



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/535289/2021

European Medicines Agency decision P/0413/2021

of 29 October 2021

on the granting of a product specific waiver for ravulizumab (Ultomiris), (EMEA-001943-PIP05-21) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

Only the English text is authentic.



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on the granting of a product specific waiver for ravulizumab (Ultomiris), (EMA-001943-PIP05-21) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

The European Medicines Agency,

Having regard to the Treaty on the Functioning of the European Union,

Having regard to Regulation (EC) No 1901/2006 of the European Parliament and of the Council of 12 December 2006 on medicinal products for paediatric use and amending Regulation (EEC) No. 1768/92, Directive 2001/20/EC, Directive 2001/83/EC and Regulation (EC) No 726/2004¹,

Having regard to Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency²,

Having regard to the application submitted by Alexion Europe SAS on 4 June 2021 under Article 13 of Regulation (EC) No 1901/2006,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 10 September 2021 in accordance with Article 13 of Regulation (EC) No 1901/2006,

Having regard to Article 25 of Regulation (EC) No 1901/2006,

Whereas:

- (1) The Paediatric Committee has given an opinion on the granting of a product specific waiver.
- (2) It is therefore appropriate to adopt a decision granting a waiver.

Has adopted this decision:

Article 1

A waiver for ravulizumab (Ultomiris), concentrate for solution for infusion, intravenous use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 2

This decision is addressed to Alexion Europe SAS, 103 - 105 rue Anatole France, 92300 - Levallois-Perret, France.

¹ OJ L 378, 27.12.2006, p.1.

² OJ L 136, 30.4.2004, p. 1.



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EMA/PDCO/344827/2021
Amsterdam, 10 September 2021

Opinion of the Paediatric Committee on the granting of a product-specific waiver

EMA-001943-PIP05-21

Scope of the application

Active substance(s):

Ravulizumab

Invented name:

Ultomiris

Condition(s):

Treatment of amyotrophic lateral sclerosis

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Concentrate for solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Alexion Europe SAS

Information about the authorised medicinal product:

See Annex II

Basis for opinion

Pursuant to Article 13 of Regulation (EC) No 1901/2006 as amended, Alexion Europe SAS submitted to the European Medicines Agency on 4 June 2021 an application for a product-specific waiver on the grounds set out in Article 11 of said Regulation for the above mentioned medicinal product.

The procedure started on 12 July 2021.



Opinion

1. The Paediatric Committee, having assessed the waiver application in accordance with Article 13 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

- to grant a product-specific waiver for all subsets of the paediatric population and the above mentioned condition(s) in accordance with Article 11(1)(c) of said Regulation, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

The Norwegian Paediatric Committee member agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The grounds for the granting of the waiver are set out in Annex I.

This opinion is forwarded to the applicant and the Executive Director of the European Medicines Agency, together with its annexes and appendix.

Annex I

Grounds for the granting of the waiver

1. Waiver

1.1. Condition:

Treatment of amyotrophic lateral sclerosis

The waiver applies to:

- all subsets of the paediatric population from birth to less than 18 years of age;
- concentrate for solution for infusion, intravenous use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible.

Annex II

Information about the authorised medicinal product

Condition(s) and authorised indication(s):

1. Treatment of paroxysmal nocturnal haemoglobinuria

Authorised indication:

- 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.

2. Treatment of atypical haemolytic uremic syndrome

Authorised indication:

- Ultomiris is indicated in the treatment of patients with a body weight of 10 kg or above with atypical haemolytic uremic syndrome (aHUS) who are complement inhibitor treatment-naïve or have received eculizumab for at least 3 months and have evidence of response to eculizumab (see section 5.1).

Authorised pharmaceutical form(s):

Concentrate for solution for infusion

Authorised route(s) of administration:

Intravenous use