EMA/22503/2024

European Medicines Agency decision

P/0047/2024

of 23 February 2024

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for frexalimab, (EMEA-002945-PIP03-23) 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 SA on 17 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 15 December 2023, 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, following a re-examination procedure of the Paediatric Committee's opinion according to Article 25(3) of Regulation (EC) No 1901/2006, 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 frexalimab, solution for injection/infusion, intravenous use, subcutaneous 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 frexalimab, solution for injection/infusion, intravenous use, subcutaneous 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 frexalimab, solution for injection/infusion, intravenous use, subcutaneous 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/31474/2024
Amsterdam, 14 February 2024

Final opinion of the Paediatric Committee on the agreement of a Paediatric Investigation plan and a deferral and a waiver

EMEA-002945-PIP03-23

Scope of the application

Active substance(s):

Frexalimab

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of type 1 diabetes mellitus

Pharmaceutical form(s):

Solution for injection/infusion

Route(s) of administration:

Intravenous use, subcutaneous use

Name/corporate name of the PIP applicant:

Sanofi Winthrop Industrie SA

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Sanofi Winthrop Industrie SA submitted for agreement to the European Medicines Agency on 17 March 2023 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

An Opinion was adopted by the Paediatric Committee on 15 December 2023, by consensus for the above mentioned product. Sanofi Winthrop Industrie SA received the Paediatric Committee Opinion on 22 December 2023.

On 19 January 2024 Sanofi Winthrop Industrie SA submitted to the European Medicines Agency a written request, including detailed grounds for re-examination of the Opinion.

The re-examination procedure started on 22 January 2024.

Final Opinion

1. The Paediatric Committee, having assessed the detailed grounds for re-examination, in accordance with Article 25(3) of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   1.1. to revise its opinion and

   - 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.

   1.2. following re-examination, to amend the measures of the paediatric investigation plan.

The Paediatric Committee members of Liechtenstein and Norway agree 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 type I diabetes mellitus

The waiver applies to:

- the paediatric population from birth to less than 1 year of age;
- solution for injection/infusion, intravenous use, subcutaneous use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

2. Paediatric investigation plan

2.1. Condition:

Treatment of type I diabetes mellitus

2.1.1. Indication(s) targeted by the PIP

Treatment of type 1 diabetes mellitus (T1D) to preserve β-cell function in newly diagnosed T1D patients 1 year to less than 18 years old with clinically meaningful residual β-cell function

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)

Solution for injection/infusion, intravenous use and subcutaneous use

2.1.4. Measures

Area	Description
Quality-related studies	**Study 1:** Development of an age-appropriate pharmaceutical form for children from 6 years to less than 18 years of age. **Study 2:** Development of an age-appropriate pharmaceutical form for children from 1 year to less than 6 years of age.
Non-clinical studies	**Study 3:** Reprotoxicity study in Cynomolgus offspring.
Clinical studies	**Study 4:** 52-week randomized, double-blind, placebo-controlled, multi-center dose-finding study with a 52-week blinded extension to evaluate the safety and efficacy of frexalimab for the preservation of pancreatic β-cell function in adolescents from 12 years of age (and adults up to 35 years of age) with newly diagnosed type 1 diabetes Stage 3 on insulin therapy (DRI17476).

	Study 5: Randomized, double-blind, placebo-controlled, parallel-group study including a 52-week blinded extension to evaluate the safety and efficacy of frexalimab for the preservation of pancreatic β-cell function in children and adolescents from 6 years of age (and adults up to 21 years of age) with newly diagnosed type 1 diabetes Stage 3 and on insulin therapy (EFC1). **Study 6:** 26-week multinational, multicentre, randomized, placebo-controlled 2-arm parallel group trial with a 26-week extension, to evaluate the efficacy and safety of frexalimab in children from 1 year to less than 6 years of age with newly diagnosed T1D Stage 3 in addition to insulin therapy (EFC2).
Modelling and simulation studies	**Study 7:** Population PK/PD modelling in children from 1 year of age, adolescents and adults with newly diagnosed T1D Stage 3 in addition to insulin therapy.
Other studies	Not applicable.
Extrapolation plan	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 March 2033
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.