PRESS RELEASE
Egetis Therapeutics AB
Stockholm, Sweden, February 18, 2022

FDA grants Orphan Drug Designation to Emcitate® for treatment of Resistance to Thyroid Hormone type beta (RTH-β)

Stockholm, Sweden, February 18, 2022. Egetis Therapeutics AB (publ) (ticker: EGTX) today announced that the U.S. Food and Drug Administration (FDA) has granted an Orphan Drug Designation (ODD) to the Company's leading candidate drug Emcitate® (tiratricol) for the treatment of resistance to thyroid hormone type beta (RTH-β).

Emcitate is Egetis Therapeutics' lead candidate drug, in Phase III clinical development targeting marketing applications in the US and Europe in 2023 for the treatment of MCT8 deficiency, a rare genetic disease with high unmet medical need and no available treatment. The ODD granted today for the treatment of RTH-β is a direct result of the Company´s efforts on indication expansion of the *Emcitate* program into related but distinct conditions.

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

"We are very proud to receive another Orphan Drug Designation (ODD) for *Emcitate*. A potential indication expansion into thyroid hormone resistance could add substantial value to and extend the lifecycle of the *Emcitate* program. The new ODD for the treatment of RTH-β confirms the high unmet medical need and the potential role for *Emcitate* to treat this condition. This further encourages us to continue to explore the potential development path to market approval also for this disease, in which we could leverage our existing capabilities and expertise. RTH-β represents a significant unmet medical need, and we remain committed in our efforts to make available therapies to rare disease patients who have no or few treatment options today" said Nicklas Westerholm, CEO, Egetis Therapeutics.

Emcitate previously holds ODD in both the EU and the US for the treatment of MCT8 deficiency and was granted Rare Pediatric Disease Designation (RPD) in November 2020 and Fast Track status in October 2021 by the US FDA.

The Orphan Drug Designation Program provides orphan status to drugs and biologics which are defined as those intended for the safe and effective treatment, diagnosis or prevention of rare diseases/disorders that affect fewer than 200,000 people in the US. Orphan Drug Designation qualifies sponsors for incentives, including tax credits for qualified clinical trials, exemption from user fees, and potential seven years of market exclusivity after approval. More information about rare diseases and the Orphan Drug Designation Program is available on www.fda.gov.

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The information was submitted for publication, through the agency of the contact person set out above, at 2022-02-18, 11:30 CET.

About Resistance to thyroid hormone (RTH-β)

RTH-β (Resistance to thyroid hormone) is a rare genetic disorder with high unmet medical need and no approved treatment, affecting 1:20,000-40,000 individuals. Thyroid hormone is crucial for the development and metabolic state of virtually all tissues and acts through binding to a nuclear receptor resulting in transcription of a range of hormone responsive genes. There are two subtypes of thyroid hormone receptors in the body (alpha and beta), preferentially expressed in different tissues. RTH-β is caused by mutations in the thyroid hormone receptor beta and leads to impaired thyroid hormone signaling in tissues dependent on this receptor subtype. Clinical manifestations of RTH-β 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. RTH-β affects both genders equally and normally displays an autosomal dominant inheritance pattern. Homozygous disease is extremely rare and results in pronounced symptoms normally leading to death during early childhood.