A Study to Investigate Efficacy and Safety of Teplizumab Compared With Placebo in Participants 1 to 25 Years of Age With Stage 3 Type 1 Diabetes

Status: RECRUITING

Eligibility Criteria

Sex: ALL
Age: 1 year to 25 years old
Healthy Volunteers: This study is NOT accepting healthy volunteers
Inclusion Criteria:

- Participants are eligible to be included in the study only if all of the following criteria apply:
- Participant must be 1 to 25 years of age inclusive, at the time of signing the informed consent.
- Participants diagnosed with T1D Stage 3 according to American Diabetes Association 2025 criteria
- Participants able to be randomized and initiate study drug within 8 weeks (56 days) of the Stage 3 T1D diagnosis
- Participants must be positive for at least one T1D autoantibody at screening:
- Glutamic acid decarboxylase (GAD-65),
- Insulinoma Antigen-2 (IA-2),
- Zinc-transporter 8 (ZnT8), or
- Insulin (if obtained not later than 14 days after exogenous insulin therapy initiation).
- Islet cell cytoplasmic autoantibodies (ICAs)
- Have random C-peptide level ≥0.2 nmol/L obtained at screening
- Enter Inclusion Criteria Sex
- Both male and female participants are eligible.
- Contraceptive use by women should be consistent with local regulations regarding the methods of contraception for those participating in clinical studies.
- A female participant is eligible to participate if she is not pregnant, and one of the following conditions applies:
- Is a woman of nonchildbearing potential (WONCBP) OR
- Is a woman of childbearing potential (WOCBP) and agrees to use a contraceptive method that is highly effective, with a failure rate of <1% during the study intervention period (to be effective before starting the intervention) and for at least 30 days after the last administration of study intervention.
- A WOCBP must have a negative highly sensitive pregnancy test at screening (serum) and within 24 hours (urine or serum as required by local regulations) before the first administration of study intervention.
- Lactating woman must interrupt breastfeeding and pump and discard breast milk during and for 20 days after last administration of study intervention.
- Capable of giving signed informed consent as described in Appendix 1 of the protocol which includes compliance with the requirements and restrictions listed in the informed consent form (ICF) and in the protocol.

Note: For minor participants, a specific ICF must also be signed by the participant's legally authorized representative (LAR).

Exclusion Criteria:

Participants are excluded from the study if any of the following criteria apply:

- Participant has diabetes other than autoimmune T1D that includes but is not limited to genetic forms of diabetes, maturity-onset diabetes of the young (MODY), diabetes secondary to medications or surgery and type 2 diabetes by judgement of the Investigator.
- Participant has an active serious infection and/or fever ≥38.5°C (101.3°F) within the 48 hours prior to the first dose (except if localized skin infection), or has chronic, recurrent or opportunistic infectious disease.
- At screening, participant has laboratory or clinical evidence of acute or clinically active infection with Epstein-Barr virus (EBV), cytomegalovirus (CMV).
- At screening, participant has positive serology for human immunodeficiency virus (HIV), hepatitis B (HBV), or hepatitis C (HCV).
- Participant has evidence of active or latent tuberculosis (TB) as documented by medical history and examination, chest X-rays (posterior anterior and lateral), and/or TB testing. Blood testing (eg, QuantiFERON® TB Gold test) is strongly preferred; if not available, any local approved TB test is allowed.
- Has other autoimmune diseases, (eg, rheumatoid arthritis, polyarticular juvenile idiopathic arthritis, psoriatic arthritis, ankylosing spondylitis, multiple sclerosis, systemic lupus erythematosus etc), except clinically stable autoimmune thyroid disease, or controlled celiac disease (at discretion of Investigator).
- Any clinically significant abnormality identified either in medical/surgical history or during screening evaluation (eg, physical examination, laboratory tests, vital signs), or any adverse event (AE) during screening period which, in the judgment of the investigator, would preclude safe completion of the study or constrains efficacy assessment.
- Participant has recent or planned vaccinations as follows:
- Live-attenuated (live) vaccines (eg, varicella, measles, mumps, rubella, cold-attenuated intranasal influenza vaccine, and smallpox) within the 8 weeks before first dose of the investigational medicinal product (IMP) or planned/required administration during treatment or up to 26 weeks after last IMP administration in any treatment course
- Inactivated or mRNA vaccines within 2 weeks before the first dose of IMP or planned required administration during treatment or up to 6 weeks after last IMP administration in any treatment course.
- Current or prior use (within 30 days before screening) of any anti-hyperglycemic agents other than insulin

- Past (within 30 days prior to screening) or current administration of any treatment that is known to cause a significant, ongoing change in the course of T1D or immunologic status, including but not limited to systemic corticosteroids (ie, oral, high doses of inhaled or injectable) with duration >14 days, adrenocorticotropic hormone, verapamil).
- Past systemic immunosuppression medicine or immune modulatory biologic therapy (such as monoclonal antibodies), within 3 months or 5 half-lifes (whichever is longer) prior to dosing.
- Current or prior (within 30 days before screening) use of any medication known to significantly influence glucose tolerance (eg, atypical antipsychotics, diphenylhydantoin, niacin).
- Participant has previously received teplizumab or other anti-CD3 treatment.
- Other medications not compatible or interfering with IMP at discretion of Investigator.
- Current enrollment OR past participation in another investigational study in which an investigational intervention (eg, drug, vaccine, invasive device) was administered within the last 8 weeks or 5 half-lifes, whichever is longer, prior to screening.
- Participant has any of the following laboratory parameters, at screening prior to first dose:
- Lymphocyte count: <1000/µL,
- Neutrophil count: <1500/µL (< 1000 /µL in participants with documented Duffy-null genotype),
- Platelet count: <150,000 platelets/µL,
- Hemoglobin: <10 g/dL,
- Aspartate aminotransferase (AST) >2.0 × upper limit of normal (ULN),
- Alanine aminotransferase (ALT) >2.0 × ULN,
- Total bilirubin >1.5 × ULN with the exception of participants with the diagnosis of Gilbert's syndrome who may be eligible provided they have no other causes leading to hyperbilirubinemia

The above information is not intended to contain all considerations relevant to a participant's potential participation in a clinical trial.

Conditions & Interventions

Interventions:
DRUG: Teplizumab, OTHER: Placebo
Conditions:
Type 1 Diabetes Mellitus

More Information

Description:
This is a multicenter, randomized, double-blind, parallel, placebo-controlled Phase 3, 2-arm study for treatment. The purpose of this study is to measure change in glycemic control and prandial insulin independency over 52 weeks with teplizumab compared with placebo, both administered by intravenous (IV) infusion, in participants with recently diagnosed Stage 3 type 1 diabetes (T1D) aged 1 to 25 years, on standard insulin therapy.

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