PRESS RELEASE
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Egetis Completes the U.S. Rolling NDA Submission for Emcitate® (tiratricol) for Treatment of MCT8 Deficiency

Priority Review and Rare Pediatric Disease Priority Review Voucher requested

Anticipated regulatory decision in September 2026

Emcitate® (tiratricol) U.S. launch expected in Q4 2026, if approved

Stockholm, Sweden, January 29, 2026. Egetis Therapeutics AB (publ) ("Egetis" or the "Company") (NASDAQ Stockholm: EGTX), today announced that the Company has completed the rolling New Drug Application (NDA) submission to the U.S. Food and Drug Administration (FDA) for Emcitate® (tiratricol) for treatment of patients with MCT8 deficiency. MCT8 deficiency is a rare, devastating life-shortening disorder, for which there are currently no approved therapies in the U.S.

The rolling NDA submission was initiated in December 2025 with the submission of the non-clinical and quality (chemistry, manufacturing and controls, or CMC) sections and has now been finalized with the submission of the clinical sections containing a robust data package comprised of multiple clinical studies and real-world evidence, including the following studies:

- **Erasmus Medical Center (EMC) Survival Study**, an international real-world cohort study that compared all-cause mortality between tiratricol-treated and untreated male patients with MCT8 deficiency which found a significant survival benefit for tiratricol-treated patients vs untreated patients.
- **ReTRIACt study** provides randomized controlled evidence of tiratricol's pharmacodynamic effect on T3, the key biochemical driver of disease morbidity and mortality. The clear impact of tiratricol treatment was seen across initiation /titration, withdrawal and ultimately reinsertion of therapy, confirming results from previous studies in the program. Tiratricol lowered T3 in patients suffering from MCT8 deficiency and its withdrawal led to a statistically significant rise in T3.
- **Triac Trial I**, in which tiratricol treatment resulted in dose-dependent and sustained suppression of serum T3 from thyrotoxic baseline levels. These biochemical effects were accompanied by clinically meaningful improvements in cardiovascular and metabolic endpoints, including reductions in heart rate and systolic blood pressure, and improvement in weight. These biochemical and clinical effects were sustained with treatment for up to 3.5 years.
- **Triac Trial II**, conducted in infants and toddlers, found that tiratricol achieved substantial suppression of serum T3 using higher doses and that such high doses were generally well tolerated in this age group. Although neurocognitive outcomes did not significantly improve, the trial provided important evidence of preserved pharmacodynamic activity and safety profile of treatment in early life with treatment for up to 4.5 years.
- **The EMC Cohort Study,** which observed a durability of response for up to 6 years in real-world practice, reporting durable reductions in serum T3, accompanied by improvements in cardiovascular endpoints and weight. No tolerance or loss of effect occurred, further confirming long-term durability of response.

Emcitate® (tiratricol) was approved in the EU for the treatment of patients with MCT8 deficiency in February 2025 and subsequently launched in Germany in May 2025, as the first and only approved treatment for this rare and serious condition.

Emcitate® (tiratricol) has received multiple FDA designations including Orphan Drug, Fast Track and Breakthrough Therapy, reflecting its potential to address this serious condition with significant unmet medical need. The completed submission of Emcitate® (tiratricol) represents the first NDA for a treatment of MCT8 deficiency in the U.S. The FDA is expected to confirm within 60 days that the NDA submission is complete. As a designated Fast Track and Breakthrough Therapy, Egetis has requested Priority Review, and if granted, the FDA review should be completed within six months following the 60-day filing review period. Thus, Egetis anticipates regulatory decision on the NDA application in September 2026.

Emcitate® (tiratricol) has also received Rare Pediatric Disease Designation, and the Company will be eligible to receive a Priority Review Voucher upon NDA approval.

