EMADOC-1700519818-1850257

European Medicines Agency decision

EMA/PE/0000243095

of 16 January 2025

on the acceptance of a modification of an agreed paediatric investigation plan for teplizumab 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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on the acceptance of a modification of an agreed paediatric investigation plan for teplizumab in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

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 European Medicines Agency's decision P/8/2010 issued on 29 January 2010 and the decision EMA/PE/0000221282 issued on 30 October 2024,

Having regard to the application submitted by Sanofi Winthrop Industrie on 19 December 2024 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 14 January 2025, in accordance with Article 22 of Regulation (EC) No 1901/2006,

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 acceptance of changes to the agreed paediatric investigation plan.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

[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

Changes to the agreed paediatric investigation plan for teplizumab, are hereby accepted in the scope set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices.

Article 2

This decision is addressed to Sanofi Winthrop Industrie, 82 Avenue Raspail, 94250 Gentilly, France.

EMADOC-1700519818-1837392
Amsterdam, 14 January 2025

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan

EMA/PE/0000243095

Scope of the application

Active substance(s):

Teplizumab

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of type I 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 22 of Regulation (EC) No 1901/2006 as amended, Sanofi Winthrop Industrie submitted to the European Medicines Agency on 19 December 2024 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/8/2010 issued on 29 January 2010 and the decision EMA/PE/0000221282 issued on 30 October 2024.

The application for modification proposed changes to the agreed paediatric investigation plan.

The procedure started on 9 January 2025.

Scope of the modification

Some measures of the Paediatric Investigation Plan have been modified.

Opinion

1. The Paediatric Committee, having assessed the application in accordance with Article 22 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree to changes to the paediatric investigation plan in the scope set out in the Annex I of this opinion.

   The Paediatric Committee member of Norway agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the 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:

- children from birth to less than 1 year;
- for 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:

2.1.1. Indication(s) targeted by the PIP

Treatments of Type 1 diabetes mellitus

2.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From 1 to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Concentrate for solution for infusion, intravenous use

2.1.4. Measures

Area	Description
Quality	Not applicable.
Non-clinical	**Study 1** Female fertility and early embryonic development, toxicokinetic and immunophenotyping study in mice. **Study 2** Male fertility, toxicokinetic and immunophenotyping study in mice. **Study 3** Study deleted during the procedure EMA/PE/0000221282 Toxicity study in mice.
Clinical	**Study 4** Randomised open label multiple-dose pharmacokinetic, safety and efficacy study.

Area	Description
	Study 5. Study deleted during procedure EMA-PE-0000243095 **Study 6** Randomised, double-blind, multicentre, 4-arm, controlled, dose-ranging efficacy and safety study in children and adults with recent-onset Type 1 Diabetes mellitus. **Study 7** Multicentre, long-term efficacy and safety study in children and adults with recent-onset Type 1 Diabetes mellitus. **Study 8** Randomised, double-blind, placebo-controlled dose ranging 4-arm efficacy and safety study, in children and adults with recent-onset Type 1 Diabetes mellitus. **Study 9** Randomised, double-blind, multicentre, 2-arm, controlled, efficacy and safety study in children with stage 3 Type I Diabetes mellitus. **Study 10** Study added during the procedure EMA/PE/0000221282. Double-blind, randomised, placebo-controlled trial to evaluate the safety and the efficacy (superiority as compared to placebo) to slow the loss of beta cells and preserve beta cell function of two 12-day courses of teplizumab in children from 8 years to less than 18 years of age with newly diagnosed type 1 diabetes (T1D). **Study 11** Study added during the procedure EMA/PE/0000221282. Non-interventional, observational, extension study to collect long-term safety and other clinical data in participants who completed the PROTECT study (PRV-031-001, PIP Study 10). **Study 12** Study added during the procedure EMA/PE/0000221282. 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 than8 years with Stage 2 type 1 diabetes (T1D). *This study is the same as Study 2 (PRV-031-005 (PETITE-T1D)) of the teplizumab PIP EMEA-000524-PIP02-24 and subsequent modifications thereof.*
Modelling and simulation analyses	**Study 13** Study added during the procedure EMA/PE/0000221282.

Area	Description
	Population Pharmacokinetic/Pharmacodynamic (PK/PD) model. *This study is the same as Study 4 of the teplizumab PIP EMEA-000524-PIP02-24 and subsequent modifications thereof.*

3. Follow-up, completion and deferral of PIP

Measures to address long term follow-up of potential safety issues and efficacy in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By December 2034
Deferral for some or all studies 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.