**PRESS RELEASE**
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# Egetis announces detailed design for small, randomized, placebo-controlled trial for Emcitate for US NDA submission

**Stockholm, Sweden, October 17, 2022. Egetis Therapeutics AB (publ) (NASDAQ Stockholm: EGTX) today announced the detailed design for the small, randomized, placebo-controlled trial for the new drug application (NDA) in the United States for its leading candidate drug *Emcitate*, which is being developed for the treatment of monocarboxylate transporter 8 (MCT8) deficiency.**

Activities are ongoing to support a submission of a marketing authorisation application (MAA) to the European Medicines Agency (EMA) in the first half of 2023, based on already available clinical data. The NDA to the US FDA is targeted for mid-2023 under the Fast Track Designation granted by the FDA. As previously communicated, the NDA requires a small, randomized, placebo-controlled trial with 16 patients, and up to 30 days treatment duration with either *Emcitate* or placebo, to be conducted to verify the results on thyroid hormone T3 levels seen in previous clinical trials and publications. An application for this clinical trial has been agreed with the US FDA and the detailed design of the trial is now available on www.clinicaltrials.gov with identifier NCT05579327. The study will commence during the next month.

**Nicklas Westerholm, CEO of Egetis, said:** "*I am delighted to report that we progress towards submitting the MAA for Emcitate in the first half of 2023 and the NDA mid-2023 under the Fast Track Designation granted by the FDA. For the United States we will initiate the small, randomized, placebo-controlled trial with 16 patients, which is required for the new drug application, as soon as possible now when the detailed design has been agreed with the FDA. At the same time, we are continuing the step-wise build up of a commercial infrastructure in Europe and the US.*"

**About the *Emcitate* randomized placebo-controlled study for the NDA in the United States (ReTRIACt)**

This is a double-blind, randomized Phase 3 multicenter placebo-controlled study in at least 16 male participants diagnosed with MCT8 deficiency. Participants, from 4 years of age and having demonstrated stable maintenance treatment with *Emcitate*, will be randomized to receive placebo or *Emcitate* for 30 days or until reaching rescue criterion (serum total tri-iodothyronine [T3] > upper limit of normal [ULN] of the participant's normal range, for a sample collected during the 30-day Randomized Treatment Period). The primary endpoint is the proportion of participants in the placebo group, compared to those who continue to receive *Emcitate*, for which removal of *Emcitate* will lead to an increase of serum total T3 concentration above the ULN.

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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 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. The recruitment target was achieved in the second quarter 2022 and 22 patients have been included in the study. Results are expected in the first half 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-beta) in the US and the EU. MCT8 deficiency and RTH-beta are two distinct indications, with no overlap in patient populations. *Emcitate* has been granted Rare Pediatric Disease Designation (RPDD) 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) overdose. 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 and study start is planned for later in 2022. *Aladote* has been granted ODD in the US and in the EU.
Egetis Therapeutics (STO: EGTX) is listed on the Nasdaq Stockholm main market. For more information, see www.egetis.com

## Attachments

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