**PRESS RELEASE**
06 September 2024 08:15:00 CEST

# Egetis highlights seven abstracts related to MCT8 deficiency, and two abstracts related to tiratricol and RTH-beta, published ahead of the Annual Meeting of the European Thyroid Association

**Stockholm, Sweden, September 6, 2024.** Egetis Therapeutics AB (publ) ("**Egetis**" or the "**Company**") (Nasdaq Stockholm: EGTX), today highlighted seven abstracts related to MCT8 deficiency, and two abstracts related to tiratricol and resistance to thyroid hormone type beta (RTH-beta), which have been published ahead of the 46th Annual Meeting of the European Thyroid Association, to be held in Athens, Greece, on September 7-10, 2024.

**Links to clinical Abstracts related to tiratricol and MCT8 deficiency:**

1. Van der Most, F. et al. T3 analogue Triiodothyroacetic acid (Triac) treatment and survival in MCT8 deficiency: an international real-world cohort study
(this abstract was previously highlighted in a press release from Egetis issued on August 21, 2024)

2. Freund, M. et al. Effect of the T3 analogue Triac on patient-centered outcome measures in patients with MCT8 deficiency: post-hoc analysis of the international Triac Trial I
(this abstract was previously highlighted in a press release from Egetis issued on August 28, 2024)

**Links to preclinical Abstracts related to MCT8 deficiency:**

3. Alcaide Martin, M. et al. Thyroid hormone transporters Mct8/Oatp1c1 deficient mice exhibit increased seizure susceptibility together with an imbalanced hippocampal neurotransmission

4. Bárez-López, S. et al. Unravelling the Role of MCT8 in Early Brain Development

5. Richter, J.-J. et al. Thyroid hormone transporters Mct8 and Oatp1c1 exhibit cell-autonomous functions within the oligodendroglia cell lineage in the mouse CNS

6. Guillén Yunta, M. et al. Blood-brain barrier leakage and neurovascular unit ultrastructural alterations as new pathophysiological mechanisms for MCT8 Deficiency

7. Alevyzaki, A. et al. Thyroid hormone transporters Mct8 and Oatp1c1 are required for proper angiogenesis in the mouse CNS

**Links to clinical Abstracts related to tiratricol and RTH-beta:**

1. Moran, C. et al. Thyroid Hormone analogue (Triac) therapy in Resistance to Thyroid Hormone beta, reduces hyperthyroid symptoms, lowers circulating thyroid hormones and metabolic rate effectively, without adverse effects

2. Meima, M. et al. Molecular mechanisms of TRIAC in the treatment of resistance to thyroid hormone beta (RTH#)

None of the above-mentioned Abstracts had co-authors from Egetis.

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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 previous studies (Triac Trial I and a long-term real-life study) *Emcitate* has shown highly significant and clinically relevant results on serum thyroid hormone T3 levels and secondary clinical endpoints. Egetis submitted a marketing authorisation application (MAA) for *Emcitate* to the European Medicines Agency (EMA) in October 2023.

After a dialogue with the FDA, Egetis is conducting a randomized, placebo-controlled pivotal study in 16 evaluable patients to verify the results on T3 levels seen in previous clinical trials and publications. Egetis will update the market as soon as recruitment has been completed and at that point inform about the timing of availability of top-line results, and the expected timing of the subsequent NDA filing.

*Emcitate* 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. *Emcitate* 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 study start has been postponed 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 (STO: EGTX) is listed on the Nasdaq Stockholm main market. For more information, see www.egetis.com

**Attachments**

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