EMA/87429/2024

European Medicines Agency decision

P/0065/2024

of 8 March 2024

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for belumosudil, (EMEA-003425-PIP01-23) 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 application submitted by Sanofi Winthrop Industrie on 20 March 2023 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 19 January 2024, 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, as amended.
[2] OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

A paediatric investigation plan for belumosudil, film-coated tablet, oral suspension, oral use, nasogastric 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 belumosudil, film-coated tablet, oral suspension, oral use, nasogastric 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 belumosudil, film-coated tablet, oral suspension, oral use, nasogastric 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 Sanofi Winthrop Industrie, 82 avenue Raspail, 94250 – Gentilly, France.

EMA/PDCO/481390/2023
Amsterdam, 19 January 2024

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

EMEA-003425-PIP01-23

Scope of the application

Active substance(s):

Belumosudil

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of graft versus host disease

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 opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Sanofi Winthrop Industrie submitted for agreement to the European Medicines Agency on 20 March 2023 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulationand a waiver under Article 13 of said Regulation.

The procedure started on 24 April 2023.

Supplementary information was provided by the applicant on 16 October 2023. The applicant proposed modifications to the paediatric investigation plan and waiver.

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 Paediatric Committee member of Norway 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 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 graft versus host disease

The waiver applies to:

- the paediatric population from birth to less than 1 year of age;
- film-coated tablet, oral suspension, oral use, nasogastric use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible.

2. Paediatric investigation plan

2.1. Condition:

Treatment of graft versus host disease

2.1.1. Indication(s) targeted by the PIP

Treatment of chronic graft versus host disease (cGVHD)

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, oral suspension

2.1.4. Measures

Area	Description
Quality-related studies	**Study 1** Development of an age-appropriate formulation for use in paediatric patients unable to swallow tablets.
Non-clinical studies	Not applicable.
Clinical studies	**Study 2 (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 3 (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.
Modelling and simulation studies	**Study 4 (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.
Other studies	**Study 5 (AA_00117)** Observational retrospective study based on medical records that describe the safety and efficacy of belumosudil in adolescent patients above 12 years of age (and adults) with chronic GvHD who received at least two lines of treatment.
Extrapolation plan	Studies 2, 3 and 4 are part of an extrapolation plan covering the paediatric population from 1 year to less than 18 years of age, as agreed by the PDCO.

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 2030
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.