EMA/553384/2023

European Medicines Agency decision

P/0524/2023

of 29 December 2023

on the acceptance of a modification of an agreed paediatric investigation plan for rilzabrutinib (EMEA-002438-PIP02-19-M03) 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/0306/2020 issued on 12 August 2020, decision P/0272/2021 issued on 8 July 2021 and the decision P/0262/2022 issued on 18 July 2022,

Having regard to the application submitted by Sanofi B.V. on 4 August 2023 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 10 November 2023, in accordance with Article 22 of Regulation (EC) No 1901/2006 and Article 21 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 on the granting of a deferral.

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

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

[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 rilzabrutinib, film-coated tablet, oral 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

A deferral for rilzabrutinib, film-coated tablet, oral 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 Sanofi B.V., Paasheuvelweg 25, 1105 BP - Amsterdam, The Netherlands.

EMA/PDCO/365465/2023
Amsterdam, 10 November 2023

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

EMEA-002438-PIP02-19-M03

Scope of the application

Active substance(s):

Rilzabrutinib

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of immune thrombocytopenia

Pharmaceutical form(s):

Film-coated tablet

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Sanofi B.V..

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Sanofi B.V. submitted to the European Medicines Agency on 4 August 2023 an application for modification of the agreed paediatric investigation plan with a waiver as set out in the European Medicines Agency's decision P/0306/2020 issued on 12 August 2020, decision P/0272/2021 issued on 8 July 2021 and the decision P/0262/2022 issued on 18 July 2022.

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

The procedure started on 11 September 2023.

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;
   - to grant a deferral, the details of which are set out in the Annex I of this opinion.

   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 immune thrombocytopenia

The waiver applies to:

- the paediatric population from birth to less than 1 year of age;
- film-coated tablet, oral 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).

The waiver applies to:

- the paediatric population from 1 year to less than 10 years of age;
- film-coated tablet, oral use;
- on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of 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 10 years to less than 18 years of age after insufficient response to a previous treatment.

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

From 10 years 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*** *Deleted in procedure EMEA-002438-PIP02-19-M02*
Non-clinical studies	***Study 2*** *Deleted in procedure EMEA-002438-PIP02-19-M02*

	Study 3 *Deleted in procedure EMEA-002438-PIP02-19-M02*
Clinical studies	**Study 4 (PRN1008-018)** Randomised, double-blind placebo-controlled study to evaluate the efficacy, safety and pharmacokinetics of rilzabrutinib in paediatric patients from 10 years to less than 18 years of age (and in adults) with immune thrombocytopenia ***Study 5*** *Deleted in procedure EMEA-002438-PIP02-19-M02*
Modelling and simulation studies	Not applicable
Other studies	Not applicable
Extrapolation plan	Study 4 is part of an extrapolation plan covering the paediatric population from 10 years to less than 18 years of age, as agreed by the PDCO. *Added in procedure EMEA-002438-PIP02-19-M03.*

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 December 2025
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.