PRESS RELEASE
18 August 2025 08:30:00 CEST

Egetis reports progress towards US NDA submission for tiratricol

- *FDA awarded tiratricol Breakthrough Therapy Designation (BTD) in July 2025, based on the Agency's review of Egetis' analysis of the survival data set from the international real-world cohort study by the Erasmus University Medical Center*
- *There are 15 evaluable patients in the ReTRIACt study*
- *In light of the above, Egetis has submitted a pre-NDA meeting request to the FDA to discuss the contents and timing of the US NDA submission for tiratricol, including the role and position of the ReTRIACt study*
- *The Company plans to initiate the submission of the NDA for tiratricol to the FDA during 2025*

Stockholm, Sweden, August 18, 2025. Egetis Therapeutics AB (publ) ("Egetis" or the "Company") (NASDAQ Stockholm: EGTX), today announced that it has submitted a pre-NDA meeting (Type B) request to the US Food and Drug Administration (FDA) to discuss the contents and timing, including the role and position of the ReTRIACt study, of its New Drug Application (NDA) for tiratricol in the US, in light of the Breakthrough Therapy Designation (BTD) awarded on July 15, 2025, by the FDA. A BTD is awarded to programs that have shown preliminary evidence of clinical benefit that results in a meaningful improvement of key signs and symptoms compared to existing therapies.

Egetis today also provided an update on the recruitment status of the ReTRIACt study (clinicaltrials.gov identifier NCT05579327), evaluating withdrawal of tiratricol in patients with MCT8 deficiency. There are 15 evaluable patients in the trial. The Company plans to initiate the submission of the NDA to the FDA during 2025.

Nicklas Westerholm, CEO of Egetis, commented: *"The clinically meaningful evidence available from treating patients with MCT8 deficiency with tiratricol was recognized by the FDA Breakthrough Therapy Designation (BTD) awarded in July this year (link to Press Release). The BTD was granted based on the Agency's review of Egetis' detailed analysis of the entire survival data set from the international real-world cohort study by the Erasmus University Medical Center (for preliminary results, see Abstract in Ref. 1), demonstrating a significant and substantial improvement in survival in tiratricol treated vs untreated patients. Receiving a BTD this late in a clinical development program is very encouraging for the forthcoming NDA process, as these designations are typically awarded at an early stage in development.*

"We have submitted a pre-NDA meeting request and look forward to discussing the NDA submission for tiratricol with the Agency, to bring this potential treatment to patients also in the United States, as soon as possible. Tiratricol treatment for MCT8-deficiency is already included in clinical guidelines by the European Thyroid Association (Ref. 2) and Emcitate® (tiratricol) was approved for the treatment for MCT8 deficiency in the European Union in February 2025."

1: van der Most, F. et al. 2024 https://www.endocrine-abstracts.org/ea/0101/ea0101op-03-04

2: Persani, L. et al. 2024 https://etj.bioscientifica.com/view/journals/etj/13/4/ETJ-24-0125.xml

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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.

On February 13, 2025, the European Commission approved Emcitate® (tiratricol) as the first and only treatment for MCT8 deficiency in EU. Egetis launched Emcitate in the first country, Germany, on May 1, 2025.

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.

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 in 2025.

Tiratricol holds FDA Breakthrough Therapy Designation and 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 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 calmangafodipir has been parked until Emcitate marketing authorization submissions for MCT8 deficiency have been completed in the EU and the USA. Aladote has been granted ODD in the US and in the EU.

Egetis Therapeutics (Nasdaq Stockholm: EGTX) is listed on the Nasdaq Stockholm main market. For more information, see www.egetis.com

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Attachments

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