EMA/481244/2023

# European Medicines Agency decision

P/0450/2023

of 27 October 2023

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for iptacopan (EMEA-002705-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 20 March 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 13 October 2023, 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 iptacopan, capsule, hard, age-appropriate oral dosage form, oral 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 iptacopan, capsule, hard, age-appropriate oral dosage form, oral 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 iptacopan, capsule, hard, age-appropriate oral dosage form, oral 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, D04 A9N6 - Dublin 4, Ireland.

EMA/PDCO/333083/2023
Amsterdam, 13 October 2023

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

EMEA-002705-PIP05-23

## Scope of the application

**Active substance(s):**

Iptacopan

**Invented name and authorisation status:**

See Annex II

**Condition(s):**

Treatment of immune-complex mediated membranoproliferative glomerulonephritis

**Pharmaceutical form(s):**

Capsule, hard

Age-appropriate oral dosage form

**Route(s) of administration:**

Oral 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 20 March 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 24 April 2023.

Supplementary information was provided by the applicant on 3 July 2023. The applicant proposed modifications to the paediatric investigation plan.

## 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)(b) of said Regulation, on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified subset(s) of the paediatric population.

   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-complex mediated membranoproliferative glomerulonephritis

The waiver applies to:

- the paediatric population from birth to less than 2 years of age;
- capsule, hard; age-appropriate oral dosage form; oral use;
- on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

# 2. Paediatric investigation plan

## *2.1. Condition:*

Treatment of immune-complex mediated membranoproliferative glomerulonephritis

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

Treatment of idiopathic immune-complex mediated membranoproliferative glomerulonephritis (IC-MPGN)

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

From 2 years to less than 18 years of age

### 2.1.3. Pharmaceutical form(s)

Capsule, hard

Age-appropriate oral dosage form

### 2.1.4. Measures

| Area | Description |
|---|---|
| Quality-related studies | Study 1:<br>Development of an age-appropriate oral solid dosage form. |
| Non-clinical studies | Study 2:<br>52-week definitive toxicity study with 27-week recovery period in juvenile dogs (1870009) |
| Clinical studies | Study 3:<br>Double-blind, randomised, placebo-controlled trial to evaluate efficacy and safety of iptacopan in paediatric patients from 12 years |

| | |
|---|---|
| | to less than 18 years of age (and adults) with idiopathic IC-MPGN. (CLNP023B12302)<br><br>Study 4:<br><br>Open-label, single-arm trial to evaluate safety, tolerability and exposure of iptacopan in paediatric patients from 2 years to less than 12 years of age with idiopathic IC-MPGN. |
| Modelling and simulation studies | Study 5:<br><br>Modelling and simulation study to support dose finding for the clinical study in children from 2 years to less than 12 years of age with idiopathic IC-MPGN. |
| Other studies | Not applicable |
| Extrapolation plan | Studies 3, 4 and 5 are part of an extrapolation plan covering the paediatric population from 2 years to less than 12 years of age, as agreed by the PDCO. |

# 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 2029 |
| 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.**