

MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
United Kingdom

gov.uk/mhra

Decision Cover Letter

Decision of the licensing authority to:

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

MHRA-102117-PIP01-25

Scope of the Application

Active Substance(s)

Efimosfermin alfa

Condition(s)

Treatment of metabolic dysfunction-associated steatohepatitis

Pharmaceutical Form(s)

Powder for solution for injection

Route(s) of Administration

Subcutaneous use

Name / Corporate name of the PIP applicant

GlaxoSmithKline UK Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, GlaxoSmithKline UK Limited submitted to the licensing authority on 17/09/2025 17:15 BST an application for a Paediatric Investigation Plan

The procedure started on 04/11/2025 10:40 GMT

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-102117-PIP01-25

Of 14/08/2026 15:34 BST

On the adopted decision for Efimosfermin alfa (MHRA-102117-PIP01-25) 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 Efimosfermin alfa, Powder for solution for injection , Subcutaneous use .

This decision is addressed to GlaxoSmithKline UK Limited, 79 New Oxford Street, London, UNITED KINGDOM, WC1A 1DG

ANNEX I

1. Waiver

1.1 Condition:

Treatment of metabolic dysfunction-associated steatohepatitis 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 metabolic dysfunction-associated steatohepatitis

2.2 Indication(s) targeted by the PIP:

Treatment of non-cirrhotic metabolic dysfunction-associated steatohepatitis (MASH) with moderate to severe fibrosis (consistent with F2-F3 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	0	Not applicable.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	1	Study 1 Double-blind, randomised, placebo-controlled study to evaluate the efficacy, safety and pharmacokinetics of efimosfermin alfa in children and adolescents from 8 years to less than 18 years of age with histologically confirmed MASH stage F2-F3 fibrosis.
Extrapolation, Modeling & Simulation Studies	1	Study 2 Population PK model to determine appropriate dosing regimens for the paediatric age groups to be evaluated in PIP Study 1.
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:	No
Date of completion of the paediatric investigation plan:	30/06/2037
Deferral of one or more studies contained in the paediatric investigation plan:	Yes

