EMA/792272/2012

European Medicines Agency decision

P/0306/2012

of 21 December 2012

on the acceptance of a modification of an agreed paediatric investigation plan for eculizumab (Soliris) (EMEA-000876-PIP02-11-M01) 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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on the acceptance of a modification of an agreed paediatric investigation plan for eculizumab (Soliris) (EMEA-000876-PIP02-11-M01) 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 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/0164/2012 issued on 23 July 2012,

Having regard to the application submitted by Alexion Europe SAS on 23 November 2012 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 7 December 2012, 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 agreed PIP 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 3

This decision is addressed to Alexion Europe SAS, 25 boulevard de l'amiral Bruix, 75016 Paris, France.

Done at London, 21 December 2012

For the European Medicines Agency
Guido Rasi
Executive Director
(Signature on file)

EMA/PDCO/760558/2012

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

EMEA-000876-PIP02-11-M01

Scope of the application

Active substance(s):

Eculizumab

Invented name:

Soliris

Condition(s):

Treatment of Shiga-Toxin Producing Escherichia Coli Hemolytic Uremic Syndrome (STEC-HUS)

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

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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 23 November 2012 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0164/2012 issued on 23 July 2012.

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

The procedure started on 5 December 2012.

Scope of the modification

Some measures and/or timelines 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 Icelandic and the Norwegian Paediatric Committee members agree with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the paediatric investigation plan 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 annex(es) and appendix.

London, 7 December 2012

On behalf of the Paediatric Committee
Dr Daniel Brasseur, Chairman
(Signature on file)

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

1. Waiver

Not applicable.

2. Paediatric Investigation Plan

2.1. Condition: Treatment of Shiga-Toxin Producing Escherichia Coli Haemolytic Uremic Syndrome (STEC-HUS)

2.1.1. Indication(s) targeted by the PIP

Treatment of Shiga-Toxin Producing Escherichia Coli Haemolytic Uremic Syndrome (STEC-HUS).

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

From birth 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 studies	Description
Quality	0	Not applicable.
Non-clinical	0	Not applicable.
Clinical	3	Study 1: Open-label, multicentre, multiple dose trial to evaluate safety and short-term activity of Eculizumab in children from 2 months of age to less than 18 years of age (and adults) with Shiga-toxin producing Escherichia Coli haemolytic uremic syndrome (STEC-HUS). Study 2: Double-blind, randomized, placebo-controlled, multicentre trial to evaluate safety and efficacy of Eculizumab in children from 1 month of age to less than 18 years of age with STEC-HUS who are receiving standard of care. Measure 3: Extrapolation of efficacy and safety data from children from 1 month to less than 18 years of age to children less than 1 month of age.

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By June 2019
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 indications:

Soliris (eculizumab) is indicated for the treatment of patients with:

- Paroxysmal nocturnal haemoglobinuria (PNH)

Evidence of clinical benefit of Soliris in the treatment of patients with PNH is limited to patients with history of transfusions.

2. Treatment of atypical haemolytic uremic syndrome (aHUS)

   Authorised indications:

Soliris (eculizumab) is indicated for the treatment of patients with:

- Atypical haemolytic uremic syndrome (aHUS).

Authorised pharmaceutical formulation(s):

Concentrate for solution for infusion

Authorised route(s) of administration:

Intravenous use