70,000 males. Emcitate obtained Orphan Drug Designation in the EU in 2017 and the US in 2019. A US Rare Paediatric Disease Designation (RPD) was received in November 2020. Upon approval of a new drug application, sponsors holding a RPD can apply to receive a US Rare Pediatric Disease Priority Review Voucher (PRV). A Phase IIb clinical trial has been completed which showed significant and clinically relevant treatment effects on thyrotoxic symptoms of the disease. A pivotal Phase IIb/III early intervention study was initiated in Q4 2020.