PRESS RELEASE
23 October 2025 08:00:00 CEST

Egetis granted a rolling NDA review by FDA for Emcitate® (tiratricol) based on currently available clinical data

The outcomes of a successful pre-NDA meeting with the FDA are:

- **As agreed with the FDA, the rolling NDA submission will commence in December 2025, targeting a complete NDA submission in early 2026 and completion of the FDA review process in Q3 2026, if Priority Review is granted**
- **The NDA for Emcitate® (tiratricol) for treatment of MCT8 deficiency will be based on currently available clinical data**
- **The ReTRIACt trial will be closed and data accrued to date will be included in the NDA**

Egetis to host a webcast today at 10.00 CEST. Further details can be found below.

Stockholm, Sweden, October 23, 2025. Egetis Therapeutics AB (publ) ("Egetis" or the "Company") (NASDAQ Stockholm: EGTX), today announced that it has held a successful pre-New Drug Application (NDA) meeting with the U.S. Food and Drug Administration (FDA) in October 2025, for Emcitate® (tiratricol) for the treatment of MCT8 deficiency. The objective of the meeting was to seek FDA advice and agreement on the overall content to support the NDA submission, with a special focus on the clinical data package.

The Company has agreed with the FDA to submit a rolling NDA for Emcitate® (tiratricol), commencing in December 2025 targeting a complete NDA submission in early 2026 and completion of FDA's review process in the third quarter of 2026, if Priority Review is granted. As Emcitate® (tiratricol) has Breakthrough Therapy Designation, Egetis will request a priority review. Acceptance of the NDA for filing will be subject to FDA's review of the entire submission.

The pre-NDA meeting was held on the back of the Breakthrough Therapy Designation granted in July 2025, based on FDA's review of Egetis' analysis of survival data from the international cohort study conducted by Erasmus University Medical Center (the EMC Survival Study).

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. As recommended by the FDA, the Statistical Analysis Plan (SAP) for the ReTRIACt trial will be revised and the study will be closed. Topline results will be communicated during the fourth quarter of 2025. Data accrued to date will be included in the NDA.

Nicklas Westerholm, CEO of Egetis, commented: *"We are strongly encouraged by our recent dialogue with the FDA on Emcitate® (tiratricol), and we appreciate the Agency's collaborative spirit as we work to deliver the first therapy to patients with MCT8 deficiency as efficiently as possible in the US. Given the feedback from the FDA, we plan to commence a rolling NDA for tiratricol in December 2025 and targeting submission of the complete NDA in early 2026 and completion of FDA's review process in Q3 2026, if Priority Review is granted.*

"I would also like to thank patients, families and investigators involved in the development of Emcitate® (tiratricol) and Professor Edward Visser and his team at the Erasmus University Medical Center, Rotterdam, The Netherlands for our long-standing fruitful collaboration."

Information about webcast:
If you wish to participate via webcast please use the link below. Via the webcast you are able to ask written questions.
https://egetis.events.inderes.com/webcast-23-october-2025/register

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If you wish to participate via teleconference please register on the link below. After registration you will be provided phone numbers and a conference ID to access the conference. You can ask questions verbally via the teleconference.
https://events.inderes.com/egetis/webcast-23-october-2025/dial-in

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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 2025-10-23 08:00 CEST.

About MCT8 Deficiency

Monocarboxylate transporter 8 (MCT8) deficiency is a rare genetic disease with high unmet medical need. 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.

The European Commission has approved Emcitate® (tiratricol) for the treatment of peripheral thyrotoxicosis in patients with monocarboxylate transporter 8 (MCT8) deficiency (Allan-Herndon-Dudley syndrome), from birth. Emcitate® (tiratricol) is not approved for use in the United States.

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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 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.

After a dialogue with the FDA, Egetis is conducting a randomized, placebo-controlled withdrawal study to provide evidence of T3 normalization with a correlation to a clinically meaningful outcome. The Company plans to initiate the submission of the NDA application for tiratricol in 2025.

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

Attachments

Egetis granted a rolling NDA review by FDA for Emcitate® (tiratricol) based on currently available clinical data