EMA/827230/2016

# European Medicines Agency decision

P/0356/2016

of 21 December 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-PIP03-14) 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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# European Medicines Agency decision

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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 12 May 2014 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 11 November 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.

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.

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

***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 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, P/0164/2012 issued on 23 July 2012, P/0046/2016 issued on 26 February 2016, P/0201/2016 issued on 28 July 2016, P/0229/2016 issued on 01 September 2016 including subsequent modifications thereof.

***Article 5***

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

EMA/PDCO/590112/2016
London, 11 November 2016

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

EMEA-000876-PIP03-14

## 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

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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 12 May 2014 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 18 June 2014.

Supplementary information was provided by the applicant on 22 August 2016. 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 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**<br>Open-label, uncontrolled trial to evaluate safety and activity of eculizumab as add-on to background immunosuppressive therapy in children from 2 to less than 18 years of age with relapsing neuromyelitis optica spectrum disorders (NMOSD) |

| Area | Number of measures | Description |
|---|---|---|
| Extrapolation, modelling and simulation studies | 2 | **Study 2**<br>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<br>**Study 3**<br>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**<br>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 April 2020 |
| 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).

## Authorised pharmaceutical form(s):

Concentrate for solution for infusion

## Authorised route(s) of administration:

Intravenous use