PRESS RELEASE
07 December 2022 08:00:00 CET

Egetis submits Expanded Access Program for Emcitate® in the USA

Stockholm, Sweden, December 7, 2022. Egetis Therapeutics AB (publ) (Nasdaq Stockholm: EGTX) today announced that the Company has submitted a protocol for an Expanded Access Program for *Emcitate* (tiratricol) in the USA, as requested by the Food and Drug Administration (FDA).

Emcitate is under development for the treatment of patients with monocarboxylate transporter 8 (MCT8) deficiency, a highly debilitating rare disease with no available treatment, but does not yet hold regulatory approval anywhere in the world. Until now, physicians treating MCT8-deficiency patients in the USA, who are not part of an ongoing clinical trial with *Emcitate*, have been able to request authorization to treat patients by submitting individual Investigational New Drug (IND) requests to the FDA. As the number of diagnosed MCT8-deficiency patients has increased, with a concomitant increase in applications for individual INDs, Egetis has decided to implement an Expanded Access Program.

Nicklas Westerholm, CEO of Egetis, commented: *"MCT8 deficiency is a devastating disease with no available approved treatment. Today more than 160 patients, in over 25 countries, are already being treated with Emcitate via Named Patient Use or Compassionate Use programs according to the local laws and regulations of each country, following a request by their treating physician. In the USA the number of diagnosed MCT8 deficiency patients continues to increase through enhanced disease awareness. By implementing our Expanded Access Program for Emcitate the administrative burden for treating physicians will be reduced, should they wish to prescribe Emcitate to MCT8 patients under their care."*

About FDA's Expanded Access program
The Food and Drug Administration (FDA) regulates the development and approval for marketing of medical products in the USA. Before regulatory approval, it is not normally possible to prescribe a pharmaceutical under development outside of clinical trials. However, in the case of a serious condition or disease for which there are no satisfactory alternatives, and where a clinical trial is not an option for a patient, a physician that wishes to prescribe an unapproved therapy may request permission through FDA's Expanded Access Program (EAP). EAPs are designed to give access to potential therapies before they are approved by the FDA and may include people not typically eligible for clinical trials. Additionally, FDA may ask a sponsor to consolidate expanded access for groups of patients under an existing IND when the agency has received a significant number of requests for individual patient expanded access to an investigational drug for the same use.

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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 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 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 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 mid 2024 and are expected to be submitted post-approval to regulatory authorities.
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.
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 for early 2023. *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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