EMA/554681/2023

European Medicines Agency decision

P/0513/2023

of 29 December 2023

on the acceptance of a modification of an agreed paediatric investigation plan for inebilizumab (Uplizna), (EMEA-001911-PIP01-15-M05) 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 European Medicines Agency's decision P/0344/2016 issued on 2 December 2016, the decision P/0136/2018 issued on 7 May 2018, the decision P/0129/2019 issued on 17 April 2019, the decision P/0428/2020 issued on 28 October 2020 and the decision P/0213/2022 issued on 10 June 2022,

Having regard to the application submitted by Horizon Therapeutics Ireland DAC on 4 August 2023 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 10 November 2023, 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 (Uplizna), concentrate for solution for infusion, solution for infusion, intravenous use, 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 is addressed to Horizon Therapeutics Ireland DAC, 70 St. Stephen's Green, D02 E2X4 - Dublin 2, Ireland.

EMA/PDCO/365763/2023
Amsterdam, 10 November 2023

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

EMEA-001911-PIP01-15-M05

Scope of the application

Active substance(s):

Inebilizumab

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of neuromyelitis optica spectrum disorders

Pharmaceutical form(s):

Concentrate for solution for infusion

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 4 August 2023 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/0344/2016 issued on 2 December 2016, the decision P/0136/2018 issued on 7 May 2018, the decision P/0129/2019 issued on 17 April 2019, the decision P/0428/2020 issued on 28 October 2020 and the decision P/0213/2022 issued on 10 June 2022.

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

The procedure started on 11 September 2023.

Scope of the modification

Some 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 neuromyelitis optica spectrum disorders

The waiver applies to:

- the paediatric population from birth to less than 2 years of age;
- solution for infusion, concentrate for solution for infusion, intravenous use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

2. Paediatric investigation plan

2.1. Condition:

Treatment of neuromyelitis optica spectrum disorders

2.1.1. Indication(s) targeted by the PIP

Maintenance treatment of patients with neuromyelitis optica spectrum disorders (NMOSD)

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)

Solution for infusion

Concentrate for solution for infusion

2.1.4. Measures

Area	Description
Quality-related studies	**Study 1** Deleted during procedure EMEA-001911-PIP01-15-M04
Non-clinical studies	**Study 2** Reprotox: enhanced pre- and postnatal development in mice
Clinical studies	**Study 3 (VIB0551.P2.S2)** Open-label, uncontrolled trial to evaluate PK/PD and safety of inebilizumab in children from 2 to less than 18 years of age with neuromyelitis optica spectrum disorders

Extrapolation, modelling and simulation studies	**Study 4** Modelling and simulation study to determine the dose of inebilizumab in the treatment of neuromyelitis optica spectrum disorders in children from 2 to less than 18 years of age **Study 5** Analysis of existing data on efficacy, safety, pharmacodynamics and pharmacokinetics of inebilizumab to evaluate the use of the product in the treatment of neuromyelitis optica spectrum disorders in children from 2 to less than 18 years of age
Other studies	Not applicable.
Other measures	Not applicable.

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 2027
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

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(s) of administration: Intravenous use
  - Authorised via centralised procedure