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

gov.uk/mhra

Decision Cover Letter

Decision of the licensing authority to:

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

MHRA-101141-PIP01-23-M01

Scope of the Application

Active Substance(s)

mavacamten

Condition(s)

Treatment of hypertrophic cardiomyopathy

Pharmaceutical Form(s)

Capsule, hard

Route(s) of Administration

ORAL 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 14/08/2023 18:18 BST an application for a Modification

The procedure started on 12/12/2023 12:14 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 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-101141-PIP01-23-M01

Of 09/01/2024 09:36 GMT

On the adopted decision for mavacamten (MHRA-101141-PIP01-23-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 mavacamten, Capsule, hard , ORAL 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 hypertrophic cardiomyopathy The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 12 years of age. Pharmaceutical form(s): Capsule, HARD Route(s) of administration: ORAL USE Reason for granting waiver: On the grounds the specific medicinal product is likely to be unsafe.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of hypertrophic cardiomyopathy.

2.2 Indication(s) targeted by the PIP:

Treatment of obstructive hypertrophic cardiomyopathy.

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

The paediatric population from 12 years to less than 18 years of age.

2.4 Pharmaceutical Form(s):

Capsule, hard

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	2	Study 1 Development of an oral lower strength dose. Study 2 Assessment of compatibility with food and drinks.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	1	Study 3 Double-blind, randomised, placebo-controlled trial to evaluate the activity, safety and tolerability of mavacamten in children from 12 years to less than 18 years of age with obstructive hypertrophic cardiomyopathy.
Extrapolation, Modeling & Simulation Studies	2	Study 4 Modelling and simulation study to derive dosing of mavacamten for use in adolescents from 12 years to less than 18 years of age with obstructive hypertrophic cardiomyopathy. Study 5 Modelling and simulation study to document the PK and PD data relationship in adolescents from 12 years to less than 18 years of age with obstructive hypertrophic cardiomyopathy.
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:	31/03/2029
Deferral of one or more studies contained in the paediatric investigation plan:	Yes