swissmedic

Public Summary SwissPAR dated 7 October 2022

Ultomiris® (active substance: ravulizumab)

Indication extension in Switzerland: 14 July 2022

Concentrate for solution for infusion for the treatment of patients weighing at least 10 kg with paroxysmal nocturnal haemoglobinuria (PNH).

Information on authorisation

Ultomiris was approved by Swissmedic on 20 January 2020 for the treatment of adults with paroxysmal nocturnal haemoglobinuria (PNH). In addition, another indication extension for Ultomiris for the treatment of adults and children weighing at least 10 kg with atypical haemolytic uraemic syndrome (aHUS) was approved on 24 August 2021.

The present indication extension means that children and adolescents weighing at least 10 kg who have paroxysmal nocturnal haemoglobinuria (PNH) can now also be treated with Ultomiris.

Ultomiris may be used for the treatment of PNH in patients in the case of haemolysis with clinical symptoms that indicate a high level of disease activity. Ultomiris can also be used in patients who have received treatment with another medicine containing the active substance eculizumab for at least the last six months.

Paroxysmal nocturnal haemoglobinuria (PNH) and atypical haemolytic uraemic syndrome (aHUS) are very rare diseases in which the complement system (an important part of the body's immune system) is uncontrollably and excessively activated due to a lack of, or the incorrect function of, key proteins for cell signalling processes.

In patients with PNH, red blood cells are destroyed, resulting in anaemia (low red blood cell count), thromboses (blood clots in the blood vessels), pancytopenia (low blood cell count) and dark urine.

As paroxysmal nocturnal haemoglobinuria (PNH) and atypical haemolytic uraemic syndrome (aHUS) are very rare, life-threatening diseases, Ultomiris has been authorised as an “orphan drug”. The term "orphan drug" is used to refer to important medicines for rare diseases.

This indication extension for Ultomiris was authorised under Article 13 of the Therapeutic Products Act (TPA). This means that the indication extension is already authorised in another country with comparable medicinal product control. In this case, Swissmedic takes into consideration the results of checks carried out by foreign regulatory agencies, provided certain requirements are fulfilled. These involve checks on the efficacy and safety of the medicinal product in the indication applied for, and the extent to which the results can be accepted for Switzerland.

The consideration of the results of foreign authorisation procedures is intended to help ensure that medicines that are already authorised abroad can be made available to patients in Switzerland as quickly as possible.

In deciding whether to authorise the indication extension for Ultomiris in Switzerland, Swissmedic accepted the assessment of the European Medicines Agency (EMA) and the EMA's authorisation decision, and has not conducted its own scientific review.

Accordingly, in the SwissPAR (Swiss Public Assessment Report) and the resulting Public Summary SwissPAR, Swissmedic refers to the Assessment Report and the short report issued by the reference authority: (www.ema.europa.eu)

Further information on the medicinal product

Information for healthcare professionals: Information for healthcare professionals Ultomiris®

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 Public Summary SwissPAR.

Swissmedic monitors medicinal products authorised in Switzerland. Swissmedic initiates the necessary action in the event of newly discovered adverse drug reactions or other safety-relevant signals. New findings that could impair the quality, efficacy or safety of this medicinal product are recorded and published by Swissmedic. If necessary, the medicinal product information is adapted.