PRESS RELEASE
PledPharma AB

PledPharma to initiate one pivotal Phase II/III study with Aladote® for marketing authorisation application in both US and EU

Stockholm, January 8, 2020. PledPharma AB (STO: PLED) announced today that following interactions with the US Food and Drug Administration (FDA) and the European Medicines Agency (EMA), the company has finalized the development program for Aladote®, a drug candidate intended to reduce liver damage associated with paracetamol poisoning. The development program consists of one pivotal Phase II/III study which is expected to be sufficient for a marketing authorisation application in both US and EU. The study is planned to be initiated mid-2020.

Aladote is a "first-in-class" drug candidate developed to prevent liver damage caused by paracetamol overdose, which has been granted Orphan Drug Designation in the US. A proof of principle study has been successfully completed, establishing safety and tolerability and providing an indication that Aladote may reduce liver injury.

Following interactions with the FDA and the EMA, PledPharma has finalized the development program for Aladote which consists of one pivotal Phase II/III study. This study is expected to be sufficient for a marketing authorisation application in both US and EU. It is targeting patients arriving late at hospital, more than 8 hours after an overdose, and for which current standard of care, N-acetylcysteine (NAC), is no longer effective. The study will consist of two stages with an interim analysis in between that includes a futility analysis and dose selection where the most effective dose will be continued.

"We are very pleased with our interactions with both the FDA and the EMA which have enabled us to finalize our development strategy for Aladote. In parallel with our interactions with the regulatory agencies to finalize specific study details, we are performing a Contract Research Organisation (CRO) selection and working on a feasibility study to enable a rapid initiation of the clinical study. We look forward to share further details about the study in due time" said PledPharma's CEO, Nicklas Westerholm.

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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 company's most advanced project **PledOx®** is being developed to reduce nerve damage associated with chemotherapy. A global phase III program is ongoing. The drug candidate **Aladote®** is being developed to reduce the risk of acute liver injury associated with paracetamol/acetaminophen poisoning. A proof of principle study has been successfully completed and the design of the next study is being finalised. Aladote® has been granted Orphan Drug Designation in the US. PledPharma (STO: PLED) is listed on the Nasdaq Stockholm main market. For more information, see http://www.pledpharma.com/

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About Aladote®

Aladote® is a “first-in-class” drug candidate with the potential to reduce the risk of acute liver injury associated with paracetamol/acetaminophen poisoning. Aladote® has shown good efficacy in relevant preclinical models, even in the time-window when N-acetylcysteine (NAC) treatment no longer is effective. A proof of principle study in patients with paracetamol poisoning has been successfully completed. The study results established the safety and tolerability of the combination of Aladote® and NAC. Further, the results indicate that Aladote® may reduce liver injury in this patient population. Aladote® has been granted Orphan Drug Designation in the US.

Paracetamol/acetaminophen is the most used drug in the world for the treatment of fever and pain, but also one of the most overdosed drugs – intentional or unintentional. Paracetamol overdose is also one of the most common method in intentional suicide attempts. When excessive amounts of paracetamol are broken down in the liver, the harmful metabolite NAPQI is formed, which can cause acute liver injury. The current standard of care for paracetamol poisoning (NAC) is effective if the patient seeks medical care within 8 hours of ingestion. However, NAC is substantially less effective if started more than 8 hours after overdose.