EMA/629630/2010

European Medicines Agency decision

P/224/2010

of 29 October 2010

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-PIP01-10) 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 application submitted by Alexion Europe SAS on 8 March 2010 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 10 September 2010, 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 decision is addressed to Alexion Europe SAS, 25, Boulevard de l'Amiral Bruix, 75016 Paris, France.

Done at London, 29 October 2010

For the European Medicines Agency
Thomas Lönngren
Executive Director

(Signature on file)

EMA/PDCO/512346/2010

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

EMEA-000876-PIP01-10

Scope of the application

Active substance(s):

Eculizumab

Invented name:

Soliris

Condition(s):

Treatment of Paroxysmal Nocturnal Haemoglobinuria

Treatment of Atypical Haemolytic Uraemic Syndrome

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 16(1) of Regulation (EC) No 1901/2006 as amended, Alexion Europe SAS submitted for agreement to the European Medicines Agency on 8 March 2010 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 15 April 2010.

Supplementary information was provided by the applicant on 28 June 2010.

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)(b) of said Regulation, on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified subset(s) of the paediatric population.

   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 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 annex(es) and appendix.

London, 10 September 2010

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

1.1. Condition: Treatment of Paroxysmal Nocturnal Haemoglobinuria

The waiver applies to:

- Children from birth to less than 2 years of age
- for concentrate for solution for infusion, intravenous use
- on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

2. Paediatric Investigation Plan

2.1. Condition: Treatment of Paroxysmal Nocturnal Haemoglobinuria

2.1.1. Indication(s) targeted by the PIP

Treatment of Paroxysmal Nocturnal Haemoglobinuria

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

From 2 years to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Concentrate for solution for infusion

2.1.4. Studies

Area	Number of studies	Description
Quality		Not applicable.
Non-clinical		Not applicable.
Clinical	1	Study 1: Open-label, multicentre, single arm trial to evaluate pharmacokinetic and pharmacodynamic of eculizumab in children from 2 to less than 18 years of age with Paroxysmal Nocturnal Haemoglobinuria.

2.2. Condition: Treatment of Atypical Haemolytic Uraemic Syndrome

2.2.1. Indication(s) targeted by the PIP

Treatment of Atypical Haemolytic Uraemic Syndrome

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

From birth to less than 18 years of age.

2.2.3. Pharmaceutical form(s)

Concentrate for solution for infusion

2.2.4. Studies

Area	Number of studies	Description
Quality		Not applicable.
Non-clinical		Not applicable.
Clinical	4	Study 2: Open-label, multicentre, single arm trial to evaluate the effect of eculizumab in adolescents from 12 to less than 18 years of age with weight ≥ 40 kg or more with plasma therapy-resistant Atypical Haemolytic Uraemic Syndrome. Study 3: Open-label, multicentre, single arm trial to evaluate the effect of eculizumab in adolescents from 12 to less than 18 years of age with weight ≥ 40 kg with plasma therapy-sensitive Atypical Haemolytic Uraemic Syndrome. Study 4: Open-label, multicentre, single arm trial to evaluate the efficacy and safety of eculizumab in children from 1 month of age with weight ≥ 5 kg to less than 18 years of age with Atypical Haemolytic Uraemic Syndrome. Study 5: Retrospective, observational, non-interventional trial to assess safety and efficacy of eculizumab in patients of any age with Atypical Haemolytic Uraemic Syndrome who received at least one dose of eculizumab outside an applicant's sponsored controlled trial.

3. Follow-up, completion and deferral of PIP

Measures to address long term follow-up of potential safety issues or efficacy in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By May 2013
Deferral for one or more studies contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Condition(s) and authorised indication(s):

Treatment of paroxysmal nocturnal haemoglobinuria

Authorised indication:

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.

EU Number	Invented name Name	Strength	Pharmaceutical form	Route of administration	Packaging	Content (concentration)	Package size
EU/1/07/393/001	Soliris	300 mg	concentrate for solution for infusion	Intravenous use	Vial (glass)	30 ml (10 mg/ml)	1 vial