MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
United Kingdom

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Decision Cover Letter

Decision of the licensing authority to:

agree a paediatric investigation plan and grant a deferral and grant a waiver

MHRA-101469-PIP01-24

Scope of the Application

Active Substance(s)

Garetosmab

Condition(s)

Treatment of fibrodysplasia ossificans progressiva (FOP)

Pharmaceutical Form(s)

Solution for infusion

Route(s) of Administration

INTRAVENOUS USE

Name / Corporate name of the PIP applicant

Regeneron UK Ltd

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Regeneron UK Ltd submitted to the licensing authority on 03/07/2024 15:02 BST an application for a Paediatric Investigation Plan

The procedure started on 09/09/2024 14:41 BST

1. The licensing authority, having assessed the application in accordance with the Human Medicines Regulations 2012, decides, as set out in the appended summary report:

to agree a paediatric investigation plan and grant a deferral and grant a waiver

2. The measures and timelines of the paediatric investigation plan are set out in the Annex I.

This decision is forwarded to the applicant, together with its annex and appendix.

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Final Decision Letter

MHRA-101469-PIP01-24

Of 23/10/2024 17:32 BST

On the adopted decision for Garetosmab (MHRA-101469-PIP01-24) in accordance with the Human Medicines Regulations 2012.

The licensing authority, in accordance with the Human Medicines Regulations 2012, has adopted this decision:

Agreement on a paediatric investigation plan

This decision applies to a Paediatric Investigation Plan for Garetosmab, Solution for infusion , INTRAVENOUS USE .

This decision is addressed to Regeneron UK Ltd, The Charter Building, Vine Street, Uxbridge, United Kingdom, London, UNITED KINGDOM, UB8 1JG

ANNEX I

1. Waiver

1.1 Condition:

Treatment of fibrodysplasia ossificans progressiva (FOP) The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 years of age Pharmaceutical form(s): Solution for infusion Route(s) of administration: INTRAVENOUS USE Reason for granting waiver: on the grounds that the specific medicinal product is likely to be unsafe.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of fibrodysplasia ossificans progressiva

2.2 Indication(s) targeted by the PIP:

Treatment of fibrodysplasia ossificans progressiva

2.3 Subset(s) of the paediatric population concerned by the paediatric development:

The paediatric population from 2 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

Solution for infusion

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	2	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.
Extrapolation, Modeling & Simulation Studies	2	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	0	Not applicable.
Other Measures	0	Not applicable.

3. Follow-up, completion and deferral of a PIP:

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	30/11/2028
Deferral of one or more studies contained in the paediatric investigation plan:	Yes