PRESS RELEASE
18 December 2024 10:20:00 CET

Egetis highlights the positive CHMP opinion for Emcitate®, recent major milestones, and gives a corporate update at Investor Day in Stockholm today

Stockholm, Sweden, December 18, 2024. Egetis Therapeutics AB (publ) ("Egetis" or the "Company") (Nasdaq Stockholm: EGTX) is today hosting an Investor Day in Stockholm, Sweden. The event will feature presentations by Professor Edward Visser, Erasmus MC, Rotterdam, The Netherlands on the recent advances with tiratricol in monocarboxylate transporter 8 (MCT8) deficiency and Professor Aled Rees, Cardiff University, UK, on resistance to thyroid hormone beta (RTH-beta), the unmet medical need and the potential opportunity for Emcitate® in this disease. In addition, members of Egetis' management team will highlight the significant progress made by Egetis towards marketing approvals of Emcitate® (tiratricol), including the positive CHMP opinion for Emcitate®, an update of the status of the ReTRIACt trial, and the prelaunch activities and commercialization plans in the EU. Nicklas Westerholm, CEO of Egetis, will also present the Company's near-term strategic objectives and long-term ambitions.

Please follow this link to attend the webcast (no preregistration required):

https://www.redeye.se/events/1061014/egetis-therapeutics-investor-day

Agenda

Time (CET /ET)	Subject	Presenter(s)
15:00/9.00am	Welcome, CHMP opinion & corporate update	Nicklas Westerholm, CEO
15:10/9.10am	MCT8 deficiency: recent advances with tiratricol	Prof. Edward Visser, Erasmus MC, NL
15:35/9.35am	Q&A	Visser & Westerholm
15:45/9.45am	Global launch preparations	Henrik Krook, Raymond Francot, Henna Oittinen-Corbinelli, Peter Verwaijen
16:20/10.20am	Q&A	Krook, Francot, Oittinen-Corbinelli, Verwaijen, Westerholm
16:30/10.30am	Break	
16:50/10.50am	US regulatory pathway & ReTRIACt study	Westerholm

17:00/11.00am	US opportunity for *Emcitate*	Anny Bedard, Ann-Marie Redmond
17:15/11.15am	Q&A	Bedard, Redmond, Westerholm
17:25/11.25am	RTH-beta and the unmet medical need	Prof. Aled Rees, Cardiff University, UK
17:50/11.50am	Q&A	Rees & Westerholm
17:55/11.55am	Concluding remarks	Mats Blom, Chairman of the Board
18:00/12.00pm	Ends	

Nicklas Westerholm, CEO of Egetis, commented: *"The recommendation from the European Medicines Agency to grant marketing authorization for Emcitate® is the single most important milestone in Egetis' history and a major step forward in building a sustainable rare disease company. I'm delighted to share our progress and plans with the investment community today and look forward to bringing the first approved medicine to MCT8 deficiency patients in the EU, pending the European Commission grants the marketing authorization."*

On December 12, 2024, the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) adopted a positive opinion, recommending the granting of a marketing authorisation for Emcitate®, intended for the treatment of MCT8 deficiency (1). The European Commission, which grants central marketing authorisations in the European Union (EU), will review the CHMP recommendation and is expected to make a final decision within 67 days. If approved, Emcitate® will become the first approved drug which addresses MCT8 deficiency.

During the Investor Day the Company will also give an update on the progress made in the ReTRIACt trial, which is pivotal for the New Drug Application (NDA) in the US. So far, 18 patients have been included, of which 8 patients have completed the randomized phase, 1 patient is in the randomized phase and 4 patients are in the run-in period. In January, 4 additional patients are planned for screening.

There is a continued large and increasing interest from physicians to treat patients suffering from MCT8 deficiency with Emcitate® (tiratricol), and it is already prescribed as part of different Managed Access Programs to patients in over 25 countries. In total, around 230 patients are now being treated, and more and more patients are gaining access to treatment.

Egetis' strategy to build a sustainable rare-disease company also explores opportunities to extend the use of Emcitate® into other indications, like RTH-beta, which is a separate condition, with a non-overlapping patient population to MCT8 deficiency.

(1) https://www.egetis.com/mfn_news/egetis-receives-positive-chmp-opinion-for-emcitate-tiratricol-for-the-treatment-of-mct8-deficiency/

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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 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.

On December 12, 2024, the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) adopted a positive opinion for Emcitate® (tiratricol) for the treatment of MCT8 deficiency.

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

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Attachments

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