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

MHRA-101235-PIP01-23

Scope of the Application

Active Substance(s)

Humanised monoclonal antibody derivative against fibroblast growth factor receptor 3

Condition(s)

Treatment of achondroplasia

Pharmaceutical Form(s)

Solution for injection

Route(s) of Administration

SUBCUTANEOUS USE

Name / Corporate name of the PIP applicant

Sanofi B.V.

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Sanofi B.V. submitted to the licensing authority on 14/11/2023 10:06 GMT an application for a Paediatric Investigation Plan

The procedure started on 12/02/2024 07:12 GMT

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

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-101235-PIP01-23

Of 05/03/2024 10:01 GMT

On the adopted decision for Humanised monoclonal antibody derivative against fibroblast growth factor receptor 3 (MHRA-101235-PIP01-23) 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 Humanised monoclonal antibody derivative against fibroblast growth factor receptor 3, Solution for injection , SUBCUTANEOUS USE .

This decision is addressed to Sanofi B.V., Paasheuvelweg 25, Amsterdam, NETHERLANDS, 1105 BP

ANNEX I

1. Waiver

1.1 Condition:

Not applicable

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of achondroplasia

2.2 Indication(s) targeted by the PIP:

Treatment of achondroplasia

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

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

2.4 Pharmaceutical Form(s):

Solution for injection

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 (FOR2337) Development of a concentrated formulation for subcutaneous administration to address dosing regimen in the paediatric population from birth.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	4	Study 2 (OBS16647) Non-interventional trial to evaluate natural history of achondroplasia caused by FGFR3 mutations in children from birth to less than 11 years of age. Study 3 (DRI16646) Open-label, multi-centre, dose escalation study to evaluate pharmacokinetics, safety, tolerability, and activity of SAR442501 in children from birth to less than 12 years of age with genetically confirmed achondroplasia. Study 4 Randomised, controlled trial to evaluate safety, efficacy, immunogenicity and pharmacokinetics of SAR442501 in children from birth to less than 18 years of age with genetically confirmed achondroplasia. Study 5 (LTS17280) Open label, uncontrolled long term extension trial to evaluate safety, activity, immunogenicity and pharmacokinetics of SAR442501 in children from 1 year to less than 18 years of age with genetically confirmed achondroplasia.
Extrapolation, Modeling & Simulation Studies	2	Study 6 Modelling and simulation population PK study, to predict the dose in the treatment of achondroplasia in children from birth to less than 18 years of age. Study 7

		Modelling and simulation population PK/PD study, to evaluate the use of the product in the treatment of achondroplasia in children from birth to less than 18 years of age.
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:	31/12/2030
Deferral of one or more studies contained in the paediatric investigation plan:	Yes