PRESS RELEASE
Egetis Therapeutics AB
Stockholm, Sweden, Jan 18, 2022

Egetis concludes that demonstrating treatment effects on T3 levels in MCT8-deficiency could provide a basis for marketing approval for Emcitate® in the US

- *Emcitate® (tiratricol) is the first potential treatment of MCT8 deficiency, a rare genetic disease with high unmet medical need and no available treatment*
- *In recent positive regulatory interactions, FDA acknowledges that a treatment effect on T3 levels and the manifestations of chronic thyrotoxicosis in MCT8-deficiency could provide a basis for marketing approval also in the US.*
- *An NDA in the US is targeted to be submitted in mid-2023 under the Fast Track Designation.*
- *A small, 30-day, placebo-controlled study in 16 treated patients, to be identified through the existing named patient program, will be conducted to verify the results on T3 levels seen in previous clinical trials and publications in a randomized controlled setting*
- *This is a major step towards a marketing application and increases the likelihood of success for Emcitate and the probability for Egetis to receive a US Rare Pediatric Disease Priority Review Voucher (PRV).*
- *Egetis will host a webcast today at 15:00 CET (9:00am ET)*

Stockholm, Sweden, January 18, 2022 - Egetis Therapeutics AB (publ) (Nasdaq Stockholm: EGTX) (the "Company") today announced that in recent regulatory interactions, the US Food and Drug Administration (FDA) acknowledges that demonstrating a treatment effect on thyroid hormone T3 levels and the manifestations of chronic thyrotoxicosis could provide a basis for marketing approval also in the US. Consequently, the Company now has an aligned regulatory strategy for EU and US. The Company intends to submit a New Drug Application (NDA) in the US for Emcitate® (tiratricol) for the treatment of monocarboxylate transporter 8 (MCT8) deficiency in mid-2023 under the Fast Track Designation granted by the FDA in October 2021. This follows the announcement in December 2021 of intention to submit the Marketing Authorisation Application (MAA) for Emcitate to the European Medicines Agency (EMA) based on existing clinical data on the manifestations of chronic thyrotoxicosis in MCT8 deficiency.

Data demonstrating significant and clinically relevant effects on T3 and manifestations of chronic thyrotoxicosis in MCT8 deficiency with Emcitate is already available from the Triac Trial I (Groeneweg et al. 2019), as well as a recently published long-term cohort study looking at treatment effects in 67 patients for up to 6 years (van Geest et al. 2021). To complete the clinical package for the US NDA, a randomized controlled study in 16 patients treated with Emcitate will be conducted to verify the results on T3, as demonstrated in the previous trials. Patients will be randomized to continued Emcitate treatment or placebo for 30 days or until meeting a rescue criterion of T3 level above ULN (upper limit of normal), whichever comes first. The primary endpoint of the study is the proportion of patients meeting the rescue criterion within the randomized treatment period.

"Today's announcement increases the likelihood of success for Emcitate and its potential to become the first approved treatment for MCT8 deficiency, a rare genetic disease, with high unmet medical need, and the probability for Egetis to receive a US Rare Pediatric Disease Priority Review Voucher (PRV) upon approval of the NDA.

We have now agreed with the FDA to perform a small study randomizing treated patients to continue treatment with Emcitate or to receive placebo for up to 30 days to verify our previous T3 results in a randomized controlled setting. It is well-established that the T3 levels in untreated MCT8 patients are significantly elevated, and we have previously shown that Emcitate is able to rapidly and durably normalize these levels. The primary source for patient selection will be through our existing named patient program. We target submission of the NDA for Emcitate in mid-2023 under the Fast Track Designation. This is a substantial opportunity for Egetis and the patients in the US suffering from MCT8 deficiency." said Nicklas Westerholm, CEO, Egetis Therapeutics.

In light of the revised regulatory strategy for submission in US and Europe (as earlier communicated), work is ongoing to confirm the content of the other components of the regulatory dossiers. The Company intends to communicate timelines during first half of 2022 for regulatory submissions of Emcitate globally.

The ongoing Triac Trial II will continue to further establish the effects of early intervention on the neurocognitive development aspects of the disease, previously seen in the Triac Trial I. Given the possibility to proceed with regulatory submissions in both EU and US prior to data available from the Triac Trial II and following discussions with regulatory agencies, Egetis will no longer conduct an interim analysis based on 48-week data but will perform the statistical analysis on the complete dataset after the full 96 weeks of treatment making the data more robust. Results are expected in Q1 2024 and data from the Triac Trial II is expected to be submitted post-approval to regulatory authorities shortly thereafter.

Emcitate holds Orphan Drug Designation (ODD) in both the EU and the US and was granted Rare Pediatric Disease Designation (RPD) in November 2020 and Fast Track designation in October 2021 by the US FDA.

Egetis will host a webcast today at 15.00 CET (9:00am ET). Please find call-in details and links below:

Weblink

https://tv.streamfabriken.com/egetis-therapeutics-presskonferens

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About Emcitate Clinical Trials

Results from Triac Trial I (clinicaltrials.gov identifier NCT02060474) were published in the *Lancet Diabetes & Endocrinology* in 2019 (Groeneweg et al. 2019), showing clinically relevant and highly significant results on serum T3 concentrations and secondary clinical endpoints following one-year treatment with Emcitate in 46 MCT8 deficiency patients of all ages. The clinical data published in the *Journal of Clinical Endocrinology & Metabolism* in October 2021 (van Geest et al. 2021) comes from the investigator-initiated real-life cohort study at 33 sites conducted by the Erasmus Medical Center, Rotterdam, The Netherlands, where the efficacy and safety of Emcitate was investigated in 67 patients with MCT8 deficiency treated with Emcitate for up to six years. The primary endpoint, the mean serum T3 concentrations, decreased significantly from baseline to the last visit. Several clinically relevant and highly significant improvements were reported among secondary endpoints covering key measurements of clinical complications of chronic peripheral thyrotoxicosis, replicating the findings from the Triac Trial I and confirming the long-term durability of the treatment effects. No drug-related serious adverse events were reported.

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 patients (<30 months of age). The first patient was dosed in December 2020 and recruitment is proceeding well and is expected to be completed during Q1 2022. Results are expected in Q1 2024.

References:
van Geest et al. *J Clin Endocrinol Metab* 2021 https://doi.org/10.1210/clinem/dgab750
Groeneweg et al. *Lancet Diabetes Endocrinol* 2019;7(9):695-706

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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 2022-01-18, 08:00 CET.

About Egetis Therapeutics

Egetis Therapeutics is an innovative, unique, and integrated pharmaceutical drug development company, focusing on projects in late-stage development for treatment of serious diseases with significant unmet medical needs in the orphan drug segment. The drug candidate Emcitate is developed as the first potential treatment for patients with MCT8 deficiency, a rare disease with high unmet medical need and no available treatment. Triac Trial I (Phase IIb) and a long-term real-life study have been completed with clinically relevant and highly significant results on serum T3 concentrations and secondary clinical endpoints. Triac Trial II is an ongoing study in very young MCT8 deficiency patients (<30 months of age) to further establish the effects of early intervention on the neurocognitive development aspects of the disease, previously seen in the Triac Trial I. Results are expected in Q1 2024. Egetis intends to submit a marketing authorisation application for Emcitate to the European Medicines Agency based on existing clinical data. Emcitate holds Orphan Drug Designation (ODD) in the US and EU and has been granted Rare Pediatric Disease Designation and Fast Track Designation by the US FDA. The drug candidate Aladote is a first in class drug candidate developed to reduce the risk of acute liver injury associated with paracetamol poisoning. A proof of principle study has been successfully completed and the design of the upcoming pivotal Phase IIb/III study for Aladote has been finalized after completed interactions with FDA, EMA and MHRA. Aladote has been granted Orphan Drug Designation in the US and an application for ODD was submitted in Europe in Q1 2021. There is an ongoing dialogue with EMA on the appropriate 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

EGETIS THERAPEUTICS

About MCT8 Deficiency

Monocarboxylate transporter 8 (MCT8) deficiency is a rare genetic disease with high unmet medical need and no available treatment, affecting 1:70,000 males. Thyroid hormone is crucial for the development and metabolic state of virtually all tissues. Thyroid hormone transport across the plasma membrane is required for the hormone's metabolism and intracellular action and is facilitated by thyroid hormone transporters, including MCT8. Mutations in the gene for MCT8, located on the X-chromosome, cause MCT8 deficiency, also called Allan-Herndon-Dudley syndrome (AHDS) in affected males. The resulting dysfunction of MCT8 leads to impaired transport of thyroid hormone into certain cells and across the blood-brain-barrier and disruption of normal thyroid hormone regulation. This leads to a complex pattern of symptoms with neurological developmental delay and intellectual disability, accompanied by strongly elevated circulating thyroid hormone concentrations which are toxic for tissues including the heart, muscle, liver and kidney and results in symptoms such as failure to thrive, cardiovascular stress, insomnia and muscle wasting. Most patients will never develop the ability to walk or even sit independently. At present there is no approved therapy available for the treatment of patients with MCT8 deficiency and the median life expectancy is only 35 years.