PRESS RELEASE
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Egetis announces first patient included and second site activated in the pivotal ReTRIACt trial

Stockholm, Sweden, July 24, 2023. Egetis Therapeutics AB (publ) (Nasdaq Stockholm: EGTX) today announced that the first patient has been included and a second site has been activated in the pivotal ReTRIACt clinical trial for the new drug application (NDA) in the USA for *Emcitate*. The trial will be conducted across three centers: at Erasmus Medical Center, Rotterdam, the Netherlands, Children's Hospital of Philadelphia, Philadelphia, PA, USA, and Addenbrooke's Hospital, Cambridge, UK. As previously reported, the Company expects topline results from the ReTRIACt study during the first half of 2024 and estimates a subsequent NDA submission in the USA in mid-2024, under the fast-track designation.

Nicklas Westerholm, CEO of Egetis, commented: *"I am delighted that the first patient has been included and that we now have two active sites in the ReTRIACt trial for Emcitate, which is pivotal for the New Drug Application in the USA. These are important milestones for the Company, and I would like to thank the investigators, patients, and parents/guardians for their commitments to participate in this trial, which is crucial for bringing the first possible treatment for MCT8 deficiency to patients in the USA. We continue to work diligently with the trial sites to facilitate a smooth and efficient execution of the ReTRIACt trial. For the EU, we are on track towards submitting the Marketing Authorisation Application for Emcitate in the early autumn of 2023, as previously communicated."*

About the ReTRIACt trial

The ReTRIACt trial (clinicaltrial.gov identifier NCT05579327) is a double-blind, randomized Phase 3 multicenter placebo-controlled study in at least 16 evaluable male participants diagnosed with MCT8 deficiency. The study protocol starts with an open-label treatment period in which a stable maintenance dose of tiratricol, essential for progression into the Randomized Treatment Period, will be established. The duration of the initial open-label treatment period will vary depending on whether the participant is currently receiving treatment with tiratricol at the time of enrollment in the study (Cohort A), or if they are considered to be tiratricol treatment-naïve (Cohort B). Participants are considered to be tiratricol-naïve if they have never previously been administered tiratricol, or have previously received tiratricol but are not receiving tiratricol at the time of enrollment. 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 triiodothyronine [T3] above upper limit of normal [ULN] of the participant's normal range, for a sample collected during the 30-day Randomized Treatment Period). The research hypothesis to be tested is that, for participants in the placebo group, removal of *Emcitate* will lead to an increase of serum total T3 concentration above the ULN and requirement of rescue treatment with *Emcitate*, compared to those who continue to receive tiratricol.

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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 drug 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 thyroid hormone T3 levels and secondary clinical endpoints. As a result of regulatory interaction Egetis intends to submit a marketing authorisation application (MAA) for *Emcitate* to the European Medicines Agency (EMA) during the early autumn of 2023 based on existing clinical data.
After a dialogue 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 2024 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 mid 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. This voucher can be transferred or sold to another sponsor.
Egetis Therapeutics (STO: EGTX) is listed on the Nasdaq Stockholm main market. For more information, see www.egetis.com

Attachments

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