EMA/2916/2017

European Medicines Agency decision

P/0034/2017

of 30 January 2017

on the agreement of a paediatric investigation plan and on the granting of a deferral for Fc- and CDR-modified humanized monoclonal antibody against C5 (EMEA-001943-PIP01-16) 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 22 February 2016 under Article 16(1) of Regulation (EC) No 1901/2006,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on London, 16 December 2016, in accordance with Article 18 of Regulation (EC) No 1901/2006 and Article 21 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.

(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.

[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 Fc- and CDR-modified humanized monoclonal antibody against C5, 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 Fc- and CDR-modified humanized monoclonal antibody against C5, 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

This decision is addressed to Alexion Europe SAS, 10-15 Avenue Edouard Belin, 92500 - Rueil-Malmaison, France.

EMA/PDCO/222217/2016 Corr
London, 16 December 2016

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

EMEA-001943-PIP01-16

Scope of the application

Active substance(s):

Fc- and CDR-modified humanized monoclonal antibody against C5

Condition(s):

Treatment of atypical haemolytic uremic 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

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 22 February 2016 an application for a paediatric investigation plan for the above mentioned medicinal product.

The procedure started on 29 March 2016.

Supplementary information was provided by the applicant on 26 September 2016. The applicant proposed modifications to the paediatric investigation plan and requested a deferral.

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.

   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 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 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

Not applicable.

2. Paediatric investigation plan

2.1. Condition

Treatment of atypical haemolytic uremic syndrome

2.1.1. Indication(s) targeted by the PIP

Treatment of atypical haemolytic uremic syndrome

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 measures	Description
Quality-related studies	0	Not applicable.
Non-clinical studies	0	Not applicable.
Clinical studies	2	Study 1: Open-label, single-arm study to evaluate pharmacokinetics, efficacy and safety of Fc- and CDR-modified humanized monoclonal antibody against C5 (ALXN1210), in adolescent (and adult) patients with atypical Haemolytic Uremic Syndrome (aHUS). Study 2: Open-label, single-arm study to evaluate the pharmacokinetics, pharmacodynamics, efficacy and safety of ALXN1210 in children from birth to less than 18 years of age with aHUS.
Extrapolation, modelling and simulation studies	1	Study 3: Modelling and simulation study to evaluate the use of ALXN1210 in aHUS in children from birth to less than 18 years of age.
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:	No
Date of completion of the paediatric investigation plan:	By March 2019
Deferral for one or more measures contained in the paediatric investigation plan:	Yes