



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/133082/2016

European Medicines Agency decision

P/0046/2016

of 26 February 2016

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for eculizumab (Soliris) (EMEA-000876-PIP05-15) 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 726/2004¹,

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 6 July 2015 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 29 January 2016, in accordance with Article 18 of Regulation (EC) No 1901/2006, and Article 21 of said Regulation and Article 13 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 agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver.
- (2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.
- (3) It is therefore appropriate to adopt a decision granting a deferral.
- (4) It is therefore appropriate to adopt a decision granting a waiver.

¹ OJ L 378, 27.12.2006, p.1.

² OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

A 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, is hereby agreed.

Article 2

A deferral 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, is hereby granted.

Article 3

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 of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 4

This agreed paediatric investigation plan 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, including subsequent modifications thereof.

Article 5

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

Done at London, 26 February 2016

For the European Medicines Agency
Zaïde Frias
Head of Division
Human Medicines Research and Development Support
(Signature on file)



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/PDCO/747181/2015

London, 29 January 2016

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a deferral and a waiver

EMA-000876-PIP05-15

Scope of the application

Active substance(s):

Eculizumab

Invented name:

Soliris

Condition(s):

Treatment of myasthenia gravis

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 16(1) of Regulation (EC) No 1901/2006 as amended, Alexion Europe SAS submitted for agreement to the European Medicines Agency on 6 July 2015 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 11 August 2015.

Supplementary information was provided by the applicant on 9 November 2015. The applicant proposed modifications to the paediatric investigation plan.

Opinion

1. The Paediatric Committee, having assessed the proposed paediatric investigation plan in accordance with Article 17 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

- to agree the paediatric investigation plan in accordance with Article 18 of said Regulation;
- to grant a deferral in accordance with Article 21 of said Regulation;
- to grant a waiver for one or more subsets of the paediatric population in accordance with Article 13 of said Regulation and 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 Norwegian Paediatric Committee member agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the agreed 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 myasthenia gravis

The waiver applies to:

- The paediatric population from birth to less than 6 years of age;
- For concentrate for solution for infusion, intravenous use;
- On the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

2. Paediatric investigation plan

2.1. Condition:

Treatment of myasthenia gravis

2.1.1. Indication(s) targeted by the PIP

Treatment of refractory acetylcholine receptor antibody (AChR-Ab)- positive generalised myasthenia gravis

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

From 6 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.

Area	Number of measures	Description
Clinical studies	1	Study 1 Open-label, multi-centre study to evaluate pharmacokinetics, safety and effect of eculizumab in paediatric patients from 6 to less than 18 years of age with refractory AChR-Ab positive generalized myasthenia gravis and to confirm the selected paediatric dosing in the modelling and simulation study (ECU-MG-303).
Extrapolation, modelling and simulation studies	2	Study 2 Modelling and simulation study to evaluate the use and support dosing regimen of eculizumab in paediatric patients from 6 to less than 18 years of age with refractory AChR-Ab positive generalized myasthenia gravis. Study 3 Extrapolation study to evaluate efficacy, pharmacokinetics/pharmacodynamic and safety of eculizumab in paediatric patients from 6 to less than 18 years of age with AChR-Ab positive generalized myasthenia gravis.
Other studies	0	Not applicable.
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 March 2022
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

Authorised indication(s):

- Soliris is indicated in adults and children for the treatment of patients with paroxysmal nocturnal haemoglobinuria (PNH). Evidence of clinical benefit is demonstrated in patients with haemolysis with clinical symptom(s) indicative of high disease activity, regardless of transfusion history.

2. Treatment of Atypical Haemolytic Uraemic Syndrome

Authorised indication(s):

- Soliris is indicated in adults and children for the treatment of patients with atypical haemolytic uremic syndrome (aHUS)

Authorised pharmaceutical form(s):

Concentrate for solution for infusion

Authorised route(s) of administration:

Intravenous use