EMA/347454/2023

European Medicines Agency decision

P/0360/2023

of 8 September 2023

on the acceptance of a modification of an agreed paediatric investigation plan for norucholic acid (EMEA-002485-PIP01-18-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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on the acceptance of a modification of an agreed paediatric investigation plan for norucholic acid (EMEA-002485-PIP01-18-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 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/0050/2020 issued on 29 January 2020,

Having regard to the application submitted by Dr. Falk Pharma GmbH on 18 April 2023 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 21 July 2023, 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 norucholic acid, capsule, hard, film-coated tablet, age-appropriate oral solid dosage form, 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 Dr. Falk Pharma GmbH, Leinenweberstr. 5, 79108 – Freiburg, Germany.

EMA/PDCO/214831/2023
Amsterdam, 21 July 2023

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

EMEA-002485-PIP01-18-M01

Scope of the application

Active substance(s):

Norucholic acid

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of primary sclerosing cholangitis

Treatment of autoimmune sclerosing cholangitis

Pharmaceutical form(s):

Capsule, hard

Film-coated tablet

Age-appropriate oral solid dosage form

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Dr. Falk Pharma GmbH

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Dr. Falk Pharma GmbH submitted to the European Medicines Agency on 18 April 2023 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/0050/2020 issued on 29 January 2020.

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

The procedure started on 22 May 2023.

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 primary sclerosing cholangitis

The waiver applies to:

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

1.2. Condition:

Treatment of autoimmune sclerosing cholangitis

The waiver applies to:

- the paediatric population from birth to less than 2 years of age;
- capsule, film-coated tablet, age-appropriate oral solid dosage form, 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 primary sclerosing cholangitis (PSC)

2.1.1. Indication(s) targeted by the PIP

Treatment of primary sclerosing cholangitis without or with features of autoimmune hepatitis (also termed ASC (autoimmune sclerosing cholangitis), or PSC/ASC) in paediatric patients from 2 to 18 years of age.

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

Film-coated tablet

Age-appropriate oral solid dosage form

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** (study deleted in procedure EMEA-002485-PIP01-18-M01) **Study 3** Double-blind, randomised, multi-centre, comparative, safety and efficacy study that will be conducted with 3 parallel treatment groups to compare oral treatment with 2 different doses of norucholic acid with placebo in children and adolescents from 2 years to less than 18 years of age with primary sclerosing cholangitis without or with features of auto-immune hepatitis (AIH).
Extrapolation, modelling and simulation studies	Not applicable.
Other studies	Not applicable.
Other measures	Not applicable.

2.2. Condition:

Treatment of autoimmune sclerosing cholangitis

2.2.1. Indication(s) targeted by the PIP

Treatment of primary sclerosing cholangitis without or with features of autoimmune hepatitis (also termed ASC (autoimmune sclerosing cholangitis), or PSC/ASC) in paediatric patients from 2 to 18 years of age.

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

From 2 years to less than 18 years of age.

2.2.3. Pharmaceutical form(s)

Capsule

Film-coated tablet

Age-appropriate oral solid dosage form

2.2.4. Measures

Area	Description
Quality-related studies	***Study 1*** *The same as for condition "Treatment of primary sclerosing cholangitis".*
Non-clinical studies	Not applicable.
Clinical studies	***Study 2*** (study deleted in procedure EMEA-002485-PIP01-18-M01) *The same as for condition "Treatment of primary sclerosing cholangitis".* ***Study 3*** *The same as for condition "Treatment of primary sclerosing cholangitis".*
Extrapolation, modelling and simulation studies	Not applicable.
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:	No
Date of completion of the paediatric investigation plan:	By December 2028
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.