Nicklas Westerholm, CEO of Egetis, commented: "*With this completed NDA submission, Egetis has reached a critical milestone towards making the first treatment for MCT8 deficiency available in the U.S. I would like to thank patients and families who participated in clinical trials, the investigators and their teams, and our colleagues at Egetis for their diligence and dedication in helping us deliver Emcitate® (tiratricol) to patients affected by this rare genetic disease. As we continue the commercial launch of Emcitate® (tiratricol) in the EU, and partnership discussions for additional geographies, we also continue building U.S. capabilities ahead of a potential launch, with a focus on medical affairs and market access.*"

About MCT8 deficiency

MCT8 deficiency, also known as Allan-Herndon-Dudley syndrome (AHDS), is a rare, devastating life-shortening disorder. It is caused by mutations in the *SLC16A2* gene, located on the X-chromosome and presenting almost exclusively in males. Mutations in the *SLC16A2* gene lead to dysfunction of monocarboxylate transporter 8 (MCT8), a key thyroid hormone (TH) transporter. A complete or substantial lack of MCT8 transport capacity leads to a complex pattern with disrupted TH signaling. Tissues solely dependent on MCT8 for TH transport (MCT8-dependent), including the central nervous system, have a complete lack of or substantial reduction in TH signaling. Because neurons and endothelial cells that form the blood-brain-barrier are dependent on MCT8 for TH transport, MCT8 deficiency leads to impaired neurocognitive development with severe intellectual and motor disabilities. Patients only rarely achieve independent sitting and are not able to maintain head control. Concurrently, a dysfunctional MCT8 transporter causes disruption of peripheral TH homeostasis. Consequently, circulating T3 levels are significantly increased, leading to excessive TH signaling in tissues presenting other transporters than MCT8, leading to pronounced symptoms of chronic thyrotoxicosis. Patients with MCT8 deficiency have markedly shortened life expectancy. Thyrotoxicosis causes failure to thrive, low body weight, muscle wasting, cardiovascular morbidity, irritability, and insomnia. Thyrotoxicosis (of different etiologies) is associated with increased CV morbidity and mortality. The relationship between aberrant TH signaling and impacts on the cardiovascular system has been well demonstrated in experimental and clinical studies, most patients who are overtly hypo- or hyperthyroid experience cardiovascular complications and both, if left untreated, can accelerate the onset of symptomatic cardiovascular disease. TH excess increases cardiac output through its effects on stroke volume and heart rate, causes tachycardia, and leads to an increased risk of atrial fibrillation. Severe thyrotoxicosis can induce high-output heart failure, even in patients without underlying heart disease. A congestive circulatory state may arise from the increases in blood volume and heart rate, with associated pulmonary arterial hypertension. The clinical utility of therapeutic approaches to normalize TH levels is well established.

Chronic thyrotoxicosis is believed to contribute to the increased mortality in patients with MCT8 deficiency, mostly due to sudden (cardiac) death, pneumonia, and other infections.

Estimated incidence is 1 in 70,000 males with a median life expectancy of only 35 years and 30 % of patients die during childhood.

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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
+46 (0) 733 011 944
karl.hard@egetis.com

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 2026-01-29 07:00 CET.

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 developed for the treatment of patients with monocarboxylate transporter 8 (MCT8) deficiency, a highly debilitating rare disease with no available treatment. In February 2025 the European Commission approved Emcitate® as the first and only treatment for MCT8 deficiency in EU. Egetis initiated the launch of Emcitate® in Germany on May 1, 2025. Emcitate® (tiratricol) is not approved in the USA.

The Company initiated a rolling New Drug Application (NDA) for Emcitate® (tiratricol) in the USA in December 2025 targeting a complete NDA submission in early 2026 and anticipated completion of FDA's review process in the third quarter of 2026. Based on feedback from the FDA, the NDA for Emcitate® (tiratricol) for treatment of MCT8 deficiency will be based on currently available clinical data from Triac Trial I, Triac Trial II, ReTRIACt, EMC Cohort Study, EMC Survival Study and the US Expanded Access Program.

Tiratricol holds Orphan Drug Designation (ODD) for MCT8 deficiency and resistance to thyroid hormone 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 Breakthrough Therapy Designation and Rare Pediatric Disease Designation (RPDD) by the FDA, which gives Egetis the opportunity to receive a Priority Review Voucher (PRV) in the US, after approval.

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 development program for Aladote® has been parked 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 is listed on the Nasdaq Stockholm main market (Nasdaq Stockholm: EGTX).
For more information, see www.egetis.com

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Attachments

Egetis Completes the U.S. Rolling NDA Submission for Emcitate® (tiratricol) for Treatment of MCT8 Deficiency