EMA/483235/2024

European Medicines Agency decision

P/0371/2024

of 25 October 2024

on the agreement of a paediatric investigation plan for cyclic GMP-AMP synthase inhibitor (BI 3000202), (EMEA-003440-PIP01-23) 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 agreement of a paediatric investigation plan for cyclic GMP-AMP synthase inhibitor (BI 3000202), (EMEA-003440-PIP01-23) 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 726/2004[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 Boehringer Ingelheim International GmbH on 27 July 2023 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 6 September 2024, in accordance with Article 17 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 agreement of a paediatric investigation plan.

(2) It is therefore appropriate to adopt a decision agreeing a 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

A paediatric investigation plan for cyclic GMP-AMP synthase inhibitor (BI 3000202), film-coated tablet, 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

This decision is addressed to Boehringer Ingelheim International GmbH, Binger Strasse 173, 55216 - Ingelheim am Rhein, Germany.

EMA/PDCO/285620/2024 Corr[1]
Amsterdam, 6 September 2024

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan

EMEA-003440-PIP01-23

Scope of the application

Active substance(s):

Cyclic GMP-AMP synthase inhibitor (BI 3000202)

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of type 1 interferonopathies

Pharmaceutical form(s):

Film-coated tablet

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Boehringer Ingelheim International GmbH

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Boehringer Ingelheim International GmbH submitted for agreement to the European Medicines Agency on 27 July 2023 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation.

The procedure started on 11 September 2023.

Supplementary information was provided by the applicant on 29 May 2024. The applicant proposed modifications to the paediatric investigation plan and withdrew its request for a deferral.

[1] 18 October 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.

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 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

Not applicable.

2. Paediatric investigation plan

2.1. Condition:

Treatment of type 1 interferonopathies

2.1.1. Indication(s) targeted by the PIP

Treatment of type 1 interferonopathies

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

From birth to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Film-coated tablet

2.1.4. Measures

Area	Description
Quality-related studies	**Study 1** Development of an age-appropriate film-coated tablet formulation (2 mm tablets) for BI 3000202 to be used from birth to less than 18 years old.
Non-clinical studies	**Study 2** Enhanced pre- and postnatal development study to evaluate safety of prenatal and early postnatal exposure to BI 3000202 to support the drug development in children from birth to 2 years of age. **Study 3** Definitive juvenile toxicity study to evaluate safety of the use of BI 3000202 in children from birth.
Clinical studies	**Study 4 (1509-0018)** Single-arm, open-label trial with intra-patient-dose escalation to evaluate pharmacokinetics (PK), pharmacodynamics (PD) and safety of BI3000202 in patients from 3 years to less than 18 years of age (and adults) diagnosed with Aicardi-Goutières syndrome (AGS), familial chilblain lupus (FCL) or coatomer subunit alpha (COPA) syndrome. **Study 5 (1509-0023)**

	Randomised, placebo-controlled study to evaluate safety and efficacy of BI3000202 in children and adolescents from birth to less than 18 years of age (and adults) with selected type 1 interferonopathies (Aicardi-Goutières syndrome (AGS), familial chilblain lupus (FCL) and coatomer subunit alpha (COPA) syndrome).
Modelling and simulation analyses	**Study 6** Population PK study to evaluate the use of BI 3000202 in the in children from birth to less than 18 years of age with selected type 1 interferonopathies. **Study 7** Population PK and exposure-response models to evaluate the use of BI 3000202 in the in children from birth to less than 18 years of age with selected type 1 interferonopathies.
Other studies	**Study 8 (1509-0022)** Chart review study to describe patients' socio-demographic, clinical characteristics and diagnostic history at the time of diagnosis of selected type 1 interferonopathies. **Study 9 (1509-0019)** Prospective study to characterise the natural history and disease-relevant biomarkers of selected type 1 interferonopathies treated with the current standard of care.
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:	Yes
Date of completion of the paediatric investigation plan:	By March 2031
Deferral for one or more measures contained in the paediatric investigation plan:	No

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

The product is not authorised anywhere in the European Community.