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Ultomiris® (active substance: ravulizumab)

Indication extension in Switzerland: 29 August 2023

Medicinal product (concentrate for solution for infusion) for the treatment of adults with NMOSD who are AQP4 antibody-positive

About the medicinal product

The medicinal product Ultomiris, containing the active substance ravulizumab, is used to treat adults with neuromyelitis optica spectrum disorder (NMOSD) who have antibodies against the protein AQP4 (Aquaporin-4).

NMOSD is a rare autoimmune disease of the central nervous system mainly affecting the optic nerves and the bone marrow. Preventing disease flare-ups is an important objective in the treatment of NMOSD. Disease rates are very low in Europe. Currently, two approved long-term treatment options are available in Switzerland (Enspryng® and Soliris®).

Ultomiris was approved by Swissmedic on 20 January 2020 for the treatment of adults with paroxysmal nocturnal haemoglobinuria (PNH). In addition, an 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. On 14 July 2022, a further indication extension for Ultomiris for the treatment of children and adolescents weighing at least 10 kg with paroxysmal nocturnal haemoglobinuria (PNH) was approved by Swissmedic for use in Switzerland.

Since NMOSD is a very rare and life-threatening disease, the present indication extension for Ultomiris has also been authorised as an “orphan drug”. The term "orphan drug" is used to refer to important medicines for rare diseases.

Mode of action

In NMOSD, the complement system (an important part of the body’s immune defence system) is uncontrollably and excessively activated due to a lack of, or the incorrect functioning of, key proteins for the cell signalling process. This causes damage to nerve cells.

The active substance in Ultomiris, ravulizumab, is a monoclonal antibody. Monoclonal antibodies are proteins that can bind specifically to other proteins. Ultomiris binds to the C5 protein, which is part of the complement system. By binding to, and so blocking, the protein, Ultomiris prevents the immune defence system from damaging cells

and thereby helps control the symptoms of the disease.

Administration

Ultomiris, containing the active substance ravulizumab, is a prescription-only medicine.

Ultomiris is a concentrate for solution for infusion available in dosages of 300 mg/30 mL, 300 mg/3 mL, and 1100 mg/11 mL. It is administered intravenously (into the veins).

The recommended dosage regimen comprises an initial dose followed by maintenance doses. The doses are based on the patient's body weight and are administered at intervals of 8 weeks (maintenance doses), starting 2 weeks after administration of the initial dose.

Before starting treatment, it should be ensured that the patient is free of active meningococcal infection or sepsis (blood poisoning) and is sufficiently vaccinated against meningococcal disease. Due to the mechanism of action of Ultomiris, there is an increased susceptibility to meningococcal infection/sepsis.

Efficacy

The efficacy of Ultomiris has been investigated in the (ongoing) Study 307, undertaken with adults with NMOSD who are AQP4 antibody-positive.
A total of 58 male and female patients were treated with Ultomiris and compared with 47 male and female patients who had received a placebo (dummy drug) in another study in adults with NMOSD. By the database lock date, almost all of them (96.6%) had completed the primary treatment phase of a median[1] 73.5 weeks. No disease flare-ups were observed during the primary treatment period in patients treated with Ultomiris. This contrasts with the 42.6% of patients in the placebo group who experienced disease flare-ups. An updated analysis shows that during an average treatment duration with Ultomiris of almost 2 years, no disease flare-ups were recorded. There were, however, imbalances in disease characteristics between the patients in the Ultomiris group and the placebo group, which hindered direct comparison of the treatment effects.

The efficacy of Ultomiris for the acute treatment of disease flare-ups in NMOSD patients has not been investigated.

Precautions, undesirable effects, and risks

Ultomiris must not be used in patients who are hypersensitive to the active substance or any of the excipients. It must also not be used in the event of meningococcal infection, if the patient is not vaccinated against meningococcal disease, or in cases of known congenital defects of the complement system.

The most frequent adverse effects (affecting more than 1 in 10 users) are diarrhoea, upper respiratory tract infections, nasopharyngitis (combined inflammation of the nose and throat), and headache.

The most severe side effects observed are meningococcal infection and meningococcal sepsis.

[1] Median: The value that lies exactly in the middle of a distribution of data is called the median or central value. Half of the data values are always less than the median, the other half are always greater.

All precautions, risks, and other possible undesirable effects are listed in the Information for healthcare professionals.

Why the medicinal product has been authorised

Although therapies already exist for the treatment of NMOSD, there is a great medical need for safe and effective treatment options.

Study 307 has shown that Ultomiris can significantly prolong the time free from flare-ups compared to placebo.

Taking all the risks and precautions into account, and based on the available data, the benefits of Ultomiris in treating NMOSD outweigh the risks. Swissmedic has therefore authorised the medicinal product Ultomiris, containing the active substance ravulizumab, for use in Switzerland.

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.