EMA/561348/2020
EMEA/H/C/5040

Oxlumo (*lumasiran*)

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

What is Oxlumo and what is it used for?

Oxlumo is a medicine used for treating primary hyperoxaluria type 1, an inherited disease in which a substance called oxalate builds up in the body, causing damage to the kidneys and other organs.

Oxlumo contains the active substance lumasiran.

Primary hyperoxaluria is rare, and Oxlumo was designated an 'orphan medicine' (a medicine used in rare diseases) on 21 March 2016. Further information on the orphan designation can be found here: ema.europa.eu/medicines/human/orphan-designations/eu/3/16/1637.

How is Oxlumo used?

Oxlumo can only be obtained with a prescription and treatment should be started and supervised by a physician experienced in the management of hyperoxaluria.

Oxlumo is given by injection under the skin. It is given once a month for the first 3 months and then either once monthly or once every 3 months. The dose and frequency depend on the patient's weight.

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

How does Oxlumo work?

Patients with primary hyperoxaluria type 1 have high levels of glyoxylate which is converted into oxalate. High levels of oxalate can cause stones in the kidney as well as injury to certain organs.

The active substance in Oxlumo, lumasiran, blocks the production of an enzyme called hydroxyacid oxidase (also known as glycolate oxidase), which is involved in the production of glyoxylate. Oxlumo is a 'small interfering RNA' (siRNA), a very short piece of synthetic genetic material, which has been designed to attach and block the genetic material in the cell that is responsible for the production of the enzyme. This blocks the production of the enzyme, thereby reducing the amount of glyoxylate and oxalate, helping to relieve the symptoms of the disease.

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

© European Medicines Agency, 2020. Reproduction is authorised provided the source is acknowledged.

What benefits of Oxlumo have been shown in studies?

Oxlumo has been shown to reduce the formation of oxalate compared to placebo (a dummy treatment) in a main study with 39 patients aged between 6 and 60 years with primary hyperoxaluria type 1.

After 6 months of treatment, the level of oxalate in urine was reduced by 65% on average in patients on Oxlumo compared with 12% in patients from 6 years of age who received placebo. Among the patients who received Oxlumo, 21 out of 25 (84%) achieved normal or near normal levels of oxalate after 6 months, compared to none in the placebo group. Similar effects were seen in a study with 18 children aged under 6 years with hyperoxaluria type 1.

What are the risks associated with Oxlumo?

The most common side effects with Oxlumo (which may affect more than 1 in 10 people) are reactions at the injection site such as reddening of the skin, pain, itching and swelling.

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

Why is Oxlumo authorised in the EU?

Oxlumo can significantly reduce the levels of oxalate in the body in patients with primary hyperoxaluria type 1. Although the number of patients recruited was small due to the rarity of the disease, the effectiveness of the medicine was shown consistently across all age groups. The side effects of Oxlumo are mild or moderate. The European Medicines Agency therefore decided that Oxlumo's benefits are greater than its risks and it can be authorised for use in the EU.

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

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

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

Other information about Oxlumo

Oxlumo received a marketing authorisation valid throughout the EU on 19 November 2020.

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

This overview was last updated in 11-2020.