EMA/307256/2024

European Medicines Agency decision

P/0277/2024

of 14 August 2024

on the acceptance of a modification of an agreed paediatric investigation plan for vonoprazan (EMEA-002703-PIP01-19-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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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 European Medicines Agency's decision P/0454/2020 issued on 4 December 2020,

Having regard to the application submitted by Phathom Pharmaceuticals, Inc. on 21 March 2024 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and proposing a waiver for a new paediatric subset,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 28 June 2024, in accordance with Article 22 of Regulation (EC) No 1901/2006 and Article 11(1)(c) 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 acceptance of changes to the agreed paediatric investigation plan and to the deferral and on the granting of a waiver.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan, including changes to the deferral.

(3) 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

Changes to the agreed paediatric investigation plan for vonoprazan, tablet, age-appropriate dosage form, oral use, gastric use, including changes to the deferral, 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

A waiver for vonoprazan, tablet, age-appropriate dosage form, oral use, gastric use, the details of which are set out in the opinion of the Paediatric Committee the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 3

This decision is addressed to Phathom Pharmaceuticals, Inc., 2150 E. Lake Cook Road, Suite 800, 60089 - Buffalo Grove, Illinois, USA.

EMA/PDCO/140969/2024
Amsterdam, 28 June 2024

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan

EMEA-002703-PIP01-19-M01

Scope of the application

Active substance(s):

Vonoprazan

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of gastroesophageal reflux disease

Treatment of *Helicobacter pylori* infection

Pharmaceutical form(s):

Tablet

Age-appropriate dosage form

Route(s) of administration:

Oral use

Gastric use

Name/corporate name of the PIP applicant:

Phathom Pharmaceuticals, Inc.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Phathom Pharmaceuticals, Inc. submitted to the European Medicines Agency on 21 March 2024 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/0454/2020 issued on 4 December 2020.

The application for modification proposed changes to the agreed paediatric investigation plan and to the deferral and proposed a waiver for a new paediatric subset.

The procedure started on 29 April 2024.

Scope of the modification

Some measures and timelines of the Paediatric Investigation Plan have been modified. A new waiver in a subset of the paediatric population for one of the conditions has been added.

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 and to the deferral in the scope set out in the Annex I of this opinion;
   - to grant a waiver for one or more subsets of the paediatric population concluded in accordance with Article 11(1)(c) of Regulation (EC) No 1901/2006 as amended, 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 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 *Helicobacter pylori* infection

The waiver applies to:

- all subsets of the paediatric population from birth to less than 18 years of age;
- tablet, age-appropriate dosage form, oral use, gastric use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

1.2. Condition:

Treatment of gastroesophageal reflux disease

The waiver applies to:

- all subsets of the paediatric population from birth to less than 1 month of age;
- tablet, age-appropriate dosage form, oral use, gastric use;
- 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:

Treatment of gastroesophageal reflux disease (GORD)

2.1.1. Indication(s) targeted by the PIP

Treatment of erosive reflux oesophagitis

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

From 1 month to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Tablet

Age-appropriate dosage form

2.1.4. Measures

Area	Description
Quality-related studies	**Study 1** Development of an age-appropriate oral solid dosage form
Non-clinical studies	**Study 2** Definitive juvenile toxicity study in rats
Clinical studies	**Study 3 (**EudraCT Number: 2023-000179-10 (Study VPED-102)) Open label, uncontrolled, parallel dose study to assess pharmacokinetics of vonoprazan in adolescents from 12 years to less than 18 years of age with gastroesophageal reflux disease **Study 4 (**EudraCT Number: 2022-003228-42 (Study VPED-103) and 2022-003229-23 (Study VPED-104)) Open label, uncontrolled, parallel dose study to assess pharmacokinetics of vonoprazan in children from 1 month to less than 12 years of age with gastroesophageal reflux disease **Study 5 (**VPED-201) Open label healing phase study followed by a double-blind placebo controlled maintenance phase safety and efficacy study in children and adolescents from 1 month to less than 18 years of age with gastroesophageal reflux disease
Extrapolation, modelling and simulation studies	**Study 6** Dose finding, modelling and simulation study **Study 7** Analysis of in-house clinical results from adults and children
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:	No
Date of completion of the paediatric investigation plan:	By August 2031
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.