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A Phase III Study to Investigate Efficacy, Safety and Tolerability of Iptacopan Compared With Placebo in Participants Aged 18 to 75 Years With gMG.

Last Update: Apr 09, 2025
A Randomized, Double-blind, Placebo-controlled Phase III Study to Evaluate the Efficacy, Safety, and Tolerability of Iptacopan in Patients With Generalized Myasthenia Gravis (gMG), Followed by an Open Label Extension Phase
ClinicalTrials.gov Identifier:
NCT06517758
Novartis Reference Number:CLNP023Q12301
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

The study is a randomized, double-blind, placebo-controlled, multicenter, Phase III study, to evaluate efficacy, safety and tolerability of iptacopan in patients with AChR+ gMG who are on stable SOC treatment. Participants who meet the eligibility criteria will be randomized in a ratio of 1:1, to receive either iptacopan or matching placebo, for 6 months (180 days) while continuing on a stable SOC treatment. The randomization will be stratified based on region. The study consists of a 6-month double-blind treatment period for the primary efficacy and safety analysis followed by a 24 month open label extension period. A safety follow up assessment will be performed, one 7 days after the last administration of study treatment and one 30 days after the last administration of study treatment for all participants.

Condition
Generalized Myasthenia Gravis
Phase
Phase3
Overall Status
Recruiting
Number of Participants
146
Start Date
Jul 31, 2024
Completion Date
May 27, 2032
Gender
All

Age(s)
18 Years - 75 Years (Adult, Older Adult)

Interventions

Drug

Iptacopan

Hard gelatin capsule
Other

Matching Placebo

Hard gelatin capsule

Eligibility Criteria

Inclusion Criteria:

* Adult patients with generalized Myasthenia Gravis (age 18-75 years)
* Positive serology testing for AChR+ antibody at screening
* Myasthenia Gravis Foundation of America (MGFA) Class II-IV gMG and likely not in need v of a respirator for the duration of the study, as judged by the Investigator.
* The confirmation of the diagnosis of gMG should be documented and supported by ≥1 of the following 3 tests:
* History of abnormal neuromuscular transmission demonstrated by single-fiber electromyography or repetitive nerve stimulation.
* History of positive edrophonium chloride test
* Patient has demonstrated improvement in MG signs on oral acetylcholinesterase inhibitors as assessed by the treating physician.
* Baseline MG-ADL score ≥6, with ≥50% of the total score due to non-ocular symptoms
* Participants not optimally controlled for ≥ 6 months on
* just one NSIST; or
* two or more NSISTs; or
* on frequent (at least quarterly) plasmapheresis, plasma exchange, or intravenous immunoglobulin to control symptoms despite treatment with steroids and NSISTs; or
* one of the following gMG treatments:
* a FcRN antagonist approved for gMG
* rituximab
* other approved gMG therapies excluding complement inhibitors.
* Consistent with all other iptacopan trials, participants will have to be vaccinated against Neisseria meningitidis and Streptococcus pneumoniae. In addition, participants will be vaccinated against Haemophilus influenzae, depending on the local regulations and on the availability of this vaccine in the countries of study conduct. The vaccination will be performed at least 2 weeks prior to first dosing with iptacopan, covering as many serotypes as possible. If iptacopan treatment will start earlier than 2 weeks post vaccination, prophylactic antibiotic treatment must be initiated and administered until 2 weeks post vaccination.

Exclusion Criteria:

* Have been treated with intravenous immunoglobulin (IVIG)/plasma exchange (PLEX) in the past month, with rituximab in the past 6 months, eculizumab in the past 2 months, ravulizumab or other complement inhibitors in the past 3 months, efgartigimod or other anti- FcRn therapies in the past 3 months, or had a thymectomy in the past 6 months or a planned thymectomy during the trial period.
* Participants with clinically significant active or chronic uncontrolled bacterial, viral, or fungal infection at screening, including patients who test positive for an active viral infection at screening with: Active Hepatitis B Virus (HBV): serologic panel test results indicative of an active (acute or chronic) infection; Active Hepatitis C Virus (HCV): serology positive for HCV-Ab; Human Immunodeficiency Virus (HIV) positive serology associated with an Acquired Immune Deficiency Syndrome (AIDS)-defining condition or with a cluster of differentiation 4 (CD4) count

* 200 cells/mm3
* Female participants who are pregnant or lactating, or are intending to become pregnant.
* Women of child-bearing potential, defined as all women physiologically capable of becoming pregnant, unless they are using effective methods of contraception during dosing of study treatment.
* Active systemic bacterial, viral (including COVID-19) or fungal infection or any major episode of infection that required hospitalization or injectable antimicrobial therapy within 14 days prior to study drug administration.
* History of recurrent invasive infections caused by encapsulated organisms, e.g., N. meningitidis and S. pneumoniae.
* Presence of fever ≥ 38 °C (100.4 °F) within 7 days prior to study drug administration

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