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

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Decision Cover Letter

Decision of the licensing authority to:

agree a paediatric investigation plan and grant a deferral.

MHRA-100139-PIP01-21

Scope of the Application

Active Substance(s)

Teplizumab

Condition(s)

Prevention or delay of (clinical) type 1 diabetes mellitus

Pharmaceutical Form(s)

concentrate solution

Route(s) of Administration

Intravenous use

Name / Corporate name of the PIP applicant

Aventis Pharma Limited (trading as Sanofi)

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Aventis Pharma Limited (trading as Sanofi) submitted to the licensing authority on 15/07/2021 23:57 BST an application for a Paediatric Investigation Plan

The procedure started on 09/10/2024 15:29 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 agree a paediatric investigation plan and grant a 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.

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Final Decision Letter

MHRA-100139-PIP01-21

Of 09/08/2024 13:34 BST

On the adopted decision for Teplizumab (MHRA-100139-PIP01-21) 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 a paediatric investigation plan

This decision applies to a Paediatric Investigation Plan for Teplizumab, Concentrate solution , Intravenous use .

This decision is addressed to Aventis Pharma Limited (trading as Sanofi), 410 Thames Valley Park Drive Reading Berkshire, Reading, UNITED KINGDOM, RG6 1PT

ANNEX I

1. Waiver

1.1 Condition:

Not applicable

2. Paediatric Investigation Plan:

2.1 Condition(s):

Prevention or delay of (clinical) type 1 diabetes mellitus

2.2 Indication(s) targeted by the PIP:

Delay to clinical type 1 diabetes (T1D) in at-risk individuals from 0 to 18 years of age

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

All subsets of the paediatric population from birth to less than 18 years of age

2.4 Pharmaceutical Form(s):

Concentrate solution

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable
Non-Clinical Studies	0	Not applicable
Clinical Studies	2	Study 1 Double-blind, randomised, placebo-controlled trial to evaluate the efficacy of a single 14-day course of teplizumab to delay or prevent clinical type 1 diabetes compared to placebo in children from 8 to less than 18 years (and adults) at-risk for type 1 diabetes. Study 2 Single arm, open-label study to assess the safety and pharmacokinetics of a 14-day regimen of teplizumab in children ages 0 to <8 years with Stage 2 type 1 diabetes.
Extrapolation, Modeling & Simulation Studies	2	Study 3 Modelling and simulation study Study 4 Extrapolation study
Other Studies	0	Not applicable
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:	30/06/2027
Deferral of one or more studies contained in the paediatric investigation plan:	Yes