PRESS RELEASE
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Egetis awarded Promising Innovative Medicine designation in the UK for Emcitate® (tiratricol) for treatment of MCT8 deficiency

Stockholm, Sweden, June 3, 2024. Egetis Therapeutics AB (publ) ("Egetis" or the "Company") (Nasdaq Stockholm: EGTX), today announced that it has received a Promising Innovative Medicine (PIM) designation from the Medicines and Healthcare products Regulatory Agency (MHRA) in the UK for the investigational drug Emcitate® (tiratricol) in the treatment of monocarboxylate transporter 8 (MCT8) deficiency.

Nicklas Westerholm, CEO of Egetis, commented: *"A Promising Innovative Medicine (PIM) designation is a recognition of Emcitate® (tiratricol) as a promising candidate drug in the UK for the treatment of patients with MCT8 deficiency. MCT8 deficiency is a debilitating condition, and we are delighted that the MHRA has recognized the potential of Emcitate® (tiratricol) for patients living with this disease. This is testament to our commitment to supporting patients with MCT8 deficiency in the UK."*

About Promising Innovative Medicine (PIM) designations
PIM designations are given to promising medicinal products, likely to offer major advantage for the treatment, diagnosis or prevention of a life-threatening or seriously debilitating condition, with the potential to address an unmet medical need. For the MHRA to grant a PIM Designation, the product must meet each of the following three criteria:

Criterion 1: The conditions should be life-threatening or seriously debilitating with high unmet need, meaning there is no method of treatment, diagnosis or prevention available, or existing methods have serious limitations.

Criterion 2: The medicinal product is likely to offer major advantage over methods currently used in the UK. Preliminary evidence should be submitted based on both non-clinical and clinical data.

Criterion 3: The potential adverse effects of the medicinal product are likely to be outweighed by the benefits, allowing for the reasonable expectation of a positive benefit risk balance.

About MCT8 deficiency
MCT8 deficiency, also called Allan-Herndon-Dudley Syndrome, is an ultra-rare genetic disorder. As one of the first X-linked neurodevelopmental syndromes to be described, MCT8 deficiency was later associated with the SLC16A2 gene in 2004. The core mechanism driving the pathogenesis of MCT8 deficiency is dysfunction of the thyroid hormone transporter, monocarboxylate transporter 8 (MCT8), due to a mutation in the SLC16A2 gene. MCT8 has a major role in regulating thyroid hormone levels, including the cellular uptake and efflux of tri-iodothyronine (T3) and thyroxine (T4). MCT8 serves an important role in the transport of thyroid hormone across the blood–brain barrier and is also widely expressed in tissues in the thyroid, liver, kidneys, heart, and muscle. This disrupted thyroid hormone homeostasis leads to neurological and endocrinological symptoms. The neurological symptoms are a consequence of too little T3 in the brain during neurodevelopment, whereas the endocrinological symptoms are due to elevated T3 in other organs outside the brain.

Parents of children with MCT8 deficiency usually report the pregnancy and birth as uneventful, and infants appear to develop as expected for the first few months of life. Early signs, such as inadequate head control due to hypotonia and failure to thrive may start to appear from around three months of age, but it usually takes a few more months before medical attention is sought. Recognizing symptoms and making an early diagnosis may help patients and their families.

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For further information about MCT8 deficiency, please see www.mct8deficiency.com

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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® (tiratricol) 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 randomized, placebo-controlled pivotal study in 16 evaluable patients to verify the results on T3 levels seen in previous clinical trials and publications. Egetis will update the market as soon as recruitment has been completed and at that point inform about the timing of availability of top-line results, and the expected timing of the subsequent NDA filing.

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® (calmangafodipir) 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. The design of a pivotal Phase IIb/III study (Albatross), with the purpose of applying for market approval in the US and Europe, has been finalized following interactions with the FDA, EMA and MHRA. The study start has been postponed until *Emcitate* marketing authorization submissions for MCT8 deficiency 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 awarded Promising Innovative Medicine designation in the UK for Emcitate® (tiratricol) for treatment of MCT8 deficiency