

EMA/591830/2019

European Medicines Agency decision

P/0379/2019

of 4 December 2019

on the acceptance of a modification of an agreed paediatric investigation plan for crizanlizumab (EMEA-002141-PIP01-17-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/0077/2018 issued on 16 March 2018 and the decision P/0060/2019 issued on 28 February 2019,

Having regard to the application submitted by Novartis Europharm Limited on 11 July 2019 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 18 October 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 crizanlizumab, 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 is addressed to Novartis Europharm Limited, Vista Building, Elm Park, Merrion Road, 4 Dublin, Ireland.

EMA/PDCO/410739/2019 Corr
Amsterdam, 18 October 2019

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA-002141-PIP01-17-M02

Scope of the application

Active substance(s):

Crizanlizumab

Condition(s):

Treatment of sickle cell disease

Pharmaceutical form(s):

Concentrate for solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Novartis Europharm Limited

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Novartis Europharm Limited submitted to the European Medicines Agency on 11 July 2019 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/0077/2018 issued on 16 March 2018 and the decision P/0060/2019 issued on 28 February 2019.

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

The procedure started on 20 August 2019.

Scope of the modification

Some measures and 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 Norwegian Paediatric Committee member agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the 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 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 sickle cell disease

The waiver applies to:

- the paediatric population from birth to less than 6 months of age;
- concentrate for solution for infusion, intravenous use;
- on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

2. Paediatric investigation plan

2.1. Condition:

Treatment of sickle cell disease

2.1.1. Indication(s) targeted by the PIP

Prevention of vaso-occlusive crises in patients with sickle cell disease

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

From 6 months 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	1	Study 1 Enhanced pre- and postnatal development toxicity study in cynomolgus monkeys
Clinical studies	1	Study 2 Open-label, uncontrolled trial to evaluate pharmacokinetics, pharmacodynamics and safety of crizanlizumab with or without hydroxyurea/ hydroxycarbamide in children from 6 months to less than 18 years of age with sickle cell disease with vaso-occlusive crisis (CSEG101B2201)

Extrapolation, modelling and simulation studies	2	Study 3 Modelling and simulation study to support the use of crizanlizumab in the prevention of vaso-occlusive crises in children from 6 months to less than 18 years of age with sickle cell disease Study 4 Extrapolation study to support the use of crizanlizumab in the prevention of vaso-occlusive crises in children from 6 months to less than 18 years of age with sickle cell disease
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:	Yes
Date of completion of the paediatric investigation plan:	By September 2023
Deferral for one or more measures contained in the paediatric investigation plan:	Yes