

New post-hoc analysis reports effects of tiratricol on patient-centered outcome measures in patients with MCT8 deficiency

- An Abstract by Dr M. Freund and co-authors from Erasmus Medical Center, Rotterdam, The Netherlands, published ahead of the Annual Meeting of the European Thyroid Association reports that treatment with the investigational drug tiratricol exerts beneficial effects on several patient-centered outcome measures in MCT8 deficiency.

Stockholm, Sweden, August 28, 2024. Egetis Therapeutics AB (publ) (“Egetis” or the “Company”) (Nasdaq Stockholm: EGTX), today announced the content of an Abstract by Matthijs Freund and co-authors, Erasmus Medical Center, Rotterdam, The Netherlands, published ahead of the 46th Annual Meeting of the European Thyroid Association, to be held in Athens, Greece, on September 7-10, 2024. In this analysis the authors performed post-hoc analyses on caregiver-reported patient-centered outcome measures in the Triac Trial I (1). In this trial, 40 patients with MCT8 deficiency completed 1 year of tiratricol treatment. At baseline, during clinical visits and at the end of the study, semi-structured interviews were held with caregivers on complex needs and daily care challenges, including motor skills, sleep problems, and seizure frequency. Moreover, parents were asked to report perceived changes in (thyrotoxic) symptoms such as increased sweating and reduction in salivary flow.

According to the Abstract, there were improvements upon tiratricol treatment reported by caregivers related to improved interaction (22/39), greater alertness (19/39), improved motor skills (12/39), improved head control (7/39), and improved sleep (8/39). For 1 patient, also negative changes were reported, specifically increased constipation and higher unsettledness. Compared to the baseline visit, excessive sweating was much less reported (48.6% vs. 8.1%) and less reduction in salivary flow was observed (30.6% vs. 22.2%) by the caregivers at the end study visit. Seizures and continence were reportedly unchanged. All parents (40/40) preferred to continue tiratricol treatment.

Results from the analysis will be presented as an oral presentation by Dr Matthijs Freund on September 9, 2024, at the 46th Annual Meeting of the European Thyroid Association, in Athens, Greece.

Link to Abstract: [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](#)

Link to presentation session: <https://apps.m-anage.com/eta2024/en-GB/pag/presentation/673266>

(1) Groeneweg, S. et al. Lancet Diabetes Endocrinol (2019) 7(9):695-706

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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

[New post-hoc analysis reports effects of tiratricol on patient-centered outcome measures in patients with MCT8 deficiency](#)