EMA/364885/2024

European Medicines Agency decision

P/0304/2024

of 16 August 2024

on the acceptance of a modification of an agreed paediatric investigation plan for humanised monoclonal antibody derivative against fibroblast growth factor receptor 3 (SAR442501) (EMEA-003253-PIP01-22-M01), 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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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/0348/2023 issued on 8 September 2023,

Having regard to the application submitted by Sanofi B.V on 22 March 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 28 June 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.

(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 humanised monoclonal antibody derivative against fibroblast growth factor receptor 3 (SAR442501), solution for injection, subcutaneous use, 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 Sanofi B.V, Paasheuvelweg 25, 1105 BP – Amsterdam, The Netherlands.

EMA/PDCO/145850/2024
Amsterdam, 28 June 2024

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

EMEA-003253-PIP01-22-M01

Scope of the application

Active substance(s):

Humanised monoclonal antibody derivative against fibroblast growth factor receptor 3 (SAR442501)

Invented name and authorisation status:

See Annex II

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 opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Sanofi B.V. submitted to the European Medicines Agency on 22 March 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/0348/2023 issued on 8 September 2023.

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

The procedure started on 29 April 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 achondroplasia

2.1.1. Indication(s) targeted by the PIP

Treatment of achondroplasia (ACH)

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

2.1.4. Measures

Area	Description
Quality-related studies	**Study 1 (FOR2337)** Development of a concentrated formulation for subcutaneous administration to address dosing regimen in the paediatric population from birth.
Non-clinical studies	Not applicable.
Clinical studies	**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.
Modelling and simulation studies	**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	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 December 2030
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.