EMADOC-1700519818-1736438

European Medicines Agency decision

EMA/PE/0000221709

of 7 November 2024

on the acceptance of a modification of an agreed paediatric investigation plan for apitegromab 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 apitegromab 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/0014/2024 issued on 31 January 2024,

Having regard to the application submitted by Yes Pharmaceutical Development Services GmbH on 8 July 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 18 October 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 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 apitegromab, 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 Yes Pharmaceutical Development Services GmbH, Basler Strasse 7, Bad Homburg, 61352- Hesse, Germany.

EMADOC-1700519818-1613610
Amsterdam, 18 October 2024

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

EMA/PE/0000221709

Scope of the application

Active substance(s):

Apitegromab

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of spinal muscular atrophy

Pharmaceutical form(s):

Solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Yes Pharmaceutical Development Services GmbH

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Yes Pharmaceutical Development Services GmbH submitted to the European Medicines Agency on 8 July 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/0014/2024 issued on 31 January 2024.

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

The procedure started on 19 August 2024.

Scope of the modification

Some measures and 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

Treatment of spinal muscular atrophy

The waiver applies to:

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

1.1. Condition:

Treatment of spinal muscular atrophy

2. Paediatric investigation plan

2.1. Condition:

Treatment of spinal muscular atrophy

2.1.1. Indication(s) targeted by the PIP

Treatment of spinal muscular atrophy (SMA)

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

From 2 months to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Solution for infusion

2.1.4. Measures

Area	**Description**
Quality-related studies	Not applicable
Non-clinical studies	**Study 1** Prenatal and Postnatal Development study including maternal function with apitegromab in rats
Clinical studies	**Study 2 (SRK-015-002 (TOPAZ))** Open-label, uncontrolled trial to evaluate safety and activity of apitegromab in children from 2 years to less than 18

	years of age (and adults) with spinal muscular atrophy (type 2 and type 3 SMA) **Study 3 (SRK-015-003 (SAPPHIRE))** Double-blind, randomised, placebo-controlled trial to evaluate safety, tolerability and efficacy of apitegromab in children from 2 years to less than 18 years of age with spinal muscular atrophy (type 2 and type 3 SMA). **Study 4 (SRK-015-005)** Double-blind, randomised trial to evaluate pharmacokinetics, safety and efficacy of 2 different doses of apitegromab in children from 2 months to less than 2 years of age with spinal muscular atrophy (type 1 and pre-symptomatic SMA).
Modelling and simulation studies	**Study 5** Modelling and simulation study to support dose finding of apitegromab in children from 2 months to less than 2 years of age with spinal muscular atrophy (type 1 SMA). **Study 6** Modelling and simulation study to support final dosing recommendations for apitegromab in the treatment of SMA in children from 2 months to less than 2 years of age
Other studies	Not applicable
Extrapolation plan	Not applicable

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 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:

The product is not authorised anywhere in the European Community.