

EMA/283075/2023

European Medicines Agency decision

P/0278/2023

of 14 July 2023

on the acceptance of a modification of an agreed paediatric investigation plan for mavacamten (EMA-002231-PIP01-17-M01) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

Disclaimer

This decision does not constitute entitlement to the rewards and incentives referred to in Title V of Regulation (EC) No 1901/2006.

Only the English text is authentic.

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on the acceptance of a modification of an agreed paediatric investigation plan for mavacamten (EMA-002231-PIP01-17-M01) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

The European Medicines Agency,

Having regard to the Treaty on the Functioning of the European Union,

Having regard to Regulation (EC) No 1901/2006 of the European Parliament and of the Council of 12 December 2006 on medicinal products for paediatric use and amending Regulation (EEC) No. 1768/92, Directive 2001/20/EC, Directive 2001/83/EC and Regulation (EC) No 726/2004¹,

Having regard to Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency²,

Having regard to the European Medicines Agency's decision P/0106/2019 issued on 22 March 2019,

Having regard to the application submitted by Bristol-Myers Squibb Pharma EEIG on 15 February 2023 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 26 May 2023, in accordance with Article 22 of Regulation (EC) No 1901/2006,

Having regard to Article 25 of Regulation (EC) No 1901/2006,

Whereas:

- (1) The Paediatric Committee of the European Medicines Agency has given an opinion on the acceptance of changes to the agreed paediatric investigation plan.
- (2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

¹ OJ L 378, 27.12.2006, p.1, as amended.

² OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for mavacamten, capsule, hard, oral use, are hereby accepted in the scope set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices.

Article 2

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

EMA/PDCO/103283/2023 Corr. ¹
Amsterdam, 26 May 2023

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA-002231-PIP01-17-M01

Scope of the application

Active substance(s):

Mavacamten

Invented name and authorisation status:

See Annex II

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 opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Bristol-Myers Squibb Pharma EEIG submitted to the European Medicines Agency on 15 February 2023 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/0106/2019 issued on 22 March 2019.

The application for modification proposed changes to the agreed paediatric investigation.

The procedure started on 27 March 2023.

¹ 27 June 2023

Scope of the modification

Some measures of the Paediatric Investigation Plan have been modified.

Opinion

1. The Paediatric Committee, having assessed the application in accordance with Article 22 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:
 - to agree to changes to the paediatric investigation plan in the scope set out in the Annex I of this opinion.

The Paediatric Committee member of Norway agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the paediatric investigation plan and the subset(s) of the paediatric population and condition(s) covered by the waiver are set out in the Annex I.

This opinion is forwarded to the applicant and the Executive Director of the European Medicines Agency, together with its annexes and appendix.

Annex I

The subset(s) of the paediatric population and condition(s) covered by the waiver and the measures and timelines of the agreed paediatric investigation plan (PIP)

1. Waiver

1.1. Condition:

Treatment of hypertrophic cardiomyopathy

The waiver applies to:

- the paediatric population from birth to less than 12 years of age;
- capsule, hard, oral use;
- on the grounds that the specific medicinal product is likely to be unsafe.

2. Paediatric investigation plan

2.1. Condition:

Treatment of hypertrophic cardiomyopathy

2.1.1. Indication(s) targeted by the PIP

Treatment of obstructive hypertrophic cardiomyopathy

2.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From 12 years to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Capsule, hard

2.1.4. Measures

Area	Description
Quality-related studies	Study 1 Development of an oral lower strength dose Study 2 Assessment of compatibility with food and drinks
Non-clinical studies	Not applicable
Clinical studies	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, modelling and simulation studies	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	Not applicable
Other measures	Not applicable

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By March 2029
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

The product is not authorised anywhere in the European Community.