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

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Decision Cover Letter

Decision of the licensing authority to:

accept change(s) to the agreed paediatric investigation plan and to the deferral

MHRA-101494-PIP01-24-M01

Scope of the Application

Active Substance(s)

LUSPATERCEPT

Condition(s)

Treatment of myelodysplastic syndromes. Treatment of beta-thalassaemia.

Pharmaceutical Form(s)

Powder for solution for injection.

Route(s) of Administration

SUBCUTANEOUS USE

Name / Corporate name of the PIP applicant

Bristol-Myers Squibb Pharma EEIG

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Bristol-Myers Squibb Pharma EEIG submitted to the licensing authority on 28/06/2024 23:51 BST an application for a Modification

The procedure started on 22/08/2024 16:30 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 accept change(s) to the agreed paediatric investigation plan and to the deferral.

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-101494-PIP01-24-M01

Of 28/08/2024 10:34 BST

On the adopted decision for LUSPATERCEPT (MHRA-101494-PIP01-24-M01) 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 modification of a paediatric investigation plan (including modification of a waiver or deferral included in that paediatric investigation plan).

This decision applies to a Modification for LUSPATERCEPT, Powder for solution for injection. , SUBCUTANEOUS USE. .

This decision is addressed to Bristol-Myers Squibb Pharma EEIG, Plaza 254, Blanchardstown Corporate Park 2, Dublin 15, IRELAND, D15 T867

ANNEX I

1. Waiver

1.1 Condition:

Treatment of myelodysplastic syndromes. The request for the waiver applies to: Paediatric Subset(s): The paediatric population from birth to less than 18 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. 1.2 Condition: Treatment of beta-thalassaemia. The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 6 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 beta-thalassaemia.

2.2 Indication(s) targeted by the PIP:

Treatment of anaemia in patients with beta-thalassemia intermedia and major.

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

The paediatric population from 6 years of age 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	Study 1, deleted in procedure MHRA-101494-PIP01-24-M01.
Non-Clinical Studies	2	Study 2 (1383657025333) Dose range-finding juvenile toxicity study. Study 3 (1381154982795) Definitive juvenile toxicity study.
Clinical Studies	2	Study 4 Study to evaluate safety and pharmacokinetics of luspatercept in paediatric patients from 6 years of age to less than 18 years of age with transfusion-dependent (TD) and non-transfusion dependent (NTD) beta-thalassaemia. Study 5, deleted in procedure MHRA-101494-PIP01-24-M01. Study 6 Double-blind, randomised, placebo controlled trial to evaluate safety and efficacy of luspatercept in paediatric patients from 6 years of age to less than 12 years of age with TD and NTD beta-thalassaemia.
Extrapolation, Modeling & Simulation Studies	2	Study 7 Modelling and simulation study to describe luspatercept serum exposure data and factors associated with the exposure variability in children from 12 years of age to less than 18 years of age (and adults)

		with beta thalassaemia. (Added in procedure EMEA-001521-PIP01-13-M05.) Study 8 Study of existing and emerging exposure-response data of luspatercept to support efficacy extrapolation from adults to children from 12 years of age to less than 18 years of age with beta thalassaemia. (Added in procedure EMEA-001521-PIP01-13-M05.)
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:	28/02/2034
Deferral of one or more studies contained in the paediatric investigation plan:	Yes