Egetis has decided to explore RTH-beta as the next indication for tiratricol (Emcitate®)

Stockholm, Sweden, December 4, 2024. Egetis Therapeutics AB (publ) (Nasdaq Stockholm: EGTX) today announced that the Company has chosen Resistance to Thyroid Hormone beta (RTHβ) as the next indication to be developed for its investigational drug tiratricol (Emcitate®). To this end, the Company is contemplating to support a multicentered investigator-initiated Phase 2 study in patients with RTHβ in collaboration with academia.

RTHβ is a rare genetic disorder with high unmet medical need and no approved treatment, affecting 1-2 individuals per 40,000 live births. Thyroid hormone is crucial for the development and metabolic state of virtually all tissues and acts through binding to nuclear thyroid hormone receptors resulting in transcription of a range of hormone responsive genes. There are two main subtypes of thyroid hormone receptors in the body (alpha and beta), preferentially expressed in different tissues. RTHβ is caused by mutations in the thyroid hormone receptor beta gene and leads to impaired thyroid hormone signaling in tissues dependent on this receptor subtype. The disease affects both females and males equally. Clinical manifestations of RTHβ include a mix of symptoms of thyrotoxicosis and hypothyroidism in different tissues, including goiter, hepatic steatosis and dyslipidemia, impaired hearing and color vision, neurocognitive dysfunction and cardiovascular stress. Recently it has been shown that patients with RTHβ have reduced survival and increased cardiovascular morbidity (Okosieme et al. 2023, Campi et al. 2024).

RTHβ is a distinct indication, with no overlap in patient populations, to MCT8 deficiency. In 2022 Egetis obtained Orphan Drug Designations for RTHβ for tiratricol (Emcitate®) in the USA and EU. A presentation about RTHβ and the unmet medical need will be given at the Egetis' Investor Day on December 18, 2024.

Further information on the specific plans to develop tiratricol (Emcitate®) in RTHβ will be communicated when finalized.

References:

1. Okosieme, O. et al. 2023. Cardiovascular morbidity and mortality in patients in Wales, UK with resistance to thyroid hormone β (RTHβ): a linked-record cohort study. Lancet Diabetes Endocrinol. 11(9):657-666
2. Campi, I. et al. 2024. Increased cardiovascular morbidity and reduced life expectancy in a large Italian cohort of patients with resistance to thyroid hormone β (RTHβ). Eur. J. Endocrinol. 191(4):407-415

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
+44 (0) 7500 525 084
karl.hard@egetis.com

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 tiratricol (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) tiratricol has shown highly significant and clinically relevant results on serum thyroid hormone T3 concentrations and secondary clinical endpoints. In June 2024, topline results were presented from the Phase 2 study, Triac Trial II, with tiratricol for the treatment of MCT8 deficiency. The study investigated a potential additional treatment effect on neurocognitive development in young children under 30 months with MCT8 deficiency. The study did not show a statistically significant improvement compared to historical controls.

Egetis submitted a marketing authorisation application (MAA) for tiratricol 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 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.

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 calmangafodipir (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. 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 tiratricol marketing authorization submissions for MCT8 deficiency have been completed in the EU and the USA. Calmangafodipir 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