PRESS RELEASE
PledPharma AB
Stockholm November 18, 2020

FDA grants Rare Pediatric Disease designation to Emcitate®

Stockholm, November 18, 2020. PledPharma AB (publ) (STO: PLED) announced today that the US Food and Drug Administration (FDA) has granted the company's application for Rare Pediatric Disease designation (RPD) for its lead candidate Emcitate® in the treatment of MCT8 deficiency.

Emcitate was recently added to PledPharma's portfolio through the acquisition of Rare Thyroid Therapeutics (RTT) and is under development for the treatment of MCT8 deficiency, a rare congenital disorder of thyroid hormone trafficking with detrimental natural history and no currently available therapy. Approximately 1 in 70,000 males is affected. A successful Phase IIb trial has been completed and a pivotal study is expected to start in Q4 2020. Emcitate holds Orphan Drug Designation (ODD) in both EU and the US.

"We are very happy that the US FDA through this designation confirms the severity of MCT8 deficiency and the need for effective therapies to treat this devastating condition" said Nicklas Westerholm, CEO of PledPharma, and continued "This is a valuable recognition and we look forward to continue working with the FDA as well as other regulatory agencies to bring Emcitate to the market as the first therapy for MCT8 deficiency."

The FDA grants RPD to therapies for serious or life-threatening diseases primarily affecting children from birth to 18 years and affecting fewer than 200,000 people in the USA. Upon approval of a new drug application, sponsors holding a RPD can apply to receive a US Rare Pediatric Disease Priority Review Voucher (PRV), which can be used to obtain accelerated FDA review of a new drug application for any drug candidate, in any indication, shortening time to market in the US. The voucher may be sold or transferred to another sponsor. Up to the end of 2019 22 PRVs for rare pediatric diseases have been awarded by FDA, whereof 12 were sold to another drug sponsor with individual voucher sale prices ranging from $67 million to $350 million. Under the terms of PledPharma's acquisition of RTT, 50% of the potential net proceeds from such a sale of a PRV related to Emcitate should be paid as an earnout to the sellers of RTT.

For more information about US Rare Pediatric Disease Designation and Voucher Program, see https://www.fda.gov/regulatory-information/search-fda-guidance-documents/rare-pediatric-disease-priority-review-vouchers

For further information, please contact:

Nicklas Westerholm, CEO
Ph: +46 73-354 20 62
Email: nicklas.westerholm@pledpharma.se

The information was submitted for publication, through the agency of the contact persons set out above, at 2020-11-18, 15:00 CET.

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 is planned to start 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 (STO: PLED) is listed on the Nasdaq Stockholm main market since October 31, 2019. For more information, see www.pledpharma.com.

About Emcitate®

Emcitate is one of PledPharma's lead candidate drugs in clinical development. It addresses MCT8 deficiency, which is a rare disease of thyroid hormone trafficking with high unmet medical need and no available treatment which affects 1:70,000 males. Emcitate holds Orphan Drug Designation in the EU and both Orphan Drug and Rare Paediatric Disease Designation in the US. A Phase IIb clinical trial was completed with significant and clinically relevant effects. A pivotal Phase IIb/III study is expected to start in 2020.