EMA/557308/2019

European Medicines Agency decision

P/0409/2019

of 6 December 2019

on the acceptance of a modification of an agreed paediatric investigation plan for maralixibat chloride (LUM001) (EMEA-001475-PIP03-17-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.

European Medicines Agency decision

P/0409/2019

of 6 December 2019

on the acceptance of a modification of an agreed paediatric investigation plan for maralixibat chloride (LUM001) (EMEA-001475-PIP03-17-M01) 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 Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency[2],

Having regard to the European Medicines Agency's decision P/0024/2018 issued on 30 January 2018,

Having regard to the application submitted by SFL Regulatory Services GmbH on 15 July 2019 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 18 October 2019, in accordance with Article 22 of Regulation (EC) No 1901/2006 and Article 21 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 acceptance of changes to the agreed paediatric investigation plan and on the granting of a deferral.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

(3) It is therefore appropriate to adopt a decision granting a deferral.

[1] OJ L 378, 27.12.2006, p.1.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for maralixibat chloride (LUM001), oral liquid, oral 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

A deferral for maralixibat chloride (LUM001), oral liquid, oral use, the details of which are set out in the opinion of the Paediatric Committee the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 3

This decision is addressed to SFL Regulatory Services GmbH Europaplatz 2/1/2, 1150 - Vienna, Austria.

EMA/PDCO/412579/2019
Amsterdam, 18 October 2019

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

EMEA-001475-PIP03-17-M01

Scope of the application

Active substance(s):

Maralixibat chloride (LUM001)

Condition(s):

Treatment of progressive familial intrahepatic cholestasis

Pharmaceutical form(s):

Oral liquid

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

SFL Regulatory Services GmbH

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, SFL Regulatory Services GmbH submitted to the European Medicines Agency on 15 July 2019 an application for modification of the agreed paediatric investigation plan as set out in the European Medicines Agency's decision P/0024/2018 issued on 30 January 2018.

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

The procedure started on 20 August 2019.

Scope of the modification

Some measures and timelines of the Paediatric Investigation Plan have been modified and a deferral has been added.

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;
    - to grant a deferral, the details of which are set out in the Annex I of this opinion.

The Norwegian Paediatric Committee member 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 annex 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 progressive familial intrahepatic cholestasis (PFIC)

2.1.1. Indication(s) targeted by the PIP

Treatment of Pruritus in Progressive Familial Intrahepatic Cholestasis

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)

Oral liquid

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable
Non-clinical studies	0	Not applicable
Clinical studies	3	**Study 1 (MRX-502)** Randomised, double-blind, placebo-controlled study in children from 12 months to <18 years with PFIC2. Subjects with PFIC1/3/4, subjects with PFIC phenotype without a known mutation or with another known mutation not described above and PFIC subjects after biliary diversion will be enrolled in a separate cohort and analysed in exploratory fashion. **Study 2 (LUM001-501)** Open label safety and efficacy study in children from 12 months to less than 18 years with PFIC1 or 2. **Study 3 (MRX-801)** *(study added during procedure EMEA-001475-PIP03-17-M01)* Open-label, uncontrolled safety study to evaluate the safety and tolerability of LUM001 in paediatric subjects with cholestatic liver diseases including, but not limited to, Alagille Syndrome and PFIC from birth to less than 12 months of age.

Extrapolation, modelling and simulation studies	0	Not applicable
Other studies	0	Not applicable
Other measures	0	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 December 2023
Deferral for one or more measures contained in the paediatric investigation plan:	Yes