

EMA/287178/2019

European Medicines Agency decision P/0199/2019

of 12 June 2019

on the acceptance of a modification of an agreed paediatric investigation plan for ravulizumab (ALXN1210) (EMA-001943-PIP01-16-M02) 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 European Medicines Agency's decision P/0034/2017 issued on 30 January 2017 and the decision P/0261/2018 issued on 15 August 2018,

Having regard to the application submitted by Alexion Europe SAS on 21 January 2019 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 26 April 2019, 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 ravulizumab (ALXN1210), 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 decision covers all conditions, indications, pharmaceutical forms, routes of administration, measures, timelines, waivers and deferrals, as agreed in the decision P/0199/2017 issued on 14 July 2017, including subsequent modifications thereof.

Article 3

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

EMA/PDCO/44129/2019
Amsterdam, 26 April 2019

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA-001943-PIP01-16-M02

Scope of the application

Active substance(s):

Ravulizumab (ALXN1210)

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 22 of Regulation (EC) No 1901/2006 as amended, Alexion Europe SAS submitted to the European Medicines Agency on 21 January 2019 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0034/2017 issued on 30 January 2017 and the decision P/0261/2018 issued on 15 August 2018.

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

The procedure started on 26 February 2019.

Scope of the modification

Some measures 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 Norwegian Paediatric Committee member agrees 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 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	1	<i>Study 1 (ALXN1210-aHUS-312) was deleted during procedure EMEA-001943-PIP01-16-M01.</i> 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. (ALXN1210-aHUS-312)
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 December 2020
Deferral for one or more measures contained in the paediatric investigation plan:	Yes