PRESS RELEASE
15 July 2025 08:00:00 CEST

Egetis receives FDA Breakthrough Therapy Designation for tiratricol for MCT8 deficiency

Stockholm, Sweden, July 15, 2025. Egetis Therapeutics AB (publ) ("Egetis" or the "Company") (NASDAQ Stockholm: EGTX), today announced that the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation for tiratricol, as a treatment for monocarboxylate transporter 8 (MCT8) deficiency.

Breakthrough Therapy Designation is a process designed to expedite the development and review of drugs that are intended to treat a serious condition and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over available therapy on a clinically significant endpoint(s).

Nicklas Westerholm, CEO of Egetis, commented: *"FDA Breakthrough Therapy Designation underscores both the urgent need for an effective treatment for patients with MCT8 deficiency and the clinically meaningful evidence demonstrated to date with tiratricol. We look forward to accelerating tiratricol through the development and regulatory process as rapidly as possible to bring this potential treatment to patients in the United States.*

"As the next step, the FDA has requested Egetis to submit a Type B meeting request. This meeting will be for a multidisciplinary comprehensive discussion of the tiratricol drug development program."

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 2025-07-15 08:00 CEST.

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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 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

Attachments

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