

Egetis Therapeutics to Feature MCT8 Deficiency on Behind the Mystery Airing on Lifetime in Honor of Rare Disease Day

Stockholm, Sweden, February 20, 2025 -- Egetis Therapeutics AB (publ) ("Egetis" or the "Company") (Nasdaq Stockholm: EGTX), an innovative pharmaceutical company specializing in late-stage development and commercialization of treatments for serious diseases with significant unmet medical needs in the orphan drug segment will be featured on Behind the Mystery™, a TV series in the U.S. that airs on the morning talk show The Balancing Act®. Sponsored by Egetis, this episode will raise awareness for Rare Disease Day, observed on February 28, and highlight MCT8 deficiency.

Spotlighting the real-life stories of MCT8 deficiency patients and their caregivers, the episode will feature the journey to diagnosis, burden of disease and need for treatment options, along with insights from Andrew J. Bauer, MD, Endocrinologist and Medical Director, Children's Hospital of Philadelphia; Larry A. Fox, MD, Jacksonville Chief, Pediatric Endocrinology and Diabetes, Nemours Children's Health; Jennifer Favre, Board Member, MCT8-AHDS Foundation; and Anny Bedard, President North America, Egetis Therapeutics. Egetis recently obtained marketing approval in the European Union for Emcitate® (tiratricol) for the treatment of MCT8 deficiency, but the drug is not approved in the U.S.

"Egetis is driven by three key pillars: Courage, Commitment and Collaboration," said Bedard. "We have the courage to tackle hard conditions like MCT8 deficiency, commitment to uphold the highest ethical standards, and value ongoing collaboration with MCT8-AHDS Foundation and The Balancing Act to heighten awareness and understanding of MCT8 deficiency. Currently, there are no approved medicines for MCT8 deficiency in the United States, creating a high unmet need we're diligently working to address."

"Educating people about the signs and symptoms of MCT8 deficiency, as well as the journey of patients like my son Colton is critically important," added Favre. "On Rare Disease Day and every day, MCT8 deficiency is a devastating disease in need of community recognition, support, resources, and, most of all, a treatment."

The Behind the Mystery MCT8 deficiency episode will air on February 24 and March 3. For more information about MCT8 deficiency and a link to a replay, please visit [MCT8deficiency.com](https://mct8deficiency.com).

About Behind the Mystery™

Behind the Mystery™ is a recurring series on The Balancing Act® television show devoted to advocating for rare and genetic diseases. The series clarifies the often-complex issues by offering easy-to-understand explanations from top experts in the field through partnerships with foundations and pharmaceutical companies. We amplify the voices of patients, families, and advocates affected by rare diseases via our national television platform. Tune in to Behind the Mystery weekdays at 7:30 a.m. (ET/PT) on Lifetime® and find all previously aired episodes on TheBalancingAct.com/rare.

About MCT8-AHDS Foundation

The MCT8-AHDS Foundation is made up of a group of passionate parents and 100% volunteer-based. We are striving to improve the quality of life and potential of our children; support hundreds of families around the world; share hope for a brighter future; learn from our collective experiences; and raise awareness of this ultra-rare condition with doctors, researchers, and the general public. Our children with MCT8-AHDS have many challenges to overcome, but we are stronger facing these together. To learn more, please visit <https://www.mct8.info/>.

About MCT8 Deficiency

Monocarboxylate transporter 8 (MCT8) deficiency is caused by mutations in the gene for MCT8. 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. Patients with MCT8 deficiency therefore have low concentrations of thyroid hormone in the central nervous system and increased concentrations of active thyroid hormone T3 in peripheral tissues. This leads to a complex pattern of symptoms with neurological developmental delay and intellectual disability. The elevated circulating thyroid hormone T3 concentrations are harmful for tissues not dependent on MCT8 for thyroid hormone transport, including the heart, muscle, liver and kidney and results in symptoms such as failure to thrive, cardiovascular stress, insomnia and muscle wasting. The consequence of the chronic thyrotoxicosis is considered to contribute to the increased mortality and shortened life expectancy observed in this patient group, with a median life expectancy of 35 years. At present there is no approved therapy available for the treatment of MCT8 deficiency in the U.S. For more information, please visit [MCT8deficiency.com](https://mct8deficiency.com).

About Rare Disease Day

Since its creation in 2008, Rare Disease Day has played a critical part in building an international rare disease community that is multi-disease, global, and diverse – but united in purpose.

Rare Disease Day is observed every year on 28 February (or 29 in leap years)–the rarest day of the year.

Rare Disease Day was set up and is coordinated by EURORDIS and 65+ national alliance patient organisation partners. Rare Disease Day provides an energy and focal point that enables rare diseases advocacy work to progress on the local, national and international levels.

For further information, please contact:

Nicklas Westerholm, CEO
+46 (0) 733 542 062
nicklas.westerholm@egetis.com

Karl Hård, Head of Investor Relations & Business Development
+44 (0) 7500 525 084
karl.hard@egetis.com

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.

On February 13, 2025, the European Commission approved Egetis' Emcitate® (tiratricol) as the first and only treatment for patients with MCT8 deficiency

The Company's lead drug candidate tiratricol (Emcitate®) 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) tiratricol has shown highly significant and clinically relevant results on serum thyroid hormone T3 concentrations and secondary clinical endpoints. In June 2024, topline results were presented from the Phase 2 study, Triac Trial II, with tiratricol for the treatment of MCT8 deficiency. The study investigated a potential additional treatment effect on neurocognitive development in young children under 30 months with MCT8 deficiency. The study did not show a statistically significant improvement compared to historical controls.

After a dialogue with the FDA, Egetis is conducting a randomized, placebo-controlled pivotal study in at least 16 evaluable patients to verify the results on T3 levels seen in previous clinical trials and publications. As previously communicated, the Company will update the market as soon as recruitment closes, and at that time, the Company will also provide information on when to expect topline results and when the Company plans to submit the NDA application.

Tiratricol 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. Tiratricol 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 calmangafodipir (Aladote®) 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 development program for calmangafodipir has been parked until tiratricol marketing authorization submissions for MCT8 deficiency have been completed in the EU and the USA. Calmangafodipir 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

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