

EMA/481452/2024

European Medicines Agency decision

P/0360/2024

of 25 October 2024

on the acceptance of a modification of an agreed paediatric investigation plan for fidrisertib, (EMA-003133-PIP01-21-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 726/2004¹,

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 Agency²,

Having regard to the European Medicines Agency's decision P/0271/2022 issued on 10 August 2022,

Having regard to the application submitted by Ipsen Pharma on 31 May 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 6 September 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 fidrisertib, capsule, hard, oral 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 Ipsen Pharma, 65 Quai Georges Gorse ,92100 - Boulogne-Billancourt, France.

EMA/PDCO/280596/2024
Amsterdam, 6 September 2024

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA-003133-PIP01-21-M01

Scope of the application

Active substance(s):

Fidrisertib

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of fibrodysplasia ossificans progressiva

Pharmaceutical form(s):

Capsule, hard

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Ipsen Pharma

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Ipsen Pharma submitted to the European Medicines Agency on 31 May 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/0271/2022 issued on 10 August 2022.

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

The procedure started on 8 July 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

1.1. Condition:

Treatment of fibrodysplasia ossificans progressiva

The waiver applies to:

- the paediatric population from birth to less than 2 years of age;
- capsule, hard, oral 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

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)

Capsule, hard

2.1.4. Measures

Area	Description
Quality-related studies	Study 1 Development of an age-appropriate oral solid dosage form.
Non-clinical studies	Not applicable.
Clinical studies	Study 2 (D-CA-60130-452) Double-blind, randomised, placebo-controlled study with 36-month open-label extension to evaluate the efficacy and safety of oral IPN60130 in paediatric patients from 2 years of age to less than 18 years of age (and adults) with fibrodysplasia ossificans progressiva.

Extrapolation, modelling and simulation studies	Study 3 (D-CA-60130-452 POP-PK and PK/PD modelling) Population pharmacokinetic (PK) modelling study to describe the PK profile of IPN60130 in children and adolescents from 2 years to under 18 years of age with fibrodysplasia ossificans progressive.
Other studies	Not applicable.
Other measures	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 June 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.