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-101435-PIP01-24

Scope of the Application

Active Substance(s)

Hemopexin, human

Condition(s)

Treatment of sickle cell disease

Pharmaceutical Form(s)

Solution for infusion

Route(s) of Administration

INTRAVENOUS USE

Name / Corporate name of the PIP applicant

CSL BEHRING GMBH

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, CSL BEHRING GMBH submitted to the licensing authority on 22/04/2024 10:10 BST an application for a Paediatric Investigation Plan

The procedure started on 13/01/2025 11:35 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-101435-PIP01-24

Of 19/02/2025 16:04 GMT

On the adopted decision for Hemopexin, human (MHRA-101435-PIP01-24) 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 Hemopexin, human , Solution for infusion , INTRAVENOUS USE .

This decision is addressed to CSL BEHRING GMBH, Emil-von-Behring Str. 76, 35041 Marburg, Marburg, GERMANY, 35041

ANNEX I

1. Waiver

1.1 Condition:

Treatment of sickle cell disease The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 6 months of age Pharmaceutical form(s): Solution for infusion Route(s) of administration: INTRAVENOUS USE Reason for granting waiver: on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of sickle cell disease

2.2 Indication(s) targeted by the PIP:

Treatment of acute vaso-occlusive crisis (VOC) in children between 6 months and less than 18 years of age with sickle cell disease (SCD)

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

The paediatric population from 6 months to less than 18 years of age

2.4 Pharmaceutical Form(s):

Solution for infusion

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	3	Study 1 (CSL889_2001) Two part (A and B) double-blind, randomised, placebo- controlled, pharmacokinetic (PK), safety and efficacy study of multiple dose of hemopexin on top of standard of care in paediatric patients from 12 years to less than 18 years of age (and adults) with sickle cell disease (SCD) of any genotype in the treatment of vaso-occlusive crisis (VOC). Study 2 Single-arm, multiple dose, open-label study to evaluate the pharmacokinetics and safety of intravenous hemopexin for the treatment of acute vaso-occlusive crisis (VOCs) in paediatric subjects from 6 months to less than 12 years of age with sickle cell disease. Study 3 Open-label extension study to evaluate the safety, and activity of repeated courses of intravenous hemopexin across separate occurrences of acute vaso-occlusive crisis in (adults and) paediatric patients from 12 years to less than 18 years of age with sickle cell disease.
Extrapolation, Modeling & Simulation Studies	2	Study 4 Population PK modelling to support dosing in Part B of PIP Study 1 and to model exposure in

		PIP Study 2 to support extrapolation of efficacy to paediatric SCD patients in VOC below 12 years of age. Extrapolation plan Study 1, 2 and 4 are part of the extrapolation plan of efficacy data from adult and adolescent patients to the paediatric population from 6 months to less than 12 years of age.
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:	31/10/2030
Deferral of one or more studies contained in the paediatric investigation plan:	Yes