Study to Assess Safety, Efficacy, and Cellular Kinetics of YTB323 in Generalized Myasthenia Gravis

Last Update: Mar 17, 2025
An Open-label, Multi-center, Phase I/II Study to Assess Safety, Efficacy, and Cellular Kinetics of YTB323 in Participants With Treatment-resistant Generalized Myasthenia Gravis
ClinicalTrials.gov Identifier:
NCT06704269
Novartis Reference Number:CYTB323O12101
See if you Pre-qualify
All compounds are either investigational or being studied for (a) new use(s). Efficacy and safety have not been established. There is no guarantee that they will become commercially available for the use(s) under investigation.

Study Description

This is a phase I/II study to assess safety, efficacy, and cellular kinetics of YTB323 in participants with treatment-resistant generalized myasthenia gravis. YTB323 is a Biological CAR-T cell therapy. This is an open-label, multi-center, non-confirmatory study intended to assess safety, efficacy, and cellular kinetics of YTB323 treatment in participants with treatment-resistant generalized myasthenia gravis in order to enable a benefit to risk assessment for further development in generalized myasthenia gravis (gMS). The study plans to enroll approximately 15 participants with treatment-resistant gMG. The study utilizes a single dose design across 2 cohorts, consisting of a sentinel cohort of 3 patients followed by an expansion cohort of an additional 12 patients.

All participants dosed with YTB323 will be followed until 15 years after YTB323 administration in the Long-Term Follow-up (LTFU).

Condition
Generalized Myasthenia Gravis
Phase
Phase1, Phase2
Overall Status
Recruiting
Number of Participants
15
Start Date
Mar 31, 2025
Completion Date
Aug 02, 2029
Gender
All

Age(s)
18 Years - 65 Years (Adult, Older Adult)

Interventions

Genetic

YTB323

CAR-T cell suspension for intravenous infusion

Eligibility Criteria

Inclusion Criteria:

1. Confirmed gMG diagnosis supported by the following:

* Documented report of positive serology testing for either AChR antibodies or MuSK antibodies at screening AND at least one of the following:
* History of abnormal neuromuscular transmission test demonstrated by repetitive nerve stimulation or single-fiber electromyography
* History of positive acetylcholinesterase inhibitor test
* Improvement in MG signs on an oral acetylcholinesterase inhibitor as assessed by the treating physician
2. MGFA Class III-IVa (gMG) at screening
3. Treatment-resistant gMG as defined by: MG-ADL score ≥ 6 at screening despite adequate treatment trials with at least two different non-steroidal immunosuppressive drugs given at adequate doses and duration of therapy.
4. If on chronic corticosteroids, the ability and willingness to taper to a maximum dose of 10 mg prednisolone daily or equivalent at least one week before leukapheresis
5. If treated with cholinesterase inhibitors, patients must be on a stable dose for at least two weeks prior to screening

Exclusion Criteria:

1. Exclusively ocular myasthenia gravis (MGFA I), mild symptoms (MGFA II), or severe bulbar disease or MG crisis, MGFA Class IVb or V at screening
2. History of bone marrow/hematopoietic stem cell or solid organ transplantation.
3. Clinically significant active, opportunistic, chronic or recurrent infection (including positive for hepatitis B or hepatitis C) confirmed by clinical evidence, imaging, or positive laboratory tests one month prior to leukapheresis
4. Other uncontrolled disease states, such as asthma, or inflammatory bowel disease, where flares are commonly treated with oral or parenteral corticosteroids, at screening
5. Participants with a known immunodeficiency syndrome (AIDS, hereditary immune deficiency, drug induced immune deficiency), or tested positive for HIV antibody, at screening
6. Prior treatment with anti-CD19 therapy, adoptive T cell therapy or any prior gene therapy product (e.g. CAR-T cell therapy).

Other protocol-defined inclusion/exclusion criteria may apply

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