

International retrospective cohort study published on "molecular mechanisms underlying the effectiveness of tiratricol in patients with resistance to thyroid hormone beta (RTH-beta)"

Stockholm, Sweden, October 16, 2026. Egetis Therapeutics AB (publ) ("Egetis" or the "Company") (NASDAQ Stockholm: EGTX), today announced the publication (Liao, Marelli et al. 2026; [https://www.thelancet.com/journals/ebiom/article/PIIS2352-3964\(26\)00396-8/fulltext](https://www.thelancet.com/journals/ebiom/article/PIIS2352-3964(26)00396-8/fulltext)) of the largest retrospective cohort study to date of patients with resistance to thyroid hormone beta (RTH-beta) treated with tiratricol (triiodothyroacetic acid or Triac), providing an integrated characterization of the effectiveness and molecular mechanisms of tiratricol in RTH-beta.

The study, led by Erasmus Medical Center, Rotterdam, The Netherlands, included 28 patients aged 0.4 to 73 years, with 18 different thyroid hormone receptor beta (*THRB*) mutations, treated at six centres in four countries. Median treatment lasted about four years (50 months). Tiratricol lowered thyroid stimulating hormone (TSH) and free thyroxine (FT4). Mean FT4 decreased 37% ($p < 0.0001$) and mean TSH decreased by 43% ($p < 0.0001$) together with reductions in heart rate of 10 bpm ($p = 0.014$), known to be associated with the lowering of thyrotoxicosis. The authors conclude that the study provides further support to the recommendations for tiratricol treatment in the guidelines by the European Thyroid Association and may support prospective clinical trials.

Nicklas Westerholm, CEO of Egetis Therapeutics, said: "We are encouraged to see these important data published in a leading peer-reviewed journal. As the largest retrospective study of tiratricol in patients with RTH-beta conducted to date, the findings provide valuable insights of both the potential clinical effects and underlying mechanism of action of tiratricol across a broad range of *THRB* mutations. Together with the significant unmet medical need in RTH-beta, these results further strengthen the scientific rationale for continued development of tiratricol in this patient population."

As previously communicated, work is ongoing at Egetis to finalize the development plan for tiratricol in RTH-beta where scientific advisory boards have been convened and preparations for regulatory interactions are ongoing. RTH-beta is a distinct indication, with no overlap in patient populations with MCT8 deficiency. Tiratricol is not currently licensed for treatment of RTH-beta.

About resistance to thyroid hormone beta

Resistance to Thyroid Hormone Beta (RTH-beta) is a rare inherited disorder caused by mutations in the *THRB* gene, leading to impaired thyroid hormone receptor beta (TR-beta) signalling. RTH-beta occurs equally in males and females. As a result of impaired TR-beta-mediated feedback regulation of the hypothalamic-pituitary-thyroid (HPT) axis, patients present with elevated circulating thyroid hormone levels, leading to clinical manifestations affecting multiple organ systems.

Patients with RTH-beta may present with goiter, as well as cardiovascular, metabolic, and neurocognitive manifestations. Recent evidence from independent patient cohort studies suggests increased cardiovascular morbidity in patients with RTH-beta. These studies report reduced life expectancy compared with the general population, with higher free thyroid hormone T4 (FT4) concentrations being associated with mortality and/or major cardiovascular events.

The incidence of RTH-beta is estimated to be between 1 per 20,000-40,000 live births. There are currently no approved therapies for RTH-beta, representing a significant unmet medical need. Tiratricol has been granted Orphan Drug Designation for the treatment of RTH-beta in both the United States and the European Union.

Reference: W. Liao, F. Marelli, et al. Molecular mechanisms underlying the effectiveness of triiodothyroacetic acid (Triac) in patients with resistance to thyroid hormone # (RTH#): a multicentre, international, retrospective cohort study, [eBioMedicine 2026; 133: 106512](#)

Disclaimer: Erasmus Medical Center is Egetis' licensor and receives royalties from Egetis; certain authors have received fees from Egetis. Egetis had no influence on the conduct or analysis of the study.

About Egetis Therapeutics

Egetis Therapeutics is a commercial-stage pharmaceutical company focused on developing and delivering innovative therapies for patients with rare diseases with significant unmet medical need. Combining scientific and clinical expertise with integrated late-stage development, regulatory and commercial capabilities and manufacturing expertise, Egetis is committed to delivering treatments and dedicated support to underserved patient communities.

The Company's lead product, EMCITATE® (tiratricol), is the first and only approved treatment for MCT8 deficiency, a rare and life-shortening genetic disorder, with marketing authorizations in the European Union and in the United States.

Headquartered in Stockholm, Sweden, with operations in the U.S. and Europe, Egetis is building a sustainable global rare disease company.

For more information, visit egetis.com and follow the Company on LinkedIn.

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Attachments

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