PRESS RELEASE
Egetis Therapeutics AB
Stockholm, Sweden, Dec 13, 2021

Egetis intends to submit a marketing authorisation application for Emcitate® to the European Medicines Agency based on existing clinical data

- *Egetis concludes, based on recent regulatory interactions, that available Triac Trial I data together with recently published long-term data are sufficient for a Marketing Authorisation Application in Europe*
- *Having all clinical data required for regulatory submission already at hand significantly reduces the remaining risk for Emcitate*
- *Revised submission timelines will be communicated as soon as all parts of the regulatory dossier are confirmed*
- *Egetis will host a webcast today at 15:00 CET (9:00am ET)*

Stockholm, Sweden, December 13, 2021 - Egetis Therapeutics AB (publ) (Nasdaq Stockholm: EGTX) today announced that after a pre-submission meeting held last week with concerned European regulatory agencies (EMA's Rapporteur and Co-Rapporteur), the Company concludes that the clinical data from the Triac Trial I (Groeneweg et al. 2019), together with the data from long-term treatment with Emcitate (tiratricol) for up to six years in 67 patients (van Geest et al. 2021) will be sufficient for a regulatory review of a Marketing Authorisation Application (MAA) to the European Medicines Agency for the treatment of monocarboxylate transporter 8 (MCT8) deficiency. Thus, all clinical data necessary for regulatory submission is already available. The ongoing Triac Trial II will continue to further establish the effects of early intervention on the neurocognitive development aspects of the disease.

"We are delighted with the outcome of the pre-submission meeting, giving us a clear path to our MAA submission, and subsequent regulatory review, based on existing clinical data. Having all clinical data required for regulatory submission already at hand significantly reduces the remaining risk for Emcitate and could also potentially enable an earlier submission in Europe than we had previously expected. This is a substantial opportunity for us and the European patients suffering from MCT8 deficiency. In parallel, as part of our efforts to make Emcitate available as soon as possible, we continue our dialogues with regulatory authorities in other jurisdictions to obtain their views on the available clinical data and its implications for regulatory submissions" said Nicklas Westerholm, CEO, Egetis Therapeutics.

In the light of the revised regulatory strategy for submission in Europe, work is ongoing to confirm the content of the other components of the regulatory dossier. As soon as this work is completed, the Company intends to communicate a firmer timeline for regulatory submission of Emcitate in Europe.

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 new 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 (tiratricol) 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. Taken together, these two clinical data sets will serve as the primary source of evidence for evaluation of the efficacy and safety of Emcitate in the MAA.

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 (<30 months of age) patients. The first patient was dosed in December 2020 and recruitment is proceeding well and is expected to be completed in Q1 2022. The ongoing Triac Trial II will continue to be conducted and remains important to further establish the effects of early intervention on the neurocognitive development aspects of the disease.

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-audiocast-2021
Participant dial in numbers
SE: +46856642705
UK: +443333009265
US: +16467224956

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 status in October 2021 by the US FDA.

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 2021-12-13, 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. A Phase IIb clinical trial and a long-term real-life study have been completed with significant and clinically relevant effects. A Phase IIb/III early intervention study has been initiated with the first patient dosed in Dec 2020. 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

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.