PRESS RELEASE
19 December 2025 07:00:00 CET

Egetis Therapeutics initiates New Drug Application in the USA for Emcitate® (tiratricol) for MCT8 deficiency

Stockholm, Sweden, December 19, 2025. Egetis Therapeutics AB (publ) ("Egetis" or the "Company") (NASDAQ Stockholm: EGTX), today announced that the Company has initiated a rolling New Drug Application (NDA) submission to the U.S. Food and Drug Administration (FDA) for Emcitate® (tiratricol), its investigational drug for the treatment of MCT8 deficiency.

Emcitate® (tiratricol) was awarded Breakthrough Therapy Designation by the FDA in July 2025 (link to press release) and in October 2025 FDA granted a rolling NDA submission and review (link to press release) based on currently available data. In November, the Company presented positive topline results from its randomized controlled withdrawal study ReTRIACt (link to press release), representing the final piece in the clinical data package to be submitted. Consequently, the Company has now submitted the non-clinical and quality (chemistry, manufacturing and controls, or CMC) sections of the NDA to the FDA.

A rolling NDA allows completed sections of an NDA to be submitted and reviewed by the FDA on an ongoing basis, enabling earlier engagement with the FDA and supporting a more efficient review process.

In parallel Egetis is finalizing the clinical sections in the NDA to encompass the latest data and plans to complete the NDA submission early 2026, together with a request for Priority Review and thus an anticipated FDA approval in the third quarter of 2026.

Nicklas Westerholm, CEO of Egetis, commented: *"We are delighted to have initiated our rolling NDA for Emcitate® (tiratricol) with the FDA. The submission of the non-clinical and CMC sections of the NDA for Emcitate® (tiratricol) marks a significant milestone for Egetis and underscores our dedication to advancing Emcitate® (tiratricol) for the benefit of patients with MCT8 deficiency.*

"In parallel with the regulatory submission, Egetis is accelerating the build-out of its U.S. organization to support a potential commercial launch. The Company is establishing core U.S. capabilities across market access, medical affairs, and commercial operations to ensure launch readiness and appropriate engagement with U.S. stakeholders, including payers, healthcare providers, and patient advocacy organizations."

At the successful pre-NDA meeting on October 21, FDA confirmed that Egetis may submit the NDA for Emcitate® (tiratricol) based on currently available data and granted a rolling NDA submission and review. The NDA will be based on results from Triac Trial I, Triac Trial II, ReTRIACt, EMC Cohort Study, EMC Survival Study and the U.S. Expanded Access Program.

Emcitate® (tiratricol) has received multiple FDA designations including Orphan Drug, Fast Track and Breakthrough Therapy, reflecting its potential to address serious conditions with significant unmet medical need. Emcitate® (tiratricol) has also received Rare Pediatric Disease Designation and will be eligible for a Priority Review Voucher upon NDA approval, provided it occurs before October 1, 2026, or later if the U.S. Government prolongs the PRV program by enacting the Give Kids a Chance Act of 2025 (bill S. 932).

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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 2025-12-19 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 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 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

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