

EMA/121686/2024

European Medicines Agency decision P/0116/2024

of 12 April 2024

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for losmapimod (EMA-003448-PIP01-23) 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 application submitted by Fulcrum Therapeutics, Inc. on 23 May 2023 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 23 February 2024, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 21 of said Regulation and Article 13 of said Regulation,

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 agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver.
- (2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.
- (3) It is therefore appropriate to adopt a decision granting a deferral.
- (4) It is therefore appropriate to adopt a decision granting a waiver.

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

A paediatric investigation plan for losmapimod, film-coated tablet, dispersible mini-tablet formulation, oral use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby agreed.

Article 2

A deferral for losmapimod, film-coated tablet, dispersible mini-tablet formulation , oral use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 3

A waiver for losmapimod, film-coated tablet, dispersible mini-tablet formulation, oral use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 4

This decision is addressed to Fulcrum Therapeutics, Inc., 26 Landsdowne Street, MA 02139 - Cambridge CA, United States.

EMA/PDCO/535426/2023
Amsterdam, 23 February 2024

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a deferral and a waiver

EMA-003448-PIP01-23

Scope of the application

Active substance(s):

Losmapimod

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of facioscapulohumeral muscular dystrophy

Pharmaceutical form(s):

Film-coated tablet

Dispersible mini-tablet formulation

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Fulcrum Therapeutics, Inc.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Fulcrum Therapeutics, Inc. submitted for agreement to the European Medicines Agency on 23 May 2023 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 10 July 2023.

Supplementary information was provided by the applicant on 14 November 2023. The applicant proposed modifications to the paediatric investigation plan.

Opinion

1. The Paediatric Committee, having assessed the proposed paediatric investigation plan in accordance with Article 17 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

- to agree the paediatric investigation plan in accordance with Article 17(1) of said Regulation;
- to grant a deferral in accordance with Article 21 of said Regulation;
- to grant a waiver for one or more subsets of the paediatric population in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(c) of said Regulation, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

The Paediatric Committee member of Norway agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the agreed 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 facioscapulohumeral muscular dystrophy

The waiver applies to:

- the paediatric population from birth to less than 2 years of age;
- film-coated tablet, age-appropriate oral formulation, oral use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

2. Paediatric investigation plan

2.1. Condition:

Treatment of facioscapulohumeral muscular dystrophy

2.1.1. Indication(s) targeted by the PIP

Treatment of facioscapulohumeral muscular dystrophy (FSHD)

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)

Film-coated tablet

Solid age-appropriate oral formulation

2.1.4. Measures

Area	Description
Quality-related studies	Study 1 Development of a dispersible mini-tablet formulation for children from 2 years to less than 12 years of age.
Non-clinical studies	Study 2 Juvenile rat repeat-dose toxicity study
Clinical studies	Study 3 Open-label, sequential-group, multicentre study to assess PK, safety, and activity of losmapimod in subjects with facioscapulohumeral muscular dystrophy (FSHD) from 2

	years to less than 18 years of age, followed by an open-label extension phase.
Modelling and simulation analyses	Study 4 Population pharmacokinetic modelling and simulation to extrapolate losmapimod pharmacokinetics from adults to children with FSHD from age 2 years to less than 18 years Study 5 Population pharmacokinetic-pharmacodynamic-efficacy modelling and simulation to estimate the efficacy of losmapimod in children from 2 years to less than 18 years of age with FSHD
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:	No
Date of completion of the paediatric investigation plan:	By September 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.