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

gov.uk/mhra

Decision Cover Letter

Decision of the licensing authority to:

accept change(s) to the agreed paediatric investigation plan (MHRA-100019-PIP01-21) and to the deferral

MHRA-100019-PIP01-21-M01

Scope of the Application

Active Substance(s)

BELUMOSUDIL MESYLATE

Condition(s)

Treatment of chronic graft versus host disease (cGVHD)

Pharmaceutical Form(s)

Film-coated tablet, Oral suspension

Route(s) of Administration

ORAL USE; NASOGASTRIC USE

Name / Corporate name of the PIP applicant

Sanofi Winthrop Industrie

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Sanofi Winthrop Industrie submitted to the licensing authority on 22/04/2024 20:17 BST an application for a Modification

The procedure started on 19/06/2024 07:29 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.

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

gov.uk/mhra

Final Decision Letter

MHRA-100019-PIP01-21-M01

Of 04/11/2024 16:09 GMT

On the adopted decision for BELUMOSUDIL MESYLATE (MHRA-100019-PIP01-21-M01) 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 BELUMOSUDIL MESYLATE, Film-coated tablet, Oral suspension , ORAL USE; NASOGASTRIC USE .

This decision is addressed to Sanofi Winthrop Industrie, 82 avenue Raspail, Gentilly, FRANCE, 94250

ANNEX I

1. Waiver

1.1 Condition:

Treatment of chronic graft versus host disease (cGVHD) The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 1 year of age Pharmaceutical form(s): Film-coated tablet Oral suspension Route(s) of administration: ORAL USE NASOGASTRIC USE Reason for granting waiver: For the paediatric population from birth to less than 3 months of age: - the grounds are 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 3 months to less than 1 year of age: - the grounds are that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of chronic graft versus host disease (cGVHD)

2.2 Indication(s) targeted by the PIP:

Treatment of chronic graft versus host disease (cGVHD)

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

The paediatric population from 1 year to less than 18 years of age

2.4 Pharmaceutical Form(s):

Film-coated tablet Oral suspension

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	2	Study 1 Development of an age-appropriate dosage form. Study 2 Generation of data on dose delivery devices, stability and compatibility with different routes of administration.
Non-Clinical Studies	0	Study 3 Deleted during procedure MHRA-100019-PIP01-21-M01. Study 4 Deleted during procedure MHRA-100019-PIP01-21-M01.
Clinical Studies	2	Study 5 (KD025-213/ DRI17633) Open-label, single arm trial to evaluate pharmacokinetics (PK), safety and activity of belumosudil in adolescent patients aged 12 years of age and above with chronic graft versus host disease (cGVHD) who have been treated with at least 2 prior lines of systemic therapy. Study 6 (DFI17893) Open-label, single arm, two part trial to evaluate the pharmacokinetics (PK) and a recommended paediatric equivalent dose (part 1), safety and activity (part 2) of belumosudil in children from 1 year to less than 18 years of age with chronic graft versus host disease (cGVHD) who have been treated

		with at least 2 prior lines of systemic therapy.
Extrapolation, Modeling & Simulation Studies	2	Study 7 (Allometrically scaled population PK modelling) Modelling and simulation study to evaluate the use of the product in the proposed paediatric indication in children from 1 year to less than 18 years of age with chronic graft versus host disease. Study 8 Deleted during procedure MHRA-100019-PIP01-21-M01 Study 9 Deleted during procedure MHRA-100019-PIP01-21-M01. Extrapolation Plan Studies 5, 6 and 7 are part of the extrapolation plan of efficacy data from adults (studies KD025-208 and KD025-213) to adolescents and to the paediatric population from 1 year to less than 12 years of age with cGVHD.
Other Studies	0	Measure deleted during procedure MHRA-100019-PIP01-21-M01.
Other Measures	0	Not applicable.

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:	31/12/2030
Deferral of one or more studies contained in the paediatric investigation plan:	Yes