MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
United Kingdom

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Decision Cover Letter

Decision of the licensing authority to:

accept change(s) to the agreed paediatric investigation plan (MHRA-101000-PIP01-23-M01) and to the deferral;

MHRA-101000-PIP01-23-M02

Scope of the Application

Active Substance(s)

Rilzabrutinib

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 the Decision

Pursuant to the Human Medicines Regulations 2012, Sanofi B.V. submitted to the licensing authority on 30/01/2024 11:53 GMT an application for a Modification

The procedure started on 10/05/2024 14:54 BST

1. The licensing authority, having assessed the application in accordance with the Human Medicines Regulations 2012, decides, as set out in the appended summary report:

to accept change(s) to the agreed paediatric investigation plan and to the deferral.

2. The measures and timelines of the paediatric investigation plan are set out in the Annex I.

This decision is forwarded to the applicant, together with its annex and appendix.

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Final Decision Letter

MHRA-101000-PIP01-23-M02

Of 30/05/2024 14:14 BST

On the adopted decision for Rilzabrutinib (MHRA-101000-PIP01-23-M02) in accordance with the Human Medicines Regulations 2012.

The licensing authority, in accordance with the Human Medicines Regulations 2012, has adopted this decision:

Agreement on modification of a paediatric investigation plan (including modification of a waiver or deferral included in that paediatric investigation plan).

This decision applies to a Modification for Rilzabrutinib, Film-coated Tablet , ORAL USE .

This decision is addressed to Sanofi B.V., Paasheuvelweg 25, Amsterdam, NETHERLANDS, 1105 BP

ANNEX I

1. Waiver

1.1 Condition:

Treatment of immune thrombocytopenia. The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 10 years of age. Pharmaceutical form(s): Film-coated Tablet Route(s) of administration: ORAL USE Reason for granting waiver: For the paediatric population from birth to less than 1 year of age: 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). For the paediatric population from 1 year to less than 10 years of age. 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(s):

Treatment of immune thrombocytopenia.

2.2 Indication(s) targeted by the PIP:

Treatment of persistent or chronic immune thrombocytopenia (ITP) in patients from 10 years to less than 18 years of age, after insufficient response to a previous treatment.

2.3 Subset(s) of the paediatric population concerned by the paediatric development:

The paediatric population from 10 years to less than 18 years of age.

2.4 Pharmaceutical Form(s):

Film-coated Tablet.

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Study 1 Deleted in MHRA-101000-PIP01-23-M01.
Non-Clinical Studies	0	Study 2 Deleted in MHRA-101000-PIP01-23-M01. Study 3 Deleted in MHRA-101000-PIP01-23-M01.
Clinical Studies	1	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 MHRA-101000-PIP01-23-M01.
Extrapolation, Modeling & Simulation Studies	0	Not applicable.
Other Studies	0	Not applicable.
Other Measures	0	Extrapolation plan Added in procedure MHRA-101000-PIP01-23-M02. 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 Regulatory Agency.

3. Follow-up, completion and deferral of a PIP:

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	Yes

Date of completion of the paediatric investigation plan:	31/12/2025
Deferral of one or more studies contained in the paediatric investigation plan:	Yes