

EMA/246251/2024

## European Medicines Agency decision P/0206/2024

of 14 June 2024

on the acceptance of a modification of an agreed paediatric investigation plan for inebilizumab (Uplizna), (EMEA-001911-PIP02-22-M01) 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

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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<sup>1</sup>,

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<sup>2</sup>,

Having regard to the European Medicines Agency's decision P/0471/2023 issued on 1 December 2023,

Having regard to the application submitted by Horizon Therapeutics Ireland Designated Activity Company (DAC) on 19 January 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 26 April 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.
- (2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

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<sup>1</sup> OJ L 378, 27.12.2006, p.1, as amended.

<sup>2</sup> 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, intravenous use, 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 Designated Activity Company (DAC), 70 Saint Stephen's Green, D02 E2X4 - Dublin 2, Ireland.

EMA/PDCO/46981/2024  
Amsterdam, 26 April 2024

## Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA-001911-PIP02-22-M01

### Scope of the application

**Active substance(s):**

Inebilizumab

**Invented name and authorisation status:**

See Annex II

**Condition(s):**

Treatment of myasthenia gravis

**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 22 of Regulation (EC) No 1901/2006 as amended, Horizon Therapeutics Ireland Designated Activity Company (DAC) submitted to the European Medicines Agency on 19 January 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/0471/2023 issued on 1 December 2023.

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

The procedure started on 26 February 2024.

### Scope of the modification

Some measures 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 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 myasthenia gravis

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 myasthenia gravis

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

Treatment of myasthenia gravis

### 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                 | <b>Study 1</b><br>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 juvenile myasthenia gravis. |
| Modelling and simulation studies | <b>Study 2</b><br>Modelling and simulation study to support dose finding of inebilizumab in children from 2 years to less than 18 years of age with juvenile myasthenia gravis.                                   |
| 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 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:***

**Condition(s) and authorised indication(s)**

1. Treatment of neuromyelitis optica spectrum disorders

Authorised indication(s):

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