EMA/95105/2024
EMEA/H/C/005517

Voydeya (*danicopan*)

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

What is Voydeya and what is it used for?

Voydeya is a medicine used in adults to treat paroxysmal nocturnal haemoglobinuria (PNH), a disease in which excessive breakdown of blood cells results in anaemia (low levels of red blood cells), thrombosis (blood clots in blood vessels), pancytopenia (low levels of blood cells) and dark urine (due to large amounts of haemoglobin - the protein in red blood cells that carries oxygen around the body - being released into the urine). Voydeya is used in addition to ravulizumab or eculizumab (other medicines for PNH) in patients who continue to have anaemia despite these treatments.

PNH is rare, and Voydeya was designated an 'orphan medicine' (a medicine used in rare diseases) on 12 December 2017. Further information on the orphan designation can be found on the EMA website.

Voydeya contains the active substance danicopan.

How is Voydeya used?

The medicine can only be obtained with a prescription; treatment should be started by a healthcare professional experienced in the management of patients with blood-related disorders.

Voydeya is available as tablets to be taken by mouth three times a day, approximately 8 hours apart.

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

How does Voydeya work?

The complement system is a set of proteins that is part of the immune system (the body's natural defences). In patients with PNH, the complement system is overactive and damages the patients' own blood cells.

The active substance in Voydeya, danicopan, blocks a protein of the complement system called factor D. By blocking factor D, Voydeya prevents the complement system from damaging cells, thereby helping to relieve the symptoms of the disease.

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What benefits of Voydeya have been shown in studies?

Voydeya was investigated in a main study involving 86 patients with PNH who had been treated with ravulizumab or eculizumab for at least the previous 6 months and had anaemia. Patients in the study took either Voydeya or placebo (a dummy treatment) in addition to ravulizumab or eculizumab.

After 12 weeks of treatment, the haemoglobin levels in patients taking Voydeya increased on average by 2.81 g/dL, compared with an increase of 0.41 g/dL on average in patients on placebo.

What are the risks associated with Voydeya?

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

The most common side effects with Voydeya (which may affect more than 1 in 10 people) include fever, headache, increased levels of liver enzymes (a sign of possible liver problems) and pain in the extremities (arms and legs).

Based on its mechanism of action, Voydeya may increase the risk of infections. Voydeya must not be used by patients who have an ongoing infection with the bacteria *Neisseria meningitidis*, or those who are not currently vaccinated against it unless they receive antibiotics to prevent infection until 2 weeks after vaccination.

Why is Voydeya authorised in the EU?

Voydeya in addition to ravulizumab or eculizumab was shown to be effective at reducing anaemia in patients with PNH, as shown by an increase in their haemoglobin levels after starting treatment. The safety of Voydeya is considered manageable, despite an increase in the occurrence of side effects (but not in their severity) in patients taking the medicine. The European Medicines Agency therefore decided that Voydeya's benefits are greater than its risks and that it can be authorised for use in the EU.

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

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

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

Other information about Voydeya

Voydeya received a marketing authorisation valid throughout the EU on 19 April 2024.

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

This overview was last updated in 04-2024.