



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

EMA/6414/2024

## European Medicines Agency decision P/0004/2024

of 11 January 2024

on the acceptance of a modification of an agreed paediatric investigation plan and on the granting of a product-specific waiver for crizanlizumab (EMEA-002141-PIP01-17-M04) 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<sup>1</sup>,

Having regard to Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency<sup>2</sup>,

Having regard to the European Medicines Agency's decision P/0077/2018 issued on 16 March 2018, the decision P/0060/2019 issued on 28 February 2019, the decision P/0379/2019 issued on 4 December 2019, and the decision P/0533/2021 issued on 9 December 2021,

Having regard to the application submitted by Novartis Europharm Limited on 8 September 2023 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 15 December 2023, 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 and to the deferral and to the waiver.
- (2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan, including changes to the deferral and to the waiver.

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<sup>1</sup> OJ L 378, 27.12.2006, p.1, as amended.

<sup>2</sup> OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

**Article 1**

Changes to the agreed paediatric investigation plan for crizanlizumab, concentrate for solution for infusion, intravenous use, including changes to the deferral and to the waiver, 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.



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

EMA/PDCO/417819/2023  
Amsterdam, 15 December 2023

## Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan and on the granting of a product-specific waiver

EMA-002141-PIP01-17-M04

### Scope of the application

#### Active substance(s):

Crizanlizumab

#### Invented name and authorisation status:

See Annex II

#### 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 8 September 2023 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, the decision P/0060/2019 issued on 28 February 2019, the decision P/0379/2019 issued on 4 December 2019, and the decision P/0533/2021 issued on 9 December 2021.



The application for modification proposed changes to the agreed paediatric investigation plan and to the deferral and changes to the waiver to cover the remaining subsets of the paediatric population.

The procedure started on 16 October 2023.

## **Opinion**

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 and to the deferral and to amend the scope of the waiver in accordance with Article 11(1)(a) of said Regulation, on the grounds that the specific medicinal product is likely to be ineffective or unsafe in part or all of the paediatric population.

The Paediatric Committee member of Norway agrees with the above-mentioned recommendation of the Paediatric Committee.

This opinion is forwarded to the applicant and the Executive Director of the European Medicines Agency, together with its annexes and appendix.

## **Annex I**

### **Grounds for the granting of the waiver**

# **1. Waiver**

## ***1.1. Condition:***

Treatment of sickle cell disease

The waiver applies to:

- all subsets of the paediatric population from birth to less than 18 years of age;
- concentrate for solution for infusion, intravenous use;
- on the grounds that the specific medicinal product is likely to be ineffective.

## **Annex II**

### **Information about the authorised medicinal product**



***Information provided by the applicant:***

**The product is not authorised anywhere in the European Community.**