12 December 2024
EMA/49758/2025
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Emcitate

tiratricol

On 12 December 2024, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Emcitate[2], intended for the treatment of MCT8 deficiency (Allan-Herndon-Dudley syndrome).

The applicant for this medicinal product is Rare Thyroid Therapeutics International AB.

Emcitate will be available as 350 µg dispersible tablets. The active substance of Emcitate is tiratricol, a thyroid hormone (ATC code: H03AA04). Tiratricol is an analogue of a naturally circulating metabolite of the active thyroid hormone T3. MCT8 is a specific thyroid hormone transporter. While T3 and T4 thyroid hormones rely on MCT8 to enter several tissues such as the brain, tiratricol can enter cells independently of MCT8. Once inside cells, tiratricol activates the thyroid hormone receptor in a similar way to endogenous T3.

The benefits of Emcitate are its ability to reduce the mean serum T3 concentration and alleviate symptoms of peripheral thyrotoxicosis in patients with MCT8 deficiency (Allan-Herndon-Dudley syndrome) after 12 months of treatment. The most common side effects are excessive sweating, irritability, anxiety and nightmares.

Emcitate is a hybrid medicine[3] of Téatrois, which has been authorised in France. Emcitate contains the same active substance as Téatrois but has a different indication.

The full indication is:

Emcitate is indicated for the treatment of peripheral thyrotoxicosis in patients with monocarboxylate transporter 8 (MCT8) deficiency (Allan-Herndon-Dudley Syndrome), from birth.

Treatment should be initiated and monitored by physicians who are experienced in the management of

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion
[2] This product was designated as an orphan medicine during its development. EMA will now review the information available to date to determine if the orphan designation can be maintained
[3] Hybrid applications rely in part on the results of pre-clinical tests and clinical trials for a reference product and in part on new data.

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patients with rare genetic disorders such as MCT8 deficiency.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.