

EMA/514061/2023

European Medicines Agency decision P/0531/2023

of 29 December 2023

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for inebilizumab (Uplizna), (EMA-001911-PIP03-23) 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 Horizon Therapeutics Ireland Designated Activity Company (DAC) on 20 January 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 10 November 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 inebilizumab (Uplizna), 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 inebilizumab (Uplizna), 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 inebilizumab (Uplizna), 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 covers all conditions, indications, pharmaceutical forms, routes of administration, measures, timelines, waivers and deferrals, as agreed in the decision P/0344/2016 issued on 2 December 2016, including subsequent modifications thereof.

Article 5

This decision is addressed to Horizon Therapeutics Ireland Designated Activity Company (DAC), 70 Saint Stephen's Green, D02 E2X4 - Dublin 2, Ireland.

EMA/PDCO/366122/2023
Amsterdam, 10 November 2023

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

EMA-001911-PIP03-23

Scope of the application

Active substance(s):

Inebilizumab

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of immunoglobulin G4-related disease

Pharmaceutical form(s):

Concentrate for solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Horizon Therapeutics Ireland Designated Activity Company (DAC)

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Horizon Therapeutics Ireland Designated Activity Company (DAC) submitted for agreement to the European Medicines Agency on 20 January 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 27 February 2023.

Supplementary information was provided by the applicant on 21 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)(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 immunoglobulin G4-related disease

The waiver applies to:

- the paediatric population from birth to less than 2 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 immunoglobulin G4-related disease

2.1.1. Indication(s) targeted by the PIP

Treatment of immunoglobulin G4-related disease (IgG4-RD)

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)

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 Open-label, uncontrolled trial to evaluate pharmacokinetics, pharmacodynamics and safety of inebilizumab in children from 2 years to less than 18 years of age with immunoglobulin G4-related disease (IgG4-RD).
Modelling and simulation studies	Study 2 Modelling and simulation study to support dose finding of inebilizumab in children from 2 years to less than 18 years of age with immunoglobulin G4-related disease (IgG4-RD).

Other studies	Not applicable
Extrapolation plan	Studies 1 and 2 are part of an extrapolation plan covering the paediatric population from 2 years to less than 18 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 March 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:

Condition(s) and authorised indication(s)

1. Treatment of neuromyelitis optica spectrum disorders (NMOSD)

Authorised indication(s):

- Uplizna is indicated as monotherapy for the treatment of adult patients with neuromyelitis optica spectrum disorders (NMOSD) who are anti-aquaporin 4 immunoglobulin G (AQP4-IgG) seropositive.
 - Invented name(s): Uplizna
 - Authorised pharmaceutical form(s): concentrate for solution for infusion
 - Authorised route of administration: intravenous use
 - Authorised via centralised procedure