EMA/566316/2024

European Medicines Agency decision

P/0400/2024

of 3 January 2025

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for golcadomide (EMEA-003161-PIP02-24) 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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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 726/2004[1],

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 Agency[2],

Having regard to the application submitted by Bristol-Myers Squibb Pharma EEIG on 24 April 2024 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 15 November 2024, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 21 of said Regulation and Article 13 of said Regulation,

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 agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver.

(2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.

(3) It is therefore appropriate to adopt a decision granting a deferral.

(4) It is therefore appropriate to adopt a decision granting a waiver.

[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

A paediatric investigation plan for golcadomide, capsule, hard, powder for oral suspension, oral use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby agreed.

Article 2

A deferral for golcadomide, capsule, hard, powder for oral suspension, oral use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 3

A waiver for golcadomide, capsule, hard, powder for oral suspension, oral use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 4

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

EMA/PDCO/400065/2024
Amsterdam, 15 November 2024

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a deferral and a waiver

EMEA-003161-PIP02-24

Scope of the application

Active substance(s):

Golcadomide

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of mature B-cell neoplasms

Pharmaceutical form(s):

Capsule, hard

Powder for oral suspension

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Bristol-Myers Squibb Pharma EEIG

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Bristol-Myers Squibb Pharma EEIG submitted for agreement to the European Medicines Agency on 24 April 2024 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 27 May 2024.

Supplementary information was provided by the applicant on 12 August 2024.

Opinion

1. The Paediatric Committee, having assessed the proposed paediatric investigation plan in accordance with Article 17 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree the paediatric investigation plan in accordance with Article 17(1) of said Regulation;
   - to grant a deferral in accordance with Article 21 of said Regulation;
   - to grant a waiver for one or more subsets of the paediatric population in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(c) of said Regulation, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

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

2. The measures and timelines of the agreed 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 mature B-cell neoplasms

The waiver applies to:

- the paediatric population from birth to less than 6 months of age;
- capsule, hard; powder for oral suspension; oral use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

2. Paediatric investigation plan

2.1. Condition:

Treatment of mature B-cell neoplasms

2.1.1. Indication(s) targeted by the PIP

Treatment of relapsed or refractory B-cell non-Hodgkin lymphoma

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

From 6 months to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Capsule, hard

Powder for oral suspension

2.1.4. Measures

Area	Description
Quality-related studies	Study 1 Development of an age-appropriate oral dosage form of golcadomide appropriate for the target population.
Non-clinical studies	Study 2 Study to determine the antitumor activity of golcadomide in human Burkitt lymphoma xenograft models.
Clinical studies	Study 3 Adaptive, prospective, multicentre, open label, platform clinical trial to evaluate the safety and efficacy of novel agents, including golcadomide in combination with rituximab followed by golcadomide monotherapy (Arm IV of

	the Glo-BNHL platform study) for the treatment of children and adolescents from 6 months to less than 18 years of age (and young adults) with relapsed or refractory B-cell non-Hodgkin lymphoma.
Modelling and simulation analyses	Study 4 Use of a PopPK(/PD) model to confirm or modify the paediatric doses of *g*olcadomide to be used in the expansion stage of Study 3. Study 5 Use of a physiologically-based (PB-PK) model to analyse (sparse) PK data collected in paediatric studies and pooled with adult PK data to inform dosing recommendation for *g*olcadomide for treatment of relapsed or refractory B-cell non-Hodgkin lymphoma in paediatric patients. Study 6 Use of a PopPK(/PD) model to confirm the doses in paediatric patients.
Other studies	Not applicable
Extrapolation plan	Not applicable

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	By January 2032
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

The product is not authorised anywhere in the European Community.