30 January 2020
EMA/CHMP/38428/2020
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Givlaari

givosiran

On 30 January 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 Givlaari[2], intended for the treatment of acute hepatic porphyria. Givlaari was reviewed under EMA's accelerated assessment programme. The applicant for this medicinal product is Alnylam Netherlands B.V.

Givlaari will be available as 189 mg/ml solution for injection. The active substance of Givlaari is givosiran, a small interfering ribonucleic acid (siRNA) that causes degradation of the messenger ribonucleic acid (mRNA) involved in synthesis of aminolevulinate acid synthase-1, leading to reduced circulating levels of the neurotoxic intermediates aminolevulinic acid and porphobilinogen.

The benefits with Givlaari is its ability to reduce the rate of porphyria attacks which would have otherwise required hospitalisation or a healthcare visit, or intravenous treatment with hemin at home.

The most common side effects are injection site reactions, nausea, fatigue and abnormalities in kidney and liver function.

The full indication is: "treatment of acute hepatic porphyria (AHP) in adults and adolescents aged 12 years and older".

Therapy should be started under the supervision of a healthcare professional experienced in the management of porphyria.

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 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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