



26 April 2019
EMA/CHMP/223053/2019
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion¹ (initial authorisation)

Ultomiris ravulizumab

On 26 April 2019, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Ultomiris, intended for the treatment of adult patients with paroxysmal nocturnal haemoglobinuria (PNH). Ultomiris was designated as an orphan medicinal product on 30 May 2016. The applicant for this medicinal product is Alexion Europe SAS.

Ultomiris will be available as a 300 mg concentrate for solution for infusion. The active substance of Ultomiris is ravulizumab, a selective immunosuppressant (ATC code: L04AA43). Ravulizumab is a monoclonal antibody that specifically binds to the complement protein C5, thereby inhibiting its cleavage to C5a and C5b and preventing the generation of C5b-9. Ravulizumab preserves the early components of complement activation that are essential for opsonisation of microorganisms and clearance of immune complexes.

The benefits of Ultomiris are its ability to reduce and maintain control of haemolysis in patients with PNH. Its most common side effects are headache, nasopharyngitis and upper respiratory tract infection.

The full indication is:

“ULTOMIRIS is indicated in the treatment of adult patients with paroxysmal nocturnal haemoglobinuria (PNH):

- in patients with haemolysis with clinical symptom(s) indicative of high disease activity
- in patients who are clinically stable after having been treated with eculizumab for at least the past 6 months (see section 5.1).”

It is proposed that Ultomiris be prescribed by physicians experienced in the treatment of patients with haematological disorders.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.

¹ Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion

