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Givlaari (*givosiran*)

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

What is Givlaari and what is it used for?

Givlaari is a medicine for treating acute hepatic porphyria in patients aged 12 years or over.

Acute hepatic porphyria is a rare genetic condition in which the liver cannot properly produce a substance called haem. As a result, substances used to make haem build up in the body, causing attacks of severe abdominal pain, vomiting and nervous system disorders.

Acute hepatic porphyria is rare, and Givlaari was designated an 'orphan medicine' (a medicine used in rare diseases) on 29 August 2016. Further information on the orphan designation can be found here: ema.europa.eu/medicines/human/orphan-designations/eu3161731.

Givlaari contains the active substance givosiran.

How is Givlaari used?

Givlaari is given by injection under the skin once a month. The dose depends on the patient's weight.

The medicine can only be obtained with a prescription and treatment should be started by a healthcare professional experienced in managing the condition. For more information about using Givlaari, see the package leaflet or contact your doctor or pharmacist.

How does Givlaari work?

The active substance in Givlaari, givosiran, is a synthetic small interfering RNA (a type of genetic material) that works by reducing the production of an enzyme involved in an early step of haem production in the liver. This prevents the build-up of the substances that cause the symptoms of the condition.

What benefits of Givlaari have been shown in studies?

Givlaari was more effective than placebo (a dummy treatment) in reducing the yearly number of serious porphyria attacks. In a main study involving 94 patients, those who received Givlaari had on average 3 serious attacks of symptoms per year compared with 13 in those receiving placebo.

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What are the risks associated with Givlaari?

The most common side effects with Givlaari (which may affect more than 1 in 5 people) are reactions at the site of the injection, nausea (feeling sick) and tiredness. For the full list of side effects and restrictions, see the package leaflet.

Why is Givlaari authorised in the EU?

A main study has shown that Givlaari is effective at reducing porphyria attacks. The side effects of Givlaari treatment were mostly mild and moderate and most resolved during the study.

The European Medicines Agency therefore decided that Givlaari'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 Givlaari?

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

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

Other information about Givlaari

Givlaari received a marketing authorisation valid throughout the EU on 2 March 2020.

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

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