EMADOC-1700519818-1776292

European Medicines Agency decision

EMA/PE/0000238005

of 27 November 2024

on the acceptance of a modification of an agreed paediatric investigation plan for inebilizumab (Uplinza) 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.

European Medicines Agency decision

EMA/PE/0000238005

of 27 November 2024

on the acceptance of a modification of an agreed paediatric investigation plan for inebilizumab (Uplinza) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of 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 European Medicines Agency's decision P/0531/2023 issued on 29 December 2023 and the decision P/0217/2024 issued on 14 June 2024,

Having regard to the application submitted by Horizon Therapeutics Ireland DAC on 19 November 2024 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 25 November 2024, 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.

(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.

[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

Changes to the agreed paediatric investigation plan for inebilizumab (Uplinza), including changes to the deferral, 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 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 3

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

EMADOC-1700519818-1776252
Amsterdam, 25 November 2024

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan

EMA/PE/0000238005

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 DAC

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Horizon Therapeutics Ireland DAC submitted to the European Medicines Agency on 19 November 2024 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/0531/2023 issued on 29 December 2023 and the decision P/0217/2024 issued on 14 June 2024.

The application for modification proposed changes to the agreed paediatric investigation plan and to the deferral.

The procedure started on 20 November 2024.

Scope of the modification

Some measures or timelines of the Paediatric Investigation Plan have been modified.

Opinion

1. 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 in the scope set out in the Annex I of this opinion.

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

2. The measures and timelines of the 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