MHRA
10 South Colonnade
Canary Wharf
London
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United Kingdom

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Decision Cover Letter

Decision of the licensing authority to:

agree a paediatric investigation plan and grant a deferral and grant a waiver

MHRA-100809-PIP01-22

Scope of the Application

Active Substance(s)

Efruxifermin

Condition(s)

Treatment of non-alcoholic steatohepatitis (NASH)

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 the Decision

Pursuant to the Human Medicines Regulations 2012, Akero Therapeutics, Inc. submitted to the licensing authority on 16/01/2023 11:37 GMT an application for a Paediatric Investigation Plan

The procedure started on 05/06/2023 15:45 BST

1. The licensing authority, having assessed the application in accordance with the Human Medicines Regulations 2012, decides, as set out in the appended summary report:

to agree a paediatric investigation plan and grant a deferral and grant a waiver

2. The measures and timelines of the paediatric investigation plan are set out in the Annex I.

This decision is forwarded to the applicant, together with its annex and appendix.

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Final Decision Letter

MHRA-100809-PIP01-22

Of 09/10/2023 07:41 BST

On the adopted decision for Efruxifermin (MHRA-100809-PIP01-22) in accordance with the Human Medicines Regulations 2012.

The licensing authority, in accordance with the Human Medicines Regulations 2012, has adopted this decision:

Agreement on a paediatric investigation plan

This decision applies to a Paediatric Investigation Plan for Efruxifermin, Powder for solution for injection , SUBCUTANEOUS USE .

This decision is addressed to Akero Therapeutics, Inc., 601 Gateway Boulevard, Suite 350, South San Francisco, UNITED STATES OF AMERICA, CA 94080

ANNEX I

1. Waiver

1.1 Condition:

Treatment of non-alcoholic steatohepatitis (NASH) The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 8 years of age Pharmaceutical form(s): Powder for solution for injection Route(s) of administration: SUBCUTANEOUS USE Reason for granting waiver: 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(s):

Treatment of non-alcoholic steatohepatitis (NASH)

2.2 Indication(s) targeted by the PIP:

Treatment of non-alcoholic steatohepatitis (NASH) with fibrosis

2.3 Subset(s) of the paediatric population concerned by the paediatric development:

The paediatric population from 8 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

Powder for solution for injection

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	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	0	Not applicable.
Clinical Studies	1	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, Modeling & Simulation Studies	1	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	0	Not applicable.
Other Measures	0	Not applicable.

3. Follow-up, completion and deferral of a PIP:

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	31/03/2035

Deferral of one or more studies contained in the paediatric investigation plan:	Yes