swissmedic

Summary report on authorisation dated 18 October 2024

Voydeya® (active substance: danicopan)

Authorisation in Switzerland: 30 April 2024

Film-coated tablets for adjunctive treatment in clinically relevant extravascular haemolysis (EVH) in adults with paroxysmal nocturnal haemoglobinuria (PNH)

About the medicinal product

Voydeya contains the active substance danicopan. It is used for the treatment of clinically relevant extravascular haemolysis (EVH) in adult patients with paroxysmal nocturnal haemoglobinuria (PNH), who have previously received treatment with a C5-inhibitor, a medicinal product for PNH that treats intravascular haemolysis, for at least 6 months. Patients receive Voydeya in addition to their current medicinal product for PNH (ravulizumab or eculizumab; C5-inhibitors).

The signs or symptoms of EVH that can occur in the context of PNH are anaemia and associated symptoms such as fatigue and shortness of breath.

Since PNH is a rare, severe, and potentially life-threatening disease, the medicinal product Voydeya has been authorised as an orphan drug. The term "orphan drug" is used to refer to important medicines for rare diseases.

Mode of action

Voydeya contains the active substance danicopan. Danicopan blocks a protein called factor D, which is part of the body's own defence system known as the "complement system". Danicopan prevents the body's immune system from destroying red blood cells outside the vascular system (extravascular haemolysis).

Administration

Voydeya is a prescription-only medicine and available as film-coated tablets with a dosage of 50 mg or 100 mg danicopan. The recommended starting dose is 150 mg 3 times daily, at intervals of approximately 8 hours. The tablets are swallowed whole. The tablets can be taken with or without food.

Depending on the response to treatment, the dose can be increased to 200 mg Voydeya 3 times daily as directed by the doctor. Voydeya is taken in addition to the currently prescribed medicinal product for PNH (ravulizumab or eculizumab).

PNH is a chronic disease and adjunctive therapy with Voydeya should be used by patients on a life-long basis, unless discontinuation of Voydeya is indicated for medical reasons.

Efficacy

The efficacy of Voydeya as adjunctive therapy to ravulizumab or eculizumab in adult patients with PNH and clinically relevant EVH was investigated in study ALXN2040-PNH-301. The 86 patients participating in the study had been treated with a stable dose of a C5-inhibitor for the last 6 months and had anaemia (haemoglobin [Hb] ≤ 9.5 g/dL) with or without transfusion support. The study participants received either Voydeya or placebo (dummy medication) for 12 weeks in addition to background treatment with ravulizumab or eculizumab and all patients were treated with Voydeya for a further 12 weeks in addition to background treatment.

Adjunctive treatment with Voydeya led to a statistically significant and clinically meaningful increase in Hb from baseline to week 12 compared to placebo treatment.

In the group treated with Voydeya, 59.5% of patients recorded an increase in Hb of > 2.0 g/dL, compared to 0% in the placebo group.

In addition, 83.3% of patients in the Voydeya group were able to avoid a transfusion versus 38.1% in the placebo group.

Precautions, undesirable effects, & risks

Voydeya must not be used in those who are hypersensitive to the active substance or any of the excipients.

The most common undesirable effects (affecting more than 1 in 10 users) are fever or high temperature, headache, diarrhoea, nausea, upper respiratory tract infections, fatigue, vomiting, sore throat, pain in the joints, arm and leg pain, increased liver enzyme levels in the blood, decrease in red blood cells (anaemia).

All precautions, risks, and other possible undesirable effects are listed in the Information for patients (package leaflet) and the Information for healthcare professionals.

Why the medicinal product has been authorised

The conducted study showed a significant and clinically meaningful improvement in haemoglobin levels in patients treated with Voydeya.

Despite the small number of patients and the relatively short duration of the observation period, efficacy is deemed convincing and the safety profile acceptable. In view of the rarity of the disease and the existing medical need, the benefit of this medicinal product outweighs the associated risks.

Swissmedic has therefore authorised the medicinal product Voydeya, containing the active substance danicopan, in Switzerland as adjunctive treatment for patients with PNH who have clinically significant EVH.

Further information on the medicinal product

Information for healthcare professionals: Information for healthcare professionals Voydeya®

Information for patients (package leaflet): Information for patients Voydeya®

Healthcare professionals can answer any further questions.

The date of revision of this text corresponds to that of the SwissPAR. New information concerning the authorised medicinal product in question will not be incorporated into the Summary report on authorisation.

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