EMA/581093/2020

European Medicines Agency decision

P/0454/2020

of 4 December 2020

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for vonoprazan (EMEA-002703-PIP01-19) 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 Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency[2],

Having regard to the application submitted by Phathom Pharmaceuticals, Inc. on 27 November 2019 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 16 October 2020, 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.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

A paediatric investigation plan for vonoprazan, tablet, dispersible tablet, age-appropriate dosage form, oral use, gastric 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 vonoprazan, tablet, dispersible tablet, age-appropriate dosage form, oral use, gastric 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 vonoprazan, tablet, dispersible tablet, age-appropriate dosage form, oral use, gastric 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 Phathom Pharmaceuticals, Inc., 2150 E. Lake Cook Road, Suite 800, 60089 - Buffalo Grove, Illinois, United States.

EMA/PDCO/417635/2020 Corr
Amsterdam, 16 October 2020

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

EMEA-002703-PIP01-19

Scope of the application

Active substance(s):

Vonoprazan

Condition(s):

Treatment of gastroesophageal reflux disease

Treatment of *Helicobacter pylori* infection

Pharmaceutical form(s):

Tablet

Dispersible 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 16(1) of Regulation (EC) No 1901/2006 as amended, Phathom Pharmaceuticals, Inc. submitted for agreement to the European Medicines Agency on 27 November 2019 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 6 January 2020.

Supplementary information was provided by the applicant on 13 July 2020. The applicant proposed modifications to the paediatric investigation plan.

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 Norwegian Paediatric Committee member 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 annex 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, dispersible 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 birth to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Tablet

Dispersible tablet

Age-appropriate dosage form

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	1	**Study 1** Development of an age-appropriate oral solid dosage form (sprinkles)
Non-clinical studies	1	**Study 2** Definitive juvenile toxicity study in rats

Area	Number of measures	Description
Clinical studies	3	**Study 3** 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** Open label, uncontrolled, parallel dose study to assess pharmacokinetics of vonoprazan in children from birth to less than 12 years of age with gastroesophageal reflux disease **Study 5** Open label healing phase study followed by a double-blind placebo controlled maintenance phase safety and efficacy study in children and adolescents from birth to less than 18 years of age with gastroesophageal reflux disease
Extrapolation, modelling and simulation studies	2	**Study 6** Dose finding, modelling and simulation study **Study 7** Analysis of in-house clinical results from adults and children
Other studies	0	Not applicable.
Other measures	0	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 July 2030
Deferral for one or more measures contained in the paediatric investigation plan:	Yes