PRESS RELEASE
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ReTRIACt trial design presented at the Annual Meeting of the European Society for Paediatric Endocrinology

Stockholm, Sweden, September 25, 2023. Egetis Therapeutics AB (publ) (Nasdaq Stockholm: EGTX) announces the poster presentation of the ReTRIACt trial design at the 61st Annual Meeting of the European Society for Paediatric Endocrinology (ESPE 2023), which was held in The Hague, The Netherlands, September 21-23, 2023. At home care and patient needs were at the core of the design to provide optimal comfort and retention for the participating families. The ReTRIACt trial aims to study the effects of tiratricol treatment withdrawal in MCT8 deficiency which is an ultra-rare condition that causes severe disabilities. This trial was requested by the US FDA as pivotal for the New Drug Application submission for tiratricol.
The patient-centred design of the ReTRIACt trial has taken into careful consideration the challenges of enrolling patients from small populations who cannot travel or mobilize independently. The trial uses specialist domiciliary nurses and home-based monitoring technology so that it can be conducted primarily in patients' homes. The first patients were enrolled in the trial in the third quarter of 2023 and the trial is estimated to complete patient enrolment during 2023. Topline results are expected during the first half of 2024.

Nicklas Westerholm, CEO of Egetis, commented: "*Studying rare diseases is a challenge due to the smaller patient populations. For the vulnerable MCT8 deficient patient population there are further challenges posed by the disabilities that the disorder causes. It was a priority for us to ensure that the ReTRIACt trial was truly accessible. We developed the trial with the patients and their carers in mind, in collaboration with a patient association. This led to its home-based approach which should allow more patients to enrol and stay in the trial, while avoiding unnecessary disruption to their quality of life. The data collated will support our US FDA New Drug Application submission for tiratricol for this patient population.*"

Poster Title:
Effects of tiratricol treatment withdrawal in monocarboxylate transporter 8 (MCT8) deficiency: ReTRIACt trial

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The poster can be accessed here.

About the ReTRIACt trial
The ReTRIACt trial (clinicaltrials.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. This trial was requested by the US FDA as pivotal for the New Drug Application submission for tiratricol. 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 four years of age and having demonstrated stable maintenance treatment with tiratricol, will be randomized to receive placebo or tiratricol 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 as follows: for participants in the placebo group, removal of tiratricol will lead to an increase of serum total T3

concentration above the ULN and requirement of rescue treatment with tiratricol, compared to those who continue to receive tiratricol.
For more information about MCT8 deficiency, please see www.mct8deficiency.com

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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) on October 9, 2023, based on existing clinical data. After a dialogue with the FDA, Egetis is conducting a small randomized, placebo-controlled pivotal 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 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.
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 after *Emcitate* submissions have been completed. *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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