PRESS RELEASE
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New publication highlights meaningful clinical benefits of Emcitate® (tiratricol) in treating Resistance to Thyroid Hormone Beta

Stockholm, Sweden, December 4, 2025. Egetis Therapeutics AB (publ) ("Egetis" or the "Company") (NASDAQ Stockholm: EGTX), today announced the publication of a scientific article describing meaningful clinical benefits associated with Emcitate® (tiratricol) treatment of patients with Resistance to Thyroid Hormone Beta (RTH-beta) in the *Journal of Clinical Endocrinology & Metabolism*. As previously communicated, Egetis has decided to explore RTH-beta as the next indication for Emcitate® (tiratricol).

RTH-beta is a rare inborn genetic disorder caused by mutations in one of the two subtypes of thyroid hormone receptors that leads to impaired thyroid hormone signaling in affected tissues. Clinical manifestations of RTH-beta 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. The incidence of RTH-beta is estimated to be between 1 per 20,000–40,000 live births. At present there is no approved therapy available for patients suffering from RTH-beta.

Emcitate® (tiratricol) exhibits greater affinity to thyroid hormone receptor (TR) -beta mutants than endogenous T3 hormone and demonstrated efficacy in restoring signaling in TR-beta mutation models *in vitro*. Emcitate® (tiratricol) received orphan drug designation (ODD) for RTH-beta from the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) in 2022. RTH-beta is a distinct indication, with no overlap in patient populations with MCT8 deficiency, and represents a longer-term label expansion opportunity for Emcitate® (tiratricol). Emcitate® (tiratricol) is not currently licensed for treatment of RTH-beta.

This independently led retrospective cohort study of 8 adult RTH-beta patients was designed to investigate whether Emcitate® (tiratricol) treatment – referred to as TRIAC in the article – can address the unique clinical challenges of the disease, in which hyperthyroidism in certain tissues coincides with a hormone-resistant, hypothyroid state in other organs. Patients were assessed in the UK's National Institute for Health and Care Research (NIHR) before and during Emcitate® (tiratricol) treatment. A validated, patient-completed questionnaire was employed to rate the severity of thyrotoxic symptoms such as nervousness, sweating, heat intolerance, hyperactivity, tremor, weakness, tachycardia, diarrhea, appetite, and impairment of daily function. The study concluded that Emcitate® (tiratricol) treatment relieved hyperthyroid symptoms, lowered resting energy expenditure and the levels of circulating thyroid hormones, while avoiding negative effects on liver response to hormones or worsening cardiac thyromimetic activity. Emcitate® (tiratricol) treatment was well-tolerated.

Reference: C. Moran, J. Martin-Grace, et al. TRIAC therapy relieves hyperthyroid symptoms, lowering T4, T3 and metabolic rate in Resistance to Thyroid Hormone beta. The Journal of Clinical Endocrinology & Metabolism, 2025, https://doi.org/10.1210/clinem/dgaf583.

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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 developed for the treatment of patients with monocarboxylate transporter 8 (MCT8) deficiency, a highly debilitating rare disease with no available treatment. In February 2025 the European Commission approved Emcitate® as the first and only treatment for MCT8 deficiency in EU. Egetis initiated the launch of Emcitate® in Germany on May 1, 2025. Emcitate® (tiratricol) is not approved in the USA.

The Company has agreed with the FDA to submit a rolling NDA for Emcitate® (tiratricol), commencing in December 2025 targeting a complete NDA submission in early 2026 and anticipated completion of FDA's review process in the third quarter of 2026. Based on feedback from the FDA, the NDA for Emcitate® (tiratricol) for treatment of MCT8 deficiency will be based on currently available clinical data from Triac Trial I, Triac Trial II, ReTRIACt, EMC Cohort Study, EMC Survival Study and the US Expanded Access Program.

Tiratricol holds Orphan Drug Designation (ODD) for MCT8 deficiency and resistance to thyroid hormone 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 Breakthrough Therapy Designation and Rare Pediatric Disease Designation (RPDD) by the FDA, which gives Egetis the opportunity to receive a Priority Review Voucher (PRV) in the US, after approval.

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 Aladote® has been parked 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 is listed on the Nasdaq Stockholm main market (Nasdaq Stockholm: EGTX).
For more information, see www.egetis.com

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Attachments

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