EMA/359364/2021

European Medicines Agency decision

P/0272/2021

of 8 July 2021

on the acceptance of a modification of an agreed paediatric investigation plan for rilzabrutinib (EMEA-002438-PIP02-19-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 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 European Medicines Agency's decision P/0306/2020 issued on 12 August 2020,

Having regard to the application submitted by Principia Biopharma, Inc. on 15 February 2021 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 21 May 2021, 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.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for rilzabrutinib, film-coated tablet, age-appropriate oral solid dosage form, oral use, gastric 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 Principia Biopharma, Inc., 220 E Grand Avenue, 94080 - South San Francisco, USA.

EMA/PDCO/145917/2021
Amsterdam, 21 May 2021

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

EMEA-002438-PIP02-19-M01

Scope of the application

Active substance(s):

Rilzabrutinib

Condition(s):

Treatment of immune thrombocytopenia

Pharmaceutical form(s):

Film-coated tablet

Age-appropriate oral solid dosage form

Route(s) of administration:

Oral use

Gastric use

Name/corporate name of the PIP applicant:

Principia Biopharma, Inc.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Principia Biopharma, Inc. submitted to the European Medicines Agency on 15 February 2021 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/0306/2020 issued on 12 August 2020.

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

The procedure started on 23 March 2021.

Scope of the modification

Some measures and timelines 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 Norwegian Paediatric Committee member 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 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 immune thrombocytopenia

The waiver applies to:

- the paediatric population from birth to less than 1 year of age;
- film-coated tablet, oral use, age-appropriate oral solid dosage form, gastric use;
- on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

2. Paediatric investigation plan

2.1. Condition:

Treatment of immune thrombocytopenia

2.1.1. Indication(s) targeted by the PIP

Treatment of persistent or chronic immune thrombocytopenia in patients from 1 year to less than 18 years of age who have failed at least one line of therapy and/or had insufficient response to therapy.

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

From 1 year to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Film-coated tablet

Age-appropriate oral solid 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 (mini-tablets or multi-particulates or powder/granules) for use in the paediatric population from 1 year to less than 12 years of age
Non-clinical studies	2	**Study 2** Dose range-finding juvenile toxicity study to select the dose levels for the definitive juvenile toxicity study and to assess the toxicokinetic profile of rilzabrutinib

		Study 3 Definitive juvenile toxicity study to evaluate the potential systemic and immunologic effects and the toxicokinetic profile of rilzabrutinib
Clinical studies	2	**Study 4** Randomised, double-blind placebo-controlled study to evaluate the efficacy, safety and pharmacokinetics of rilzabrutinib in paediatric patients from 12 years to less than 18 years of age (and in adults) with immune thrombocytopenia (PRN1008-018) **Study 5** Randomised, double-blind placebo-controlled study to evaluate the efficacy, safety and pharmacokinetics of rilzabrutinib in paediatric patients from 1 year to less than 12 years of age with immune thrombocytopenia
Extrapolation, modelling and simulation studies	0	Not applicable
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:	Yes
Date of completion of the paediatric investigation plan:	By June 2028
Deferral for one or more measures contained in the paediatric investigation plan:	Yes