PRESS RELEASE
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Egetis receives positive opinion on orphan designation for Aladote® from the European Medicines Agency for the Prevention of Acute Liver Failure

Stockholm, Sweden, July 13, 2022. Egetis Therapeutics AB (publ) (Nasdaq Stockholm: EGTX) today announced that the Committee for Orphan Medicinal Products (COMP) of the European Medicines Agency (EMA) has adopted a positive opinion recommending *Aladote* (calmangafodipir) for designation as orphan medicinal product for the Prevention of Acute Liver Failure, a life-threatening condition.

Aladote is a first-in-class drug candidate developed for the treatment of paracetamol overdose in combination with N-acetylcysteine (NAC) to prevent acute liver failure. A proof-of-principle study has been successfully completed and the design of the upcoming pivotal Phase IIb/III study with the purpose of applying for market approval in the US and Europe for *Aladote* has been finalized after interactions with the US Food and Drug Administration (FDA), EMA and the UK Medicines and Healthcare products Regulatory agency (MHRA). Egetis expects to start this pivotal study for *Aladote* in 2022.

The positive opinion adopted by the EMA on the application for orphan designation for the Prevention of Acute Liver Failure follows the Orphan Drug Designation for the Treatment of Acetaminophen Overdose granted by the US FDA in 2019.

Nicklas Westerholm, CEO, Egetis Therapeutics, commented: "*We are delighted to receive a positive opinion for orphan designation from EMA for Aladote for the prevention of acute liver failure. There is a substantial medical need for additional treatment options for the approximately 25% of patients presenting to hospital after eight hours of paracetamol poisoning. These patients are at increased risk of Acute Liver Failure. We look forward to initiating our pivotal Phase IIb/III study with Aladote later in 2022.*"

N-acetylcysteine (NAC) is the current standard of care antidote for paracetamol poisoning. It is most effective if given within eight hours of the overdose. Patients arriving later to the hospital, and for those with a severe overdose, there is a need for more efficacious treatment options. The scientific rationale as well as clinical results from a completed proof-of-principle study indicate that *Aladote* in combination with NAC has the potential to reduce liver damage in the specified patient population.

In the EU, orphan drug status is given to products that treat, prevent, or diagnose a disease which is life-threatening or chronically debilitating and affects less than 5 in 10,000 people across the EU. Sponsors who obtain orphan designation in the EU benefit from protocol assistance, a type of scientific advice specific for designated orphan medicines, waivers or reductions of certain fees as well as a ten-year market exclusivity once the medicine is on the market. The European Commission is responsible for granting the orphan designation based on the positive opinion from EMA. For more information about orphan designation in the EU, please see www.ema.europa.eu/en/human-regulatory/overview/orphan-designation-overview

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About Egetis Therapeutics

Egetis Therapeutics is an innovative and integrated pharmaceutical company, focusing on projects in late-stage development for commercialization for treatments of serious diseases with significant unmet medical needs in the orphan drug segment.
The Company's lead candidate *Emcitate* is under development for the treatment of patients with monocarboxylate transporter 8 (MCT8) deficiency, a highly debilitating rare disease with no available treatment. In previous studies (Triac Trial I and a long-term real-life study) *Emcitate* has shown highly significant and clinically relevant results on serum T3 levels and secondary clinical endpoints. As a result of fruitful regulatory interaction Egetis intends to submit a marketing authorisation application (MAA) for *Emcitate* to the European Medicines Agency (EMA) in the first half of 2023 based on existing clinical data.
In the US, after discussions with the FDA, Egetis will conduct a small randomized, placebo-controlled study in 16 patients to verify the results on T3 levels seen in previous clinical trials and publications. Egetis intends to submit a new drug application (NDA) in the US for *Emcitate* in mid-2023 under the Fast-Track Designation granted by FDA.
Emcitate is currently being investigated in the fully recruited Triac Trial II, a Phase II/III study in very young MCT8 deficiency patients (<30 months of age) exploring potential disease modifying effects of early intervention from a neurocognitive and neurodevelopmental perspective. Results are expected in the first quarter of 2024 and are expected to be submitted post-approval to regulatory authorities shortly thereafter.
Emcitate holds Orphan Drug Designation (ODD) for MCT8 deficiency and resistance to thyroid hormone type beta (RTH- #) in the US and the EU. *Emcitate* has been granted Rare Pediatric Disease Designation (RPD) which gives Egetis the opportunity to receive a Priority Review Voucher (PRV) in the US, after approval.
The drug candidate *Aladote* is a first in class drug candidate 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 upcoming pivotal Phase IIb/III study with the purpose of applying for market approval in the US and Europe for *Aladote* has been finalized after completed interactions with FDA, EMA and MHRA. *Aladote* has been granted ODD in the US and has received a positive opinion for ODD in the EU.
Egetis Therapeutics (STO: EGTX) is listed on the Nasdaq Stockholm main market. For more information, see www.egetis.com

Attachments

Egetis receives positive opinion on orphan designation for Aladote® from the European Medicines Agency for the Prevention of Acute Liver Failure