EMA/493296/2024

European Medicines Agency decision

P/0386/2024

of 25 October 2024

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for teplizumab, (EMEA-000524-PIP02-24) 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 22 February 2024 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 18 October 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 teplizumab, concentrate for solution for infusion, intravenous 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 teplizumab, concentrate for solution for infusion, intravenous 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 teplizumab, concentrate for solution for infusion, intravenous 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/335020/2024
Amsterdam, 18 October 2024

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

EMEA-000524-PIP02-24

Scope of the application

Active substance(s):

Teplizumab

Invented name and authorisation status:

See Annex II

Condition(s):

Prevention of stage 3 type 1 diabetes mellitus

Pharmaceutical form(s):

Concentrate for solution for infusion

Route(s) of administration:

Intravenous 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 22 February 2024 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation

The procedure started on 2 April 2024.

Supplementary information was provided by the applicant on 8 July 2024. The applicant proposed modifications to the paediatric investigation plan and requested a 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:

Prevention of stage 3 type 1 diabetes mellitus

The waiver applies to:

- the paediatric population from birth to less than 1 year of age;
- concentrate for solution for infusion, intravenous 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:

Prevention of stage 3 type 1 diabetes mellitus

2.1.1. Indication(s) targeted by the PIP

To delay the onset of stage 3 type 1 diabetes in paediatric patients with stage 2 type 1 diabetes.

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)

Concentrate for solution for infusion

2.1.4. Measures

Area	**Description**
Quality-related studies	Not applicable
Non-clinical studies	Not applicable
Clinical studies	Study 1 (TN-10) Double-blind, randomised, placebo-controlled study to evaluate the efficacy of a single 14-day course of teplizumab to delay or prevent clinical type 1 diabetes in children from 8 to less than 18 years (and adults) at-risk for type 1 diabetes. Study 2 (PETITE-T1D) *This study is the same as Study 12 (PRV-031-005 (PETITE-T1D)) of the PIP for teplizumab (EMA/PE/0000221282) for treatment of type I diabetes mellitus and subsequent modifications thereof.*

	Single arm, open-label study to assess the safety and pharmacokinetics of a 14-day regimen of teplizumab in children aged from birth to less than 8 years with Stage 2 type 1 diabetes (T1D). Study 3 Randomized, double-blind, placebo-controlled study to evaluate safety and efficacy of teplizumab to delay or prevent clinical type 1 diabetes in children from 1 year to less than 8 years of age with stage 2 Type I Diabetes mellitus.
Modelling and simulation analyses	Study 4 *This study is the same as Study 13 of the teplizumab EMA/PE/0000221282 for treatment of type I diabetes mellitus and subsequent modifications thereof.* Population Pharmacokinetic/Pharmacodynamic (PK/PD) model.
Other studies	Study 5 Real World Evidence Study to assess the similarity of time to progression from stage 2 to stage 3 T1D between the TN10 trial placebo group (PIP study 1) and a sample of European individuals with T1D.
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 December 2034
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.