EMA/134196/2023

European Medicines Agency decision

P/0124/2023

of 14 April 2023

on the refusal of a modification of an agreed paediatric investigation plan for eculizumab (Soliris), (EMEA-000876-PIP03-14-M06) 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 refusal of a modification of an agreed paediatric investigation plan for eculizumab (Soliris), (EMEA-000876-PIP03-14-M06) 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 European Medicines Agency's decision P/0356/2016 issued on 21 December 2016, the decision P/0375/2017 issued on 1 December 2017, the decision P/0364/2018 issued on 6 December 2018, the decision P/0252/2019 issued on 16 July 2019, the decision P/0075/2020 issued on 21 March 2020 and the decision P/0514/2020 issued on 22 December 2020,

Having regard to the application submitted by Alexion Europe SAS on 17 November 2022 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 24 February 2023, in accordance with Article 22 of Regulation (EC) No 1901/2006 and of its own motion in accordance with Article 12 of said Regulation,

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

Whereas:

(1) The Paediatric Committee of the European Medicines Agency has given an opinion on the refusal of changes to the agreed paediatric investigation plan and on the granting of a waiver.

(2) It is therefore appropriate to adopt a decision on the refusal of changes to the agreed paediatric investigation plan.

(3) 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

Changes to the agreed paediatric investigation plan for eculizumab (Soliris), 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, are hereby refused.

Article 2

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

Article 3

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

EMA/PDCO/904835/2022
Amsterdam, 24 February 2023

Opinion of the Paediatric Committee on the refusal of a modification of an agreed Paediatric Investigation Plan and on the granting of a product-specific waiver

EMEA-000876-PIP03-14-M06

Scope of the application

Active substance(s):

Eculizumab

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of neuromyelitis optica spectrum disorders

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 22 of Regulation (EC) No 1901/2006 as amended, Alexion Europe SAS submitted to the European Medicines Agency on 17 November 2022 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/0356/2016 issued on 21 December 2016, the decision P/0375/2017 issued on 1 December 2017, the decision P/0364/2018 issued on 6 December 2018, the decision P/0252/2019 issued on 16 July 2019, the decision P/0075/2020 issued on 21 March 2020 and the decision P/0514/2020 issued on 22 December 2020.

The application for modification proposed changes to the agreed paediatric investigation plan.

The procedure started on 3 January 2023.

Scope of the modification

The waiver has been extended to cover all subsets of the paediatric population.

Opinion

The Paediatric Committee, having assessed the application in accordance with Article 22 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

- to refuse the changes proposed by the applicant regarding the paediatric investigation plan and in accordance with Article 12 of Regulation (EC) No 1901/2006 as amended, recommends to extend the product-specific waiver of its own motion to all subsets of the paediatric population concluded 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 of Norway agrees with the above-mentioned recommendation of the Paediatric Committee.

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 neuromyelitis optica spectrum disorders

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):

- Soliris is indicated in adults and children for the treatment of patients with paroxysmal nocturnal haemoglobinuria (PNH).
  - Invented name(s): Soliris
  - Authorised pharmaceutical form(s): Concentrate for solution for infusion
  - Authorised route(s) of administration: Intravenous use
  - Authorised via centralised procedure

2. Treatment of atypical haemolytic uraemic syndrome (aHUS)

Authorised indication(s):

- Soliris is indicated in adults and children for the treatment of patients with atypical haemolytic uraemic syndrome (aHUS).
  - Invented name(s): Soliris
  - Authorised pharmaceutical form(s): Concentrate for solution for infusion
  - Authorised route(s) of administration: Intravenous use
  - Authorised via centralised procedure

3. Treatment of myasthenia gravis

Authorised indication(s):

- Soliris is indicated in adults for the treatment of refractory generalized myasthenia gravis (gMG) in patients who are anti-acetylcholine receptor (AChR) antibody-positive.
  - Invented name(s): Soliris
  - 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

Authorised indication(s):

- Soliris is indicated in adults for the treatment of neuromyelitis optica spectrum disorder in patients who are anti-aquaporin-4 (AQP4) antibody-positive with a relapsing course of the disease.
  - Invented name(s): Soliris
  - Authorised pharmaceutical form(s): Concentrate for solution for infusion
  - Authorised route(s) of administration: Intravenous use
  - Authorised via centralised procedure