PRESS RELEASE
Egetis Therapeutics AB
Stockholm, Sweden, Jan 26, 2022

Egetis secures conditional acceptance for Emcitate® as brand name in the US

Stockholm, Sweden, January 26, 2022 - Egetis Therapeutics AB (publ) (Nasdaq Stockholm: EGTX) today announced that it has received a conditional acceptance from the US Food and Drug Administration (FDA) for the use of the brand name Emcitate® in the US. The company has earlier received the corresponding support from the European Medicines Agency for the use of Emcitate as the brand name in Europe. This is the best possible outcome towards securing a global brand name and the final approval process for the proprietary name Emcitate will be linked to the regulatory submission in the respective markets.

Egetis Therapeutics intends to submit a New Drug Application (NDA) in the US for Emcitate for the treatment of monocarboxylate transporter 8 (MCT8) deficiency mid-2023 under the granted Fast Track Designation.

"I am delighted that our pre-launch preparations run according to plan. It is very positive that also the FDA supports the brand name Emcitate since it is advantageous to have the same name globally. In this setting it is important to stress that we, in compliance with pharma legislations, will not promote Emcitate until marketing authorizations have been granted. However, there are other initiatives that we run already now with the aim to improve the situation for those affected by MCT8 deficiency," said Henrik Krook, VP Commercial Operations at Egetis Therapeutics.

"Our disease awareness initiatives will hopefully contribute to that more physicians understand how to diagnose and manage MCT8 deficiency. Starting this year, we will arrange disease awareness activities in connection with international congresses in neurology and endocrinology on top of an expanding digital presence through channels such as our already established platform www.mct8deficiency.com", he continued.

One of the disease awareness initiatives that the company launched 2021, the MCT8 deficiency focused "Cuddly toy campaign", has been shortlisted at the prestigious Pharmaceutical Marketing Society awards in the category of best health care professional disease awareness campaign. The awards ceremony will be held in London March 2022.

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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) investigating neurocognitive effects of early intervention with Emcitate. Results are expected in Q1 2024. Egetis intends to submit a marketing authorization application for Emcitate to the European Medicines Agency based on existing clinical data. Before submission for a New Drug Application in the US targeted in mid-2023, Egetis will conduct a randomized, placebo-controlled study in 16 treated patients to verify the results on T3 levels seen in previous clinical trials and publications. 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.