EMA/250309/2024

# European Medicines Agency decision

P/0198/2024

of 14 June 2024

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for ianalumab, (EMEA-002338-PIP05-23) 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 Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency[2],

Having regard to the application submitted by Novartis Europharm Limited on 28 June 2023 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 26 April 2024, in accordance with Article 17 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.

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

Has adopted this decision:

***Article 1***

A paediatric investigation plan for ianalumab, 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 ianalumab, 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 ianalumab, 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 is addressed to Novartis Europharm Limited, Vista building, Elm Park, Merrion Road, 4 – Dublin, Ireland.

EMA/PDCO/47388/2024 Corr[1]
Amsterdam, 26 April 2024

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

EMEA-002338-PIP05-23

## Scope of the application

**Active substance(s):**

Ianalumab

**Invented name and authorisation status:**

See Annex II

**Condition(s):**

Treatment of immune thrombocytopenia

**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 16(1) of Regulation (EC) No 1901/2006 as amended, Novartis Europharm Limited submitted for agreement to the European Medicines Agency on 28 June 2023 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 14 August 2023.

Supplementary information was provided by the applicant on 19 January 2024. The applicant proposed modifications to the paediatric investigation plan.

[1] 8 May 2024

## 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 17(1) 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 Paediatric Committee member of Norway 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 immune thrombocytopenia

The waiver applies to:

- the paediatric population from birth to less than 5 years 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 immune thrombocytopenia

### 2.1.1. Indication(s) targeted by the PIP

Treatment of paediatric patients aged 5 years and above with primary immune thrombocytopenia (ITP) who had an insufficient response to or relapsed after first-line corticosteroid therapy

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

From 5 years to less than 18 years of age

### 2.1.3. Pharmaceutical form(s)

Concentrate for solution for infusion

### 2.1.4. Measures

| **Area** | **Description** |
|---|---|
| Quality-related studies | Not applicable. |
| Non-clinical studies | Not applicable. |
| Clinical studies | **Study 1**<br>Open label, single-arm study to evaluate activity, pharmacokinetics and safety of ianalumab in addition to eltrombopag in paediatric patients from 5 years to less than 18 years of age with primary immune thrombocytopenia (ITP) who had an insufficient response to or relapsed after first-line corticosteroid therapy. |
| Modelling and simulation analyses | **Study 2**<br>Use of population pharmacokinetic-pharmacodynamic (PopPK/PD) and exposure-response (E-R) models, developed in adults, to define the |

| | |
|---|---|
| | initial paediatric dose(s) in Study 1, and subsequently to confirm and/or modify the paediatric posology based on the results of Study 1. |
| Other studies | Not applicable. |
| Extrapolation plan | Study CVAY736Q12301 in adults and paediatric Study 1 are a part of the extrapolation plan of efficacy data from adult to the paediatric population from 5 years to less than 18 years of age with immune thrombocytopenia (ITP). |

# 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 2031 |
| Deferral for one or more measures contained in the paediatric investigation plan: | Yes |

# Annex II

# Information about the authorised medicinal product

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

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