21 July 2022
EMA/647175/2022
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Nulibry

fosdenopterin

On 21 July 2022, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation under exceptional circumstances[2] for the medicinal product Nulibry[3], intended for the treatment of patients with molybdenum cofactor deficiency (MoCD) Type A. The applicant for this medicinal product is Comharsa Life Sciences Ltd.

Nulibry will be available as a 9.5 mg powder for solution for injection. The active substance of Nulibry is fosdenopterin, a metabolism product (ATC code: A16AX19). Nulibry is a substrate replacement therapy that provides an exogenous source of cyclic pyranopterin monophosphate (cPMP) for patients with the ultrarare neurodegenerative condition molybdenum cofactor deficiency (MoCD) Type A, in whom the product is synthesised into molybdenum cofactor via an intermediary step. Molybdenum cofactor is needed for the activation of molybdenum-dependent enzymes, including sulphite oxidase (SOX), an enzyme that reduces levels of neurotoxic sulphites.

The main benefit of Nulibry is that it increases overall survival, as observed in a pooled analysis of 15 patients in a historical comparison with untreated patients. Additional benefits include improved feeding without assistance, growth, motor and cognitive development, seizure control and a reduction of urinary S-sulfocysteine. The most common side effects are infusion-related adverse events.

The full indication is:

> NULIBRY is indicated for the treatment of patients with molybdenum cofactor deficiency (MoCD) Type A.

Nulibry should be initiated and supervised in hospital by a healthcare professional experienced in the management of inborn errors of metabolism.

[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] In exceptional circumstances, an authorisation may be granted subject to certain specific obligations, to be reviewed annually. This happens when the applicant can show that they are unable to provide comprehensive data on the efficacy and safety of the medicinal product, due to the rarity of the condition it is intended for, limited scientific knowledge in the area concerned, or ethical considerations involved in the collection of such data.
[3] 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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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.