PRESS RELEASE
09 October 2023 07:00:00 CEST

Egetis submits marketing authorisation application for Emcitate for treatment of MCT8 deficiency to the European Medicines Agency

- Submission based on efficacy data from two studies in a total of 86 MCT8 deficiency patients with up to 6 years of treatment with Emcitate
- To date an accumulated 500 patient years of Emcitate treatment have been gathered in patients with MCT8 deficiency
- If approved, Emcitate would become the first treatment for MCT8 deficiency

Stockholm, Sweden, October 9, 2023. Egetis Therapeutics AB (publ) ("**Egetis**" or the "**Company**") (Nasdaq Stockholm: EGTX), today announced that the Company has submitted a marketing authorisation application (MAA) to the European Medicines Agency (EMA) for Emcitate (tiratricol) for the treatment of MCT8 deficiency. This is an important step towards bringing the first approved treatment for MCT8 deficiency to patients and a transformative milestone for the Company.

As agreed with the EMA, the MAA submission is based on results from Triac Trial I (Groeneweg et al. 2019), which showed statistically significant and clinically relevant treatment effects on key aspects of the disease, including reduction in serum thyroid hormone T3 concentrations, as well as improvements in bodyweight, heart rate and blood pressure. The submission is supported by real-world, retrospective data collected in a multi-centre cohort study in which patients were treated with Emcitate for up to six years (van Geest et al. 2022).

Nicklas Westerholm, CEO of Egetis, commented: *"MCT8 deficiency is a severely debilitating ultra-rare disease without any approved treatments. We are delighted to have reached this transformative milestone for the Company in our efforts to bring the first approved treatment of MCT8 deficiency to patients. We look forward to engaging with the EMA on our submission."*

Emcitate has been granted Orphan Drug Designation by the EMA for MCT8 deficiency, and will following approval by the European Commission be eligible for 10 years of market exclusivity within the EU. The median review time for marketing authorisation applications in the EU is around 13-14 months.

As agreed with the US FDA, Egetis is conducting a randomized, placebo-controlled pivotal study (ReTRIACt) in 16 evaluable patients to verify the results on thyroid hormone T3 levels seen in previous clinical trials and publications. The study started recruiting in July this year and patient recruitment is proceeding according to plan. Topline results are expected during the first half of 2024 and Egetis intends to submit a new drug application (NDA) in the USA for Emcitate in mid-2024 under the Fast-Track Designation granted by the FDA.

In addition to the completed and ongoing clinical trials with Emcitate, more than 180 patients in over 25 countries, are currently being treated with Emcitate as part of the Company's Expanded Access/ Named Patient Use/ Compassionate Use programs.

Links to clinical trials:
Triac Trial I
ReTRIACt

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References:
Groeneweg, S. et al. Lancet Diabetes Endocrinol. 2019; 7(9):695-706
Van Geest, F.S. et al. J. Clin. Endocrinol. Metab. 2022; 107(3):e1136-e1147

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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. Egetis submitted a marketing authorisation application (MAA) for *Emcitate* to the European Medicines Agency (EMA) in October 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

Egetis submits marketing authorisation application for Emcitate for treatment of MCT8 deficiency to the European Medicines Agency