EMA/459638/2018
EMEA/H/C/004438

Mepsevii (*vestronidase alfa*)

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

What is Mepsevii and what is it used for?

Mepsevii is a medicine to treat mucopolysaccharidosis type VII (MPS VII, also known as Sly syndrome), an inherited disease caused by a lack of an enzyme needed to break down complex carbohydrates known as glycosaminoglycans (GAGs).

The disease leads to build up of GAGs in the body, which causes a wide range of problems, including joint stiffness, short stature, enlarged liver and spleen, hearing loss, cataract and delays in development.

MPS VII is rare, and Mepsevii was designated an 'orphan medicine' (a medicine used in rare diseases) on 21 March 2012. Further information on the orphan designation can be found here: ema.europa.eu/Find medicine/Human medicines/Rare disease designation

Mepsevii contains the active substance vestronidase alfa.

How is Mepsevii used?

Mepsevii is given every two weeks as an infusion (drip) into a vein lasting 4 hours. The recommended dose for each infusion is 4 mg per kilogram of body weight. Before the infusion the patient is given antihistamine treatment to reduce the risk of allergic reactions.

The medicine can only be obtained with a prescription and should be given by an appropriately trained healthcare professional who can manage medical emergencies. Treatment should also be supervised by a healthcare professional with experience in the treatment of MPS VII.

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

How does Mepsevii work?

Mepsevii is an enzyme replacement therapy that works by replacing the missing enzyme (beta-glucuronidase) in patients with MPS VII, helping to break down GAGs and stopping them building up in the body.

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What benefits of Mepsevii have been shown in studies?

Mepsevii has been shown to reduce the levels of GAGs in the body and reduce or stabilise symptoms in patients with MPS VII.

In a main study of 12 patients with MPS VII, patients treated with Mepsevii for 6 months had a 65% reduction in their urine levels of GAGs. In 11 out of 12 patients, symptoms, including those affecting vision and movement, improved or did not get worse.

What are the risks associated with Mepsevii?

The most common side effects with Mepsevii (which may affect more than 1 in 10 people) are anaphylactic reaction (sudden, severe allergic reaction), urticaria (itchy rash) and swelling at the infusion site.

Mepsevii must not be used in patients who have ever had anaphylactic reaction to vestronidase alfa or any of the other ingredients of the medicine. For the full list of side effects and restrictions, see the package leaflet.

Why is Mepsevii authorised in the EU?

Mepsevii reduces the levels of GAGs in the body and improves or at least stabilises the symptoms of MPS VII. In addition, most adverse reactions are mild to moderate in severity.

Although only a limited amount of data is available from clinical studies, the European Medicines Agency took into account the life-threatening and debilitating nature of MPS VII and the lack of authorised medicines. EMA decided that Mepsevii's benefits are greater than its risks and that it can be authorised for use in the EU.

Mepsevii has been authorised under 'exceptional circumstances'. This is because it has not been possible to obtain complete information about Mepsevii due to the rarity of the disease and for scientific reasons. 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 Mepsevii?

Since Mepsevii has been authorised under exceptional circumstances, the company that markets Mepsevii will conduct a study to provide data on the long-term effectiveness and safety of Mepsevii as well as on the disease itself, including its progression and development of symptoms.

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

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

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

Other information about Mepsevii

Mepsevii received a marketing authorisation under exceptional circumstances valid throughout the EU on 23 August 2018.

Further information on Mepsevii can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European public assessment reports.

This overview was last updated in 08-2018.