EMA/554543/2023

European Medicines Agency decision

P/0519/2023

of 29 December 2023

on the acceptance of a modification of an agreed paediatric investigation plan for satralizumab (Enspyng), (EMEA-001625-PIP01-14-M07) 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/0154/2015 issued on 10 July 2015, the decision P/0026/2017 issued on 27 January 2017, the decision P/0061/2019 issued on 8 March 2019, the decision P/0220/2019 issued on 17 June 2019, the decision P/0204/2020 issued on 16 June 2020 and the decision P/0477/2020 issued on 1 December 2020,

Having regard to the application submitted by Roche Registration GmbH on 11 September 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 satralizumab (Enspyng), solution for injection, subcutaneous 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 Roche Registration GmbH, 1 Emil-Barell-Strasse, 79639 - Grenzach-Wyhlen, Germany.

EMA/PDCO/423794/2023
Amsterdam, 10 November 2023

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

EMEA-001625-PIP01-14-M07

Scope of the application

Active substance(s):

Satralizumab

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of neuromyelitis optica

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 22 of Regulation (EC) No 1901/2006 as amended, Roche Registration GmbH submitted to the European Medicines Agency on 11 September 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/0154/2015 issued on 10 July 2015, the decision P/0026/2017 issued on 27 January 2017, the decision P/0061/2019 issued on 8 March 2019, the decision P/0220/2019 issued on 17 June 2019, the decision P/0204/2020 issued on 16 June 2020 and the decision P/0477/2020 issued on 1 December 2020.

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

The procedure started on 16 October 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

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 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

2.1.1. Indication(s) targeted by the PIP

Treatment of neuromyelitis optica spectrum disorders

2.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From 2 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	**Study 1** Development of solution for injection for subcutaneous use appropriate for the paediatric population from 2 years of age
Non-clinical studies	**Study 2** Reprotox enhanced pre- and postnatal development study in cynomolgus monkeys
Clinical studies	**Study 3** Double-blind, randomised, placebo-controlled trial to evaluate pharmacokinetics, safety and efficacy of satralizumab as add-on to baseline immunosuppressant therapy in children from 12 to less than 18 years of age (and in adults) with relapsing neuromyelitis optica and neuromyelitis optica spectrum disorders (NMO/NMOSD)

	Study 7 Open-label, uncontrolled trial to evaluate pharmacokinetics, safety and efficacy of satralizumab in adolescents from 12 to less than 18 years of age (and adults) with neuromyelitis optica spectrum disorders (NMOSD) **Study 4** Open-label, uncontrolled trial to evaluate pharmacokinetics, activity and safety of satralizumab in children from 2 to less than 12 years of age with positive AQP4 IgG serostatus neuromyelitis optica spectrum disorders (NMOSD)
Extrapolation, modelling and simulation studies	**Study 5** Modelling and simulation study to evaluate the dose of satralizumab in the treatment of relapsing NMO/NMOSD in children from 2 to less than 12 years of age **Study 8** Analysis of existing data on efficacy, safety and pharmacokinetics of satralizumab to evaluate the use of the product in the treatment of relapsing NMO/NMOSD in children from 12 to less than 18 years of age **Study 6** Analysis of existing data on efficacy, safety and pharmacokinetics of satralizumab to evaluate the use of the product in the treatment of NMOSD in children from 2 to less than 12 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 November 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

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): Enspyng
  - Authorised pharmaceutical form(s): solution for injection
  - Authorised route(s) of administration: subcutaneous use
  - Authorised via centralised procedure