15 October 2020
EMA/CHMP/529305/2020
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Oxlumo

lumasiran

On 15 October 2020, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Oxlumo[2], intended for the treatment of primary hyperoxaluria type 1 (PH1). Oxlumo was reviewed under EMA's accelerated assessment programme. The applicant for this medicinal product is Alnylam Netherlands B.V.

Oxlumo will be available as solution for injection (189 mg/ml). The active substance of Oxlumo is lumasiran, a small interfering ribonucleic acid (siRNA) that causes degradation of the messenger ribonucleic acid (mRNA) involved in the synthesis of the enzyme glycolate oxidase (GO) in the liver, leading to decreased GO enzyme. This results in reduction of plasma and urinary oxalate levels, the underlying cause of disease manifestations in patients with PH1.

The benefits of Oxlumo is its ability to reduce plasma oxalate levels and 24-hour urinary oxalate excretion compared to placebo, as observed during a 6-month double-blind, controlled clinical trial. The most common side effects are injection site reaction and abdominal pain.

The full indication is:

> Treatment of primary hyperoxaluria type 1 (PH1) in all age groups.

Oxlumo should be initiated and supervised by physicians experienced in the management of hyperoxaluria.

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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