A randomized, double-blind, placebo-controlled phase III study to evaluate the efficacy, safety, and tolerability of iptacopan in patients with generalized Myasthenia Gravis, followed by an open label extension phase

Estado	**Tipo de Participantes**	**Rangos de Edad**
Reclutando	No aportado	Mayores de 64 , Adultos (18-64 años)
Género	**Fases**	**Participantes esperados**
Ambos	Fase III	81
Resultados	**Bajo nivel intervención**	**Enfermedad rara**
Sin resultados	No	Si
Cobertura geográfica	**Ámbitos del ensayo**	**Terapia avanzada**
Sin determinar	tratamiento, seguridad, eficacia, farmacocinética, farmacodinamica, farmacogenética, farmacoeconómica	NO

Información

Identificador
2023-507064-39-00 (CTIS)

Cod. Protocolo
CLNP023Q12301

Área terapéutica
Enfermedades [C] - Sistema Nervioso [C10]|

Enfermedad investigada
generalized Myasthenia Gravis

Título Científico
A randomized, double-blind, placebo-controlled phase III study to evaluate the efficacy, safety, and tolerability of iptacopan in patients with generalized Myasthenia Gravis, followed by an open label extension phase

Justificación

No aportado

Objetivo Principal

To demonstrate efficacy of iptacopan compared to placebo in patients with gMG on stable SOC in reducing the total score of the Myasthenia Gravis Activity of Daily Living (MG-ADL) scale at 6 months (Day 180) of treatment

Variables de Evaluación Primaria

Change from baseline to Month 6 in Myasthenia Gravis