PRESS RELEASE
Egetis Therapeutics AB
Stockholm, Sweden, September 9, 2021

Egetis receives approval in Turkey for compassionate use of Emcitate® for MCT8 deficiency

Egetis Therapeutics AB (publ) (ticker: EGTX) today announced that it has received approval from the Turkish Medicines Agency for a compassionate use program of its leading candidate drug Emcitate to treat the rare genetic disease MCT8 deficiency.

Compassionate use and named patient programs are mechanisms to allow early access to a medicine prior to regulatory marketing approval, granted to pharmaceuticals under development for situations with high unmet medical needs and where no available treatment alternatives exist or are suitable.

Compassionate use programs, such as the current Turkish example, need to go through a detailed data review process by the national medicines agencies to become approved. Compassionate use programs are initiated by pharmaceutical companies and allow access to a group of patients in a certain country, when the treating physician considers it appropriate.

Outside Turkey, Emcitate is already being supplied on a named patient basis, following individual regulatory approval from the national regulatory agencies, to more than 120 patients in over 20 countries.

“I am happy that we now have a solution in place to be able to supply this promising therapy to the MCT8 patients also in Turkey under a compassionate use program. The approval from the Turkish Medicines Agency is another important step towards our goal to secure access to Emcitate to MCT8 patients across the globe and also verifies the substantial unmet medical need and interest to treat patients that suffer from MCT8 deficiency with Emcitate” said Nicklas Westerholm, CEO, Egetis Therapeutics.

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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 rare/niche 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 has been completed with significant and clinically relevant effects. A pivotal Phase IIb/III early intervention study has been initiated with the first patient dosed in Dec 2020 and interim results are expected in 2022. Emcitate holds Orphan Drug Designation (ODD) in the US and EU and was granted Rare Pediatric Disease Designation by the US FDA in November 2020. 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.

Egetis Therapeutics (STO: EGTX) is listed on the Nasdaq Stockholm main market. For more information, see http:// www.egetis.com/

About MCT8 Deficiency

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 monocarboxylate transporter 8 (MCT8). Mutations in the gene for MCT8, located at 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 severely 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 MCT8 deficiency.