

Egetis announces EMA validation of Marketing Authorisation Application for *Emcitate* for the treatment of MCT8 deficiency

Stockholm, Sweden, October 27, 2023. Egetis Therapeutics AB (publ) (“**Egetis**” or the “**Company**”) (Nasdaq Stockholm: EGTX), today announced that its Marketing Authorisation Application (MAA) to the European Medicines Agency (EMA) for *Emcitate* (tiratricol) for the treatment of MCT8 deficiency has been validated. On October 9, 2023, Egetis announced the submission of the MAA. EMA performs a validation of the applications it receives. The objective is to make sure all essential regulatory elements required for scientific assessment are included in the application prior to the start of the review procedure. The *Emcitate* MAA is as of October 26, 2023, under review by the Committee for Medicinal Products for Human Use (CHMP).

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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* 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, based on existing clinical data.

After a dialogue with the FDA, Egetis is conducting a small randomized, placebo-controlled pivotal study in 16 patients to verify the results on T3 levels seen in previous clinical trials and publications. Egetis intends to submit a new drug application (NDA) in the US for *Emcitate* in mid 2024 under the Fast-Track Designation granted by FDA.

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* 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 and the design of the upcoming pivotal Phase I/III study with the purpose of applying for market approval in the US and Europe for *Aladote* has been finalized after completed interactions with FDA, EMA and MHRA and study start is planned after *Emcitate* submissions 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



PRESS RELEASE

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Attachments

[Egetis announces EMA validation of Marketing Authorisation Application for Emcitate for the treatment of MCT8 deficiency](#)