PRESS RELEASE
06 April 2022 08:00:00 CEST

Egetis announces that the recruitment target has been met in the Emcitate® Triac Trial II study

Stockholm, Sweden, April 06, 2022. Egetis Therapeutics AB (publ) (Nasdaq Stockholm: EGTX) today announced that the recruitment target of 16 patients has been met in the Triac Trial II clinical study with Emcitate® (tiratricol) in patients with MCT8 deficiency.

Triac Trial II (clinicaltrials.gov identifier NCT02396459) is an ongoing international, open label, multi-center study in children with MCT8 deficiency, conducted in both Europe and North America, investigating neurocognitive effects of early intervention with *Emcitate* in very young (<30 months of age) patients. The first patient was dosed in December 2020. The primary endpoint is to evaluate the effects of *Emcitate* after 96 weeks of treatment as measured by the Gross Motor Function Measure (GMFM)-88 assessment and the Bayley Scales of Infant Development (BSID-III) Gross Motor Skill Domain score. The study will also evaluate the effect of *Emcitate* on specific motor milestones, such has holding head and sitting independently, as well as the effect on clinical and biochemical thyrotoxic features.
The recruitment target of 16 patients has been met in the Triac Trial II clinical study but to include additional patients who has already been identified but not yet screened, the trial will remain open for recruitment a few additional weeks. Results of Triac Trial II are expected in the first quarter of 2024 and are expected to be submitted post-approval to regulatory authorities.
Data from preclinical studies suggest that *Emcitate* restores abnormal neuronal development and myelination in animal models of MCT8 deficiency if given in early postnatal life. In the Triac Trial I study, effects on neurocognitive development were included as exploratory endpoints (in a subset of patients) andthe results indicate a potential for *Emcitate* to positively influence neurocognitive development when treatment is introduced early, with the largest increase in gross motor function seen in patients where treatment was started before the age of 4 years.

Nicklas Westerholm, CEO of Egetis, commented: "*We are very excited to have reached this important recruitment milestone for Triac Trial II. Although Triac Trial II is not required for regulatory approvals for Emcitate it remains an important study for establishing neurodevelopmental effects of early intervention with Emcitate. We would like to thank participating families, study staff and investigators for taking part in Triac Trial II.*"

As a result of fruitful regulatory interactions, 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* has been granted Rare Pediatric Disease Designation (RPD) which gives Egetis the opportunity to receive a Priority Review Voucher (PRV) in the US.

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The information was submitted for publication, through the agency of the contact persons set out above, on April 06, at 8:00 CET.

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 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 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-beta) in the US and for MCT8 deficiency in the EU. *Emcitate* has been granted Rare Pediatric Disease Designation (RPD) which gives Egetis the opportunity to receive a Priority Review Vouched (PRV) in the US.
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 EU for *Aladote* has been finalized after completed interactions with FDA, EMA and MHRA. *Aladote* has been granted ODD in the US and an application for ODD was submitted in Europe in the first quarter of 2021. There is an ongoing dialogue with EMA on the appropriate scope of the indication for an 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 announces that the recruitment target has been met in the Emcitate® Triac Trial II study