EMADOC-1700519818-1976710

European Medicines Agency decision

EMA/PE/0000232315

of 19 March 2025

on the acceptance of a modification of an agreed paediatric investigation plan for spesolimab (Spevigo) 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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on the acceptance of a modification of an agreed paediatric investigation plan for spesolimab (Spevigo) 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[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/0247/2023 issued on 14 July 2023 and the decision P/0341/2024 issued on 13 September 2024,

Having regard to the application submitted by Boehringer Ingelheim International GmbH on 18 October 2024 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 31 January 2025, 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.

[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 spesolimab (Spevigo), 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 Boehringer Ingelheim International GmbH, Binger Strasse 173, 55216 Ingelheim Am Rhein Rhineland-Palatinate, Germany.

EMADOC-1700519818-1758906
Amsterdam, 31 January 2025

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

EMA/PE/0000232315

Scope of the application

Active substance(s):

Spesolimab

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of Netherton syndrome

Pharmaceutical form(s):

Solution for injection

Solution for infusion

Route(s) of administration:

Subcutaneous use

Intravenous use

Name/corporate name of the PIP applicant:

Boehringer Ingelheim International GmbH

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Boehringer Ingelheim International GmbH submitted to the European Medicines Agency on 18 October 2024 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0247/2023 issued on 14 July 2023 and the decision P/0341/2024 issued on 13 September 2024.

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

The procedure started on 25 November 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 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

Not applicable

2. Paediatric investigation plan

2.1. Condition:

Treatment of Netherton syndrome

2.1.1. Indication(s) targeted by the PIP

Treatment of patients with Netherton syndrome

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

From birth to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Solution for injection

Solution for infusion

2.1.4. Measures

Area	**Description**
Quality-related studies	Not applicable
Non-clinical studies	Not applicable
Clinical studies	Study 1 (1368-0104) Double-blind, randomised, placebo-controlled trial to evaluate pharmacokinetics, safety, and efficacy of spesolimab in children from 12 years to less than 18 years of age (and adults) with Netherton syndrome, with an open label extension to evaluate long term safety and efficacy Study 2 (1368-NS-Ped1) Open-label non-randomised uncontrolled trial to evaluate safety, pharmacokinetics and immunogenicity of spesolimab in children from birth to less than 12 years of age with Netherton syndrome.
Modelling and simulation studies	Study 3 Modelling and simulation study to evaluate the adequacy of dose of spesolimab in the treatment of Netherton syndrome in children from 12 years to less than 18 years of age with Netherton syndrome.

	Study 4 Modelling and simulation study to inform the design of Study 2 (spesolimab in the treatment of Netherton syndrome in children from birth to less than 12 years of age with Netherton syndrome) Study 5 Modelling and simulation study to evaluate the adequacy of dose of spesolimab in the treatment of Netherton syndrome in children from birth to less than 12 years of age with Netherton syndrome.
Other studies	Not applicable
Extrapolation plan	Studies 1, 2 ,3, 4, 5 are part of an extrapolation plan covering the paediatric population from birth to less than 12 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 September 2033
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 Generalised Pustular Psoriasis (GPP)

Authorised indication(s):

- Spevigo is indicated for the treatment of flares in adult patients with generalised pustular psoriasis (GPP) as monotherapy.
  - Invented name(s): Spevigo
  - Authorised pharmaceutical form(s): concentrate for solution for infusion (sterile concentrate)
  - Authorised route(s) of administration: intravenous infusion
  - Authorised via centralised procedure