Amsterdam, 12 October 2023
EMA/CHMP/458203/2023EMA/CHMP/458203/2023
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Agamree

vamorolone

On 12 October 2023, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Agamree[2], intended for the treatment of Duchenne muscular dystrophy (DMD). The applicant for this medicinal product is Santhera Pharmaceuticals (Deutschland) GmbH.

Agamree will be available as a 40 mg/ml oral suspension. The active substance of Agamree is vamorolone, a dissociative corticosteroid (ATC code: not yet assigned) that selectively binds to the glucocorticoid receptor, triggering anti-inflammatory effects. Vamorolone also inhibits mineralocorticoid receptor activation by aldosterone. The precise mechanism by which Agamree exerts its therapeutic effects in patients with DMD is unknown.

The benefits of Agamree are that it reduces the time it takes to stand from a supine position and improves walking ability, as assessed in a pivotal study in ambulant children with DMD. The most common side effects are Cushingoid features, vomiting, weight gain and irritability.

The full indication is:

> AGAMREE is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients aged 4 years and older.

Agamree should only be initiated by specialist physicians with experience in the management of Duchenne muscular dystrophy.

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.

[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

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