EMA/673857/2022

European Medicines Agency decision

P/0302/2022

of 10 August 2022

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for efruxifermin (EMEA-003114-PIP01-21) 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 726/2004[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 application submitted by Akero Therapeutics, Inc. on 13 September 2021 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 24 June 2022, 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 efruxifermin, powder for solution for injection, subcutaneous 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 efruxifermin, powder for solution for injection, subcutaneous 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 efruxifermin, powder for solution for injection, subcutaneous 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 Akero Therapeutics, Inc., 601 Gateway Blvd, Suite 350, CA 94080 - South San Francisco, United States.

EMA/PDCO/189181/2022
Amsterdam, 24 June 2022

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

EMEA-003114-PIP01-21

Scope of the application

Active substance(s):

Efruxifermin

Condition(s):

Treatment of non-alcoholic steatohepatitis

Pharmaceutical form(s):

Powder for solution for injection

Route(s) of administration:

Subcutaneous use

Name/corporate name of the PIP applicant:

Akero Therapeutics, Inc.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Akero Therapeutics, Inc. submitted for agreement to the European Medicines Agency on 13 September 2021 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 19 October 2021.

Supplementary information was provided by the applicant on 21 March 2022. 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 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

1.1. Condition:

Treatment of non-alcoholic steatohepatitis

The waiver applies to:

- the paediatric population from birth to less than 8 years of age;
- powder for solution for injection, subcutaneous use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

2. Paediatric investigation plan

2.1. Condition:

Treatment of non-alcoholic steatohepatitis

2.1.1. Indication(s) targeted by the PIP

Treatment of non-alcoholic steatohepatitis (NASH) with fibrosis in children and adolescents from 8 years to less than 18 years of age.

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

From 8 years 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	Study 1 Development of a drug product presentation using lyophilised Efruxifermin in single-use dual chamber syringe (DCS) which can deliver doses that will be selected for paediatric patients.
Non-clinical studies	Not applicable.
Clinical studies	Study 2 Double-blind, randomised, placebo-controlled study conducted in 2 parts - Part A will evaluate safety, pharmacokinetics (PK) and pharmacodynamics (PD) over 6 weeks and Part B will evaluate PK, PD, safety and efficacy over 12 months in children and adolescents from 8 years to less than 18 years with NASH.

Extrapolation, modelling and simulation studies	Study 3 Dose-finding modelling and simulation study to determine the dose for children and adolescents from 8 years to less than 18 years of age with NASH.
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 March 2035
Deferral for one or more measures contained in the paediatric investigation plan:	Yes