EMA/291519/2024

European Medicines Agency decision

P/0225/2024

of 5 July 2024

on the acceptance of a modification of an agreed paediatric investigation plan for setrusumab (EMEA-002169-PIP01-17-M03) 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.

European Medicines Agency decision

P/0225/2024

of 5 July 2024

on the acceptance of a modification of an agreed paediatric investigation plan for setrusumab (EMEA-002169-PIP01-17-M03) 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/0241/2018 issued on 15 August 2018, and the decision P/0536/2022 issued on 30 December 2022,

Having regard to the application submitted by Mereo BioPharma Ireland Limited on 23 February 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 31 May 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 setrusumab, powder for solution for injection, intravenous 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 Mereo BioPharma Ireland Limited, 6 Lapp's Quay, T12 TA48 – Cork, Ireland.

EMA/PDCO/90763/2024
Amsterdam, 31 May 2024

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

EMEA-002169-PIP01-17-M03

Scope of the application

Active substance(s):

Setrusumab

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of osteogenesis imperfecta

Pharmaceutical form(s):

Powder for solution for injection

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Mereo BioPharma Ireland Limited

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Mereo BioPharma Ireland Limited submitted to the European Medicines Agency on 23 February 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/0241/2018 issued on 15 August 2018, and the decision P/0536/2022 issued on 30 December 2022.

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

The procedure started on 2 April 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.
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 osteogenesis imperfecta

The waiver applies to:

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

2. Paediatric investigation plan

2.1. Condition:

Treatment of osteogenesis imperfecta

2.1.1. Indication(s) targeted by the PIP

Treatment of osteogenesis imperfecta

2.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From 2 to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Powder for solution for injection

2.1.4. Measures

Area	Description
Quality-related studies	Not applicable
Non-clinical studies	**Study 1** Dose range-finding juvenile toxicity study **Study 2** Definitive juvenile toxicity study
Clinical studies	**Study 3** Randomised, double-blind, placebo-controlled parallel group study (adaptive design) to select a dose and to assess the efficacy, safety and pharmacokinetics of intravenous setrusumab in growing paediatric patients from 5 years to less than 18 years (and 18 to less than 26) years of age with severe osteogenesis imperfecta (OI) Type I, III, or IV (UX143-CL301)

	Study 4 Randomised, open-label, active-controlled, parallel group, study to assess the efficacy, safety and pharmacokinetics of intravenous setrusumab compared with standard-of-care intravenous bisphosphonates in paediatric subjects 2 to less than 7 years of age with OI Types I, III, or IV confirmed by COL1A1 or COL1A2 mutation (UX-143-CL314) **Study 5** Study deleted in EMEA-002169-PIP01-17-M02
Extrapolation, modelling and simulation studies	**Study 6** Modelling and simulation study to support the use of setrusumab for the treatment of osteogenesis imperfecta in children from 2 years to less than 18 years of age
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 October 2026
Deferral for one or more measures contained in the paediatric investigation plan:	No

Annex II

Information about the authorised medicinal product

The product is not authorised anywhere in the European Community.