PRESS RELEASE
13 February 2025 15:25:00 CET

European Commission approves Egetis' Emcitate® (tiratricol) as the first and only treatment for patients with MCT8 deficiency

Stockholm, Sweden, February 13, 2025. Egetis Therapeutics AB (publ) ("Egetis" or the "Company") (Nasdaq Stockholm: EGTX), today announced that the European Commission (EC) has approved Emcitate® (tiratricol) for the treatment of patients with monocarboxylate transporter 8 (MCT8) deficiency. Emcitate is the first and only medicine authorised in the EU to treat MCT8 deficiency. The full indication is: Emcitate is indicated for the treatment of peripheral thyrotoxicosis in patients with monocarboxylate transporter 8 (MCT8) deficiency (Allan-Herndon-Dudley Syndrome), from birth.

Nicklas Westerholm, CEO of Egetis, commented: *"We are proud of the European Commission approval of Emcitate, which marks the first and only approved treatment for patients with MCT8 deficiency. This approval represents the single most important milestone in Egetis' history and a major step forward in building a sustainable rare disease company. We are delighted to bring this much needed new treatment to patients.*

"I would like to thank all patients, parents, caregivers and investigators who have taken part in the comprehensive development program for Emcitate and all Egetis employees and collaborators for their dedicated and hard work, in particular the group of Prof. Dr. Edward Visser at the Erasmus University Medical Center, Rotterdam, The Netherlands.

"We look forward to initiating pricing and reimbursement processes and discussions in Europe and expect the first launch in the second quarter of 2025."

MCT8 deficiency is a rare, chronic and severely debilitating disease caused by mutations in the gene coding for the thyroid hormone transporter MCT8 protein. This prevents thyroid hormones from entering cells in the brain and delays brain development in individuals with MCT8 deficiency. This lack of thyroid hormone in the brain leads to severe intellectual and motor disability. Patients only rarely achieve independent sitting, and most will not be able to maintain head control.

At the same time, there is a build-up of thyroid hormones in other parts of the body, which can cause peripheral thyrotoxicosis manifesting itself in symptoms such as increased heart rate, weight loss, muscle weakness and restless sleep.

The approval of Emcitate is primarily based on Triac Trial I (clinicaltrials.gov identifier NCT02060474, Groeneweg et al. 2019), supported by The Erasmus University Medical Center Cohort Study (van Geest et al. 2022) and preliminary results of Triac Trial II (NCT0239645).

Triac Trial I was a single-arm, open-label trial conducted in children and adults treated with an individually adjusted dose of tiratricol for up to 12 months. Emcitate reduced the mean serum T3 concentration by more than 63% at month 12. All patients improved in at least one of the study endpoints: body weight, resting heart rate, or systolic blood pressure. The average number of premature atrial contractions (extra heartbeats) also decreased.

The most frequent side effects in patients treated with Emcitate were excessive sweating, irritability, anxiety and nightmares. These reactions usually occurred at the start of treatment and / or when the dose was increased, and generally resolved during treatment.

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The decision from the European Commission follows a positive recommendation from the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) on December 12, 2024. The EU approval is applicable to all 27 European Union member states, as well as Iceland, Norway, and Liechtenstein. Emcitate was granted Orphan Drug Designation (ODD) in 2017. Maintenance of ODD was recently confirmed by the Committee for Orphan Medicinal Products (COMP) of the EMA.

1. Groeneweg et al. 2019 Lancet Diabetes Endocrinol. 7(9):695-706.
2. van Geest et al. 2022 J Clin Endocrinol Metab. 107(3):e1136–e1147

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This information is information that Egetis Therapeutics is obliged to make public pursuant to the EU Market Abuse Regulation. The information was submitted for publication, through the agency of the contact persons set out above, at 2025-02-13 15:25 CET.

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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.

On February 13, 2025, the European Commission approved Egetis' Emcitate® (tiratricol) as the first and only treatment for patients with MCT8 deficiency

The Company's lead drug candidate tiratricol (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) tiratricol has shown highly significant and clinically relevant results on serum thyroid hormone T3 concentrations and secondary clinical endpoints. In June 2024, topline results were presented from the Phase 2 study, Triac Trial II, with tiratricol for the treatment of MCT8 deficiency. The study investigated a potential additional treatment effect on neurocognitive development in young children under 30 months with MCT8 deficiency. The study did not show a statistically significant improvement compared to historical controls.

After a dialogue with the FDA, Egetis is conducting a randomized, placebo-controlled pivotal study in at least 16 evaluable patients to verify the results on T3 levels seen in previous clinical trials and publications. As previously communicated, the Company will update the market as soon as recruitment closes, and at that time, the Company will also provide information on when to expect topline results and when the Company plans to submit the NDA application.

Tiratricol 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. Tiratricol 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 calmangafodipir (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. The design of a pivotal Phase IIb/III study (Albatross), with the purpose of applying for market approval in the US and Europe, has been finalized following interactions with the FDA, EMA and MHRA. The development program for calmangafodipir has been parked until tiratricol marketing authorization submissions for MCT8 deficiency have been completed in the EU and the USA. Calmangafodipir 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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