EMA/389665/2023

European Medicines Agency decision

P/0388/2023

of 7 September 2023

on the granting of a product specific waiver for ravulizumab (Ultomiris), (EMEA-001943-PIP06-23) 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), (EMEA-001943-PIP06-23) 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 726/2004[1],

Having regard to Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency[2],

Having regard to the application submitted by Alexion Europe SAS on 24 April 2023 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 21 July 2023 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.

[1] OJ L 378, 27.12.2006, p.1, as amended.
[2] OJ L 136, 30.4.2004, p. 1, as amended.

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.

EMA/222878/2023
Amsterdam, 21 July 2023

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

EMEA-001943-PIP06-23

Scope of the application

Active substance(s):

Ravulizumab

Invented name and authorisation status:

See Annex II

Condition(s):

Prevention of kidney injury in high-risk patients with chronic kidney disease undergoing cardiopulmonary bypass

Pharmaceutical form(s):

Concentrate for solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Alexion Europe SAS

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 24 April 2023 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 22 May 2023.

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 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 Paediatric Committee member Norway 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:

Prevention of kidney injury in high-risk patients with chronic kidney disease undergoing cardiopulmonary bypass

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

Information provided by the applicant:

Condition(s) and authorised indication(s)

1. Treatment of paroxysmal nocturnal haemoglobinuria (PNH)

Authorised indication(s):

- Treatment of adult and paediatric patients with a body weight of 10 kg or above 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
    - Invented name(s): Ultomiris
    - Authorised pharmaceutical form(s): concentrate for solution for infusion
    - Authorised route(s) of administration: intravenous use
    - Authorised via centralised procedure
- 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
    - Invented name(s): Ultomiris
    - Authorised pharmaceutical form(s): solution for injection (on-body injector)
    - Authorised route(s) of administration: subcutaneous use
    - Authorised via centralised procedure

2. Treatment of atypical haemolytic uremic syndrome (aHUS)

Authorised indication(s):

- 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
  - Invented name(s): Ultomiris
  - Authorised pharmaceutical form(s): concentrate for solution for infusion
  - Authorised route(s) of administration: intravenous use
  - Authorised via centralised procedure
- Treatment of adult patients 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
  - Invented name(s): Ultomiris
  - Authorised pharmaceutical form(s): solution for injection (on-body injector)

- Authorised route(s) of administration: subcutaneous use
- Authorised via centralised procedure

3. Treatment of Generalized myasthenia gravis (gMG)

Authorised indication(s):

- Add-on to standard therapy for the treatment of adult patients with gMG who are anti-acetylcholine receptor (AChR) antibody-positive
  - Invented name(s): Ultomiris
  - Authorised pharmaceutical form(s): concentrate for solution for infusion
  - Authorised route(s) of administration: intravenous use
  - Authorised via centralised procedure

4. Treatment of Neuromyelitis optica spectrum disorder (NMOSD)

Authorised indication(s):

- Treatment of adult patients with NMOSD who are anti-aquaporin 4 (AQP4) antibody-positive
  - Invented name(s): Ultomiris
  - Authorised pharmaceutical form(s): concentrate for solution for infusion
  - Authorised route(s) of administration: intravenous use
  - Authorised via centralised procedure