EMADOC-1700519818-2039917

European Medicines Agency decision

EMA/PE/0000228269

of 15 April 2025

on the acceptance of a modification of an agreed paediatric investigation plan for garetosmab 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 garetosmab 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/0103/2024 issued on 12 April 2024,

Having regard to the application submitted by Regeneron Ireland Designated Activity Company on 22 November 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 28 February 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 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.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for garetosmab, 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 Regeneron Ireland Designated Activity Company, One Warrington Place, Dublin 2 - D02 HH27, Ireland.

[1] OJ L 378, 27.12.2006, p.1, as amended.
[2] OJ L 136, 30.4.2004, p. 1, as amended.

EMADOC-1700519818-1797859
Amsterdam, 28 February 2025

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

EMA/PE/0000228269

Scope of the application

Active substance(s):

Garetosmab

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of fibrodysplasia ossificans progressiva

Pharmaceutical form(s):

Solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Regeneron Ireland Designated Activity Company

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Regeneron Ireland Designated Activity Company submitted to the European Medicines Agency on 22 November 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/0103/2024 issued on 12 April 2024.

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

The procedure started on 2 January 2025.

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

1.1. Condition:

Treatment of fibrodysplasia ossificans progressiva

The waiver applies to:

- the paediatric population from birth to less than 2 years of age;
- solution for infusion, intravenous use;
- on the grounds that the specific medicinal product is likely to be unsafe.

2. Paediatric investigation plan

2.1. Condition:

Treatment of fibrodysplasia ossificans progressiva

2.1.1. Indication(s) targeted by the PIP

Treatment of fibrodysplasia ossificans progressiva (FOP)

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 infusion

2.1.4. Measures

Area	Description
Quality-related studies	Not applicable.
Non-clinical studies	Not applicable.
Clinical studies	**Study 1** (R2477-FOP-2413 Part A) Randomised, placebo-controlled study to assess the safety, tolerability, pharmacokinetics, and efficacy of garetosmab on heterotopic bone formation in adolescents from 12 years to less than 18 years of age with fibrodysplasia ossificans progressiva. **Study 2** (R2477-FOP-2413 Part B) Randomised, placebo-controlled study to assess the safety, tolerability, pharmacokinetics, and efficacy of garetosmab on heterotopic bone formation in children from 2 years to

	less than 12 years of age with fibrodysplasia ossificans progressiva.
Modelling and simulation analyses	**Study 3** (R2477-FOP-2413 PopPK Part A) Modelling and simulation analyses to support dose selection of garetosmab in adolescents from 12 years to less than 18 years of age with fibrodysplasia ossificans progressiva. **Study 4** (R2477-FOP-2413 PopPK Part B) Modelling and simulation analyses to support dose selection of garetosmab in children from 2 years to less than 12 years of age with fibrodysplasia ossificans progressiva.
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:	Yes
Date of completion of the paediatric investigation plan:	By November 2028
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.