

EMA/412202/2024

European Medicines Agency decision

P/0324/2024

of 13 September 2024

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for Iptacopan (Fabhalta), (EMA-002705-PIP06-23) 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 726/2004¹,

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 Agency²,

Having regard to the application submitted by Novartis Europharm Limited on 6 October 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 July 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 Iptacopan (Fabhalta), 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 (Fabhalta), 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 (Fabhalta), 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 covers all conditions, indications, pharmaceutical forms, routes of administration, measures, timelines, waivers and deferrals, as agreed in the decision P/0322/2021 issued on 12 August 2021, including subsequent modifications thereof.

Article 5

This decision is addressed to Novartis Europharm Limited, Vista Building Elm Park, Merrion Road, D04 A9N6 - Dublin 4, Ireland.

EMA/PDCO/223725/2024
Amsterdam, 26 July 2024

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

EMA-002705-PIP06-23

Scope of the application

Active substance(s):

Iptacopan

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of myasthenia gravis

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 6 October 2023 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and waiver under Article 13 of said Regulation.

The procedure started on 20 November 2023.

Supplementary information was provided by the applicant on 18 April 2024. 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)(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 myasthenia gravis

The waiver applies to:

- the paediatric population from birth to less than 2 years;
- capsule, hard; age-appropriate oral dosage form, oral 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 myasthenia gravis

2.1.1. Indication(s) targeted by the PIP

Treatment of generalised myasthenia gravis in children and adolescents aged 2 years to less than 18 years who are anti-acetylcholine receptor (AChR) antibody-positive

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: Development of age-appropriate formulation
Non-clinical studies	Study 2: Definitive juvenile animal toxicity study in dogs
Clinical studies	Study 3: Open label, single-arm study to evaluate the pharmacokinetics, pharmacodynamics, safety, tolerability, and exploratory efficacy of iptacopan in patients from 2

	years to less than 18 years of age with generalised myasthenia gravis (gMG)
Modelling and simulation analyses	Study 4: Modelling and simulation analyses (population PK) to establish the dose for Study 3 in paediatric patients from 2 years to less than 18 years of age with generalised myasthenia gravis (gMG) Study 5: Modelling and simulation analyses (population PK) to confirm the dose required for paediatric patients from 2 years to less than 18 years of age with generalised myasthenia gravis (gMG)
Other studies	Not applicable
Extrapolation plan	The phase III study planned in adult gMG patients (CLNP023Q12301), PIP Study 3, PIP Study 4 and PIP Study 5 are part of the extrapolation plan of efficacy data from adults covering the paediatric population from 2 years to less than 18 years of age with gMG

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 September 2032
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:

Condition(s) and authorised indication(s)

1. Treatment of paroxysmal nocturnal haemoglobinuria (PNH)

Authorised indication(s):

- FABHALTA is indicated as monotherapy in the treatment of adult patients with paroxysmal nocturnal haemoglobinuria (PNH) who have haemolytic anaemia.
- Invented name(s): FABHALTA
- Authorised pharmaceutical form(s): Hard capsules
- Authorised route(s) of administration: Oral Use
- Authorised via centralised procedure