PRESS RELEASE
13 December 2023 07:00:00 CET

Egetis provides update on disease awareness and the Expanded Access Program for tiratricol (Emcitate) in the USA

Stockholm, Sweden, December 13, 2023. Egetis Therapeutics AB (publ) ("**Egetis**" or the "**Company**") (Nasdaq Stockholm: EGTX), today provided an update on disease awareness of MCT8 deficiency and the Expanded Access Program (EAP) for tiratricol (*Emcitate*) for eligible patients with MCT8 deficiency in the USA.

MCT8 deficiency is an ultra-rare genetic condition that was described only in 2004 and Egetis is the first pharmaceutical company to develop a possible therapy for this disease. Consequently, the general understanding of the disease and its diagnosis is still very low, even among specialist physicians, and a large proportion of patients remain undiagnosed. The Company has focused its medical affairs activities on improving disease awareness and diagnosis, including participation and dialogues at scientific conferences, patient identification partnerships with genetic testing companies, Key Opinion Leader engagements, advisory boards and interactions with Patient Advocacy Groups. This has resulted in the identification of an additional 50 MCT8 deficiency patients in the USA, previously not diagnosed or known to the Company.
To make sure these new patients are given the opportunity to be included in the Expanded Access Program, the Company has partnered with AnovoRx (anovorx.com), a specialty pharmacy company focussed on rare diseases, to optimize the execution of and further broaden the outreach and implementation of the EAP for eligible MCT8 deficiency patients in the USA.

Nicklas Westerholm, CEO of Egetis, commented: *"I am very pleased to see that the dedicated efforts by our medical affairs team are bearing fruit, leading to identification of more than 50 additional MCT8 deficiency patients in the USA over such a short period of time. This makes me confident that the number of diagnosed MCT8 deficiency patients will continue to increase. Today over 190 patients, in more than 25 countries, are already being treated with tiratricol through various Managed Access Programs according to local laws and regulations of each country, following a request by their treating physician. Through broad implementation of our Expanded Access Program for tiratricol in the USA the administrative burden for treating physicians will be reduced, should they wish to prescribe tiratricol to MCT8 patients under their care. The EAP is also important for patients who finish the ReTRIACt trial, to secure continued access to the treatment. MCT8 deficiency is a severely debilitating ultra-rare disease without any approved treatments and tiratricol has the opportunity to become the first approved treatment of MCT8 deficiency."*

The EAP was submitted to the US Food and Drug Administration (FDA) in October 2022, on request by the FDA. Healthcare professionals interested in making a request for access to the EAP or learning more about the criteria for the program can visit www.clinicaltrials.gov, study NCT05911399. According to the FDA rules for Early Access Programs, any patient enlisted in an EAP should first be evaluated for participation in ongoing clinical trials.

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.

PRESS RELEASE
13 December 2023 07:00:00 CET

For further information, please contact:

Nicklas Westerholm, CEO
+46 (0) 733 542 062
nicklas.westerholm@egetis.com

Karl Hård, Head of Investor Relations & Business Development
+46 (0) 733 011 944
karl.hard@egetis.com

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.
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 provides update on disease awareness and the Expanded Access Program for tiratricol (Emcitate) in the USA