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# Nulibry (*fosdenopterin*)

An overview of Nulibry and why it is authorised in the EU

## What is Nulibry and what is it used for?

Nulibry is a medicine used to treat patients with molybdenum cofactor deficiency (MoCD) type A.

MoCD type A is an inherited disease where patients do not have enough of a substance called 'molybdenum cofactor', a molecule needed for the production of certain enzymes. Without these enzymes, the toxic chemical sulfite builds up in the brain causing brain damage.

In the 'type A' form of the disease, the absence of molybdenum cofactor is due to patients lacking a substance called 'cyclic pyranopterin monophosphate (cPMP)', which the body needs to make molybdenum cofactor.

MoCD type A is rare, and Nulibry was designated an 'orphan medicine' (a medicine used in rare diseases) on 20 September 2010. Further information on the orphan designation can be found here.

Nulibry contains the active substance fosdenopterin.

## How is Nulibry used?

Nulibry can only be obtained with a prescription. Treatment with Nulibry should be started and supervised by a healthcare professional experienced in managing hereditary metabolic disorders.

Nulibry is given as an infusion (drip) into a vein once a day. The recommended dose is 0.90 mg per kilogram body weight. For patients less than one year of age a lower starting dose and titration schedule are recommended. The starting dose and titration schedule depend on the gestational age at birth. Treatment needs to be continued for life if the condition is confirmed by genetic testing.

For more information about using Nulibry, see the package leaflet or contact your doctor or pharmacist.

## How does Nulibry work?

The active substance in Nulibry, fosdenopterin, is a synthetic form of cPMP. As patients with MoCD type A do not have enough cPMP the medicine works by replacing this substance. The body then uses this substance to produce molybdenum cofactor, allowing it to start producing molybdenum-dependent enzymes and reducing the levels of sulfite in the brain.

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## What benefits of Nulibry have been shown in studies?

The benefits of Nulibry were studied in five main studies involving a total of 52 patients with MoCD type A. The studies looked at the effect of Nulibry on survival after one year of treatment. The results in the 15 patients treated with Nulibry were compared with historical data from two studies involving 37 patients who did not receive Nulibry or any other treatment. After one year, around 93% of patients using Nulibry were alive compared with around 75% of those who received no treatment. The studies also indicated that early treatment with Nulibry (i.e. before patients develop major brain damage) preserves the ability to take food by mouth, and improves growth and development of motor (movement) and cognitive (mental) functions.

## What are the risks associated with Nulibry?

The most common side effects with Nulibry (which may affect more than 1 in 10 people) are complications associated with the catheter (tube) delivering the medicine.

For the full list of side effects and restrictions of Nulibry, see the package leaflet.

## Why is Nulibry authorised in the EU?

Because MoCD type A is a very rare disease, the studies were small, but Nulibry was shown to be effective at improving survival of patients with MoCD type A. The studies also indicate that early treatment with Nulibry improves the quality of life of patients and delays disease progression. The side effects seen to date are considered manageable. Given the seriousness of the condition and the lack of existing treatments, the European Medicines Agency decided that Nulibry's benefits are greater than its risk and it can be authorised for use in the EU.

Nulibry has been authorised under 'exceptional circumstances'. This is because it has not been possible to obtain complete information about Nulibry due to the rarity of the disease. Every year, the Agency will review any new information that becomes available and this overview will be updated as necessary.

## What information is still awaited for Nulibry?

Since Nulibry has been authorised under exceptional circumstances, the company that markets Nulibry will provide yearly updates on any new information concerning the safety and efficacy of Nulibry. In addition the company will conduct and submit the results of a study of patients with MoCD type A treated with Nulibry in clinical practice to further characterise the long-term safety and efficacy of the medicine.

## What measures are being taken to ensure the safe and effective use of Nulibry?

The company that markets Nulibry will provide educational material to all healthcare professionals expected to prescribe Nulibry, which should be shared with patients or caregivers expected to use Nulibry in the home setting. The material will include instructions on how to use the medicine and an infusion diary.

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Nulibry have also been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Nulibry are continuously monitored. Suspected side effects reported with Nulibry are carefully evaluated and any necessary action taken to protect patients.

## Other information about Nulibry

Nulibry received a marketing authorisation under exceptional circumstances valid throughout the EU on 15 September 2022.

Further information on Nulibry can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/nulibry.

This overview was last updated in 09-2022.