

EMA/21206/2023

## European Medicines Agency decision P/0028/2023

of 31 January 2023

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for satralizumab (Enspryng), (EMA-001625-PIP04-22) 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

P/0028/2023

of 31 January 2023

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for satralizumab (Enspryng), (EMA-001625-PIP04-22) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

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 application submitted by Roche Registration GmbH on 17 March 2022 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 16 December 2022, 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.

---

<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**

A paediatric investigation plan for satralizumab (Enspryng), solution for injection, subcutaneous 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 satralizumab (Enspryng), solution for injection, subcutaneous 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 satralizumab (Enspryng), solution for injection, subcutaneous 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/0154/2015 issued on 10 July 2015, including subsequent modifications thereof.

**Article 5**

This decision is addressed to Roche Registration GmbH, Emil-Barell-Strasse 1, 79639 - Grenzach-Wyhlen, Germany.

EMA/PDCO/781881/2022  
Amsterdam, 16 December 2022

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

EMA-001625-PIP04-22

### Scope of the application

**Active substance(s):**

Satralizumab

**Invented name and authorisation status:**

See Annex II

**Condition(s):**

Treatment of autoimmune encephalitis

**Pharmaceutical form(s):**

Solution for injection

**Route(s) of administration:**

Subcutaneous use

**Name/corporate name of the PIP applicant:**

Roche Registration GmbH

### Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Roche Registration GmbH submitted for agreement to the European Medicines Agency on 17 March 2022 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 25 April 2022.

Supplementary information was provided by the applicant on 12 September 2022. 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)(b) of said Regulation, on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified subset(s) of the paediatric population.

The Paediatric Committee member of Liechtenstein and Norway agree 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 autoimmune encephalitis

The waiver applies to:

- the paediatric population from birth to less than 2 years of age;
- solution for injection, subcutaneous use;
- on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

# 2. Paediatric investigation plan

## 2.1. Condition:

Treatment of autoimmune encephalitis

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

Treatment of N-methyl-D-aspartic acid receptor (NMDAR) autoimmune encephalitis (AIE)

### 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 injection

### 2.1.4. Measures

Area	Description
Quality-related studies	<b>Study 1</b> Development of solution for injection for subcutaneous use appropriate for the paediatric population from 2 years of age.
Non-clinical studies	<b>Study 2</b> Evaluation of effects of satralizumab on enhanced pre- and postnatal development in cynomolgus monkeys. (CH5333787)
Clinical studies	<b>Study 3</b> Randomised, double-blind, placebo-controlled trial to evaluate pharmacokinetics, pharmacodynamics, safety and efficacy of satralizumab compared to placebo in children from 12 years to less than 18 years of age (and adults) with N-methyl-D-aspartic acid receptor (NMDAR) autoimmune encephalitis (AIE). (WN43174)

	<b>Study 4</b> Clinical trial to evaluate pharmacokinetics, safety and tolerability of satralizumab in children from 2 years to less than 12 years of age with NMDAR AIE.
Modelling and simulation studies	<b>Study 5</b> Modelling and simulation study to predict the appropriate paediatric dose in children from 2 years to less than 12 years of age with NMDAR AIE.
Other studies	Not applicable
Extrapolation plan	Data from adults from Study 3 (WN43174) 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:	Yes
Date of completion of the paediatric investigation plan:	By June 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

Authorised indication(s):

- Enspryng is indicated as a monotherapy or in combination with immunosuppressive therapy (IST) for the treatment of neuromyelitis optica spectrum disorders (NMOSD) in adult and adolescent patients from 12 years of age who are anti-aquaporin-4 IgG (AQP4-IgG) seropositive.
  - Invented name(s): Enspryng 120 mg solution for injection in pre-filled syringe
  - Authorised pharmaceutical form(s): Solution for injection
  - Authorised route(s) of administration: Subcutaneous use
  - Authorised via centralised procedure