PRESS RELEASE
PledPharma AB
Stockholm, June 25, 2018

Aladote® is concluded as safe in first clinical study – PledPharma aims to apply for Orphan Drug Status in the US

Today PledPharma (publ) announces that the company has received a positive conclusion of the safety evaluation from the Data Safety Monitoring Board for the Aladote proof of principle study in patients with paracetamol poisoning. As per the study design, complete results including exploratory endpoints such as biomarkers of liver damage will be available in September 2018, at the completion of the 90 day follow-up period from last patient dosed.
Subject to complete clinical study results, the company's ambition is to apply for an Orphan Drug Designation in the US.

The proof-of-principle trial (phase 1b/2a) is being conducted at the Queen's Medical Research Institute, University of Edinburgh under the supervision of Dr. James Dear, one of the leading experts in the treatment of paracetamol/acetaminophen intoxication.
The primary goal of the trial is to evaluate the safety and tolerability of Aladote® in combination with N-acetylcysteine (NAC). NAC is current standard of care for the treatment of paracetamol poisoning. In addition, several biomarkers of liver damage will be measured. In total, 24 patients were recruited to three dose cohorts with eight patients per dose cohort. In each cohort, six patients were treated with the combination of Aladote® and NAC and two were treated with NAC alone.

The independent data safety monitoring board has now concluded their review of the safety information from all patients and has not found any safety concerns after the treatment with Aladote® at three different dose cohorts in combination with NAC.

Furthermore, subject to complete clinical study results available in September, the company's ambition is to apply for an Orphan Drug Designation (ODD) in the US. ODD is designated to drugs being developed for the treatment of rare diseases/disorders that affect fewer than 200,000 people in the US.

"There is a great unmet medical need for a new therapy to prevent liver injury in patients who present late after paracetamol overdose. Aladote® has the potential to address this need and become an important therapy for this common medical emergency", says Dr. James Dear, University of Edinburgh, the leading investigator of the study.

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"Today's positive conclusion regarding Aladote's® safety profile is an important step forward in the continued global development of the product. Subject to final clinical study results, we aim to apply for an Orphan Drug Designation in the US.", says PledPharma's CEO Nicklas Westerholm.

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About Aladote®
Aladote® is a "first-in-class" drug candidate with the potential to prevent the development of acute liver failure caused by paracetamol overdose. Aladote® has shown good efficacy in relevant preclinical models, even in the time-window when N-acetylcysteine (NAC) treatment is no longer is effective. A proof of principle study in patients with paracetamol poisoning is ongoing at the Royal Infirmary of Edinburgh.
Paracetamol 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 failure. 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.

About PledPharma
PledPharma develops new drugs that protect the body against oxidative stress – a potentially debilitating and sometimes life-threatening condition that can be caused by chemotherapy treatment and following acetaminophen (paracetamol) overdose. The company's most advanced project PledOx® is being developed to reduce nerve damage associated with chemotherapy. A phase IIb study has been conducted and serves as the basis for the initiated global phase III program. The drug candidate Aladote® is being developed to reduce the risk of acute liver failure associated with acetaminophen poisoning. A proof of principle study has been conducted and will serve as the basis for the continued development.
PledPharma (STO: PLED) is listed on Nasdaq First North. Erik Penser Bank is the company's Certified Adviser (tel +46 8 463 80 00). For more information, see http://www.pledpharma.se/

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