EMA/369854/2019

European Medicines Agency decision

P/0252/2019

of 16 July 2019

on the acceptance of a modification of an agreed paediatric investigation plan for eculizumab (Soliris) (EMEA-000876-PIP03-14-M03) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

Disclaimer

This decision does not constitute entitlement to the rewards and incentives referred to in Title V of Regulation (EC) No 1901/2006.

Only the English text is authentic.

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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 Community procedures for the authorisation and supervision of medicinal products for human and veterinary 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 and the decision P/0375/2017 issued on 1 December 2017 and the decision P/0364/2018 issued on 6 December 2018,

Having regard to the application submitted by Alexion Europe SAS on 25 February 2019 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 29 May 2019, in accordance with Article 22 of Regulation (EC) No 1901/2006,

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 acceptance of changes to the agreed paediatric investigation plan.

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

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

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for eculizumab (Soliris), concentrate for solution for infusion, intravenous use, are hereby accepted in the scope set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices.

Article 2

This decision covers all conditions, indications, pharmaceutical forms, routes of administration, measures, timelines, waivers and deferrals, as agreed in the decision P/224/2010 issued on 29 October 2010 and P/0046/2016 issued on 26 February 2016, including subsequent modifications thereof.

Article 3

This decision is addressed to Alexion Europe SAS, 1-15 avenue Edouard Belin, 92500 - Rueil-Malmaison, France.

EMA/PDCO/161596/2019
Amsterdam, 29 May 2019

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan

EMEA-000876-PIP03-14-M03

Scope of the application

Active substance(s):

Eculizumab

Invented name:

Soliris

Condition(s):

Treatment of neuromyelitis optica spectrum disorders

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 22 of Regulation (EC) No 1901/2006 as amended, Alexion Europe SAS submitted to the European Medicines Agency on 25 February 2019 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 and the decision P/0375/2017 issued on 1 December 2017 and the decision P/0364/2018 issued on 6 December 2018.

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

The procedure started on 1 April 2019.

Scope of the modification

Some measures of the Paediatric Investigation Plan have been modified.

Opinion

1. 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 agree to changes to the paediatric investigation plan in the scope set out in the Annex I of this opinion.

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

2. The measures and timelines of the paediatric investigation plan and the subset(s) of the paediatric population and condition(s) covered by the waiver are set out in the 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

The subset(s) of the paediatric population and condition(s) covered by the waiver and the measures and timelines of the agreed paediatric investigation plan (PIP)

1. Waiver

1.1. Condition

Treatment of neuromyelitis optica spectrum disorders

The waiver applies to:

- the paediatric population from birth to less than 2 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(s) are not feasible.

2. Paediatric investigation plan

2.1. Condition

Treatment of neuromyelitis optica spectrum disorders

2.1.1. Indication(s) targeted by the PIP

Treatment of relapsing neuromyelitis optica spectrum disorders (NMOSD) in the paediatric population

2.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From 2 to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Concentrate for solution for infusion

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable
Non-clinical studies	0	Not applicable
Clinical studies	1	**Study 1** Open-label, single-arm trial to evaluate safety and activity of eculizumab in children from 2 to less than 18 years of age with relapsing neuromyelitis optica spectrum disorders (NMOSD).

Extrapolation, modelling and simulation studies	2	**Study 2** Modelling and simulation study to determine the dose of eculizumab in the treatment of neuromyelitis optica spectrum disorders in children from 2 to less than 18 years of age. **Study 3** Analysis of existing efficacy and safety data on eculizumab for treatment of NMOSD using adult efficacy data from study ECU-NMO-301, paediatric efficacy and safety data from study ECU-NMO-303 and paediatric safety data in prevention of paroxysmal nocturnal haemoglobinuria (PNH) and atypical haemolytic uraemic syndrome (aHUS).
Other studies	1	**Study 4** Observational Paediatric Data Collection Study on NMOSD paediatric patients.
Other measures	0	Not applicable

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By October 2021
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

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).

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).

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.

Authorised pharmaceutical form(s):

Concentrate for solution for infusion

Authorised route(s) of administration:

Intravenous use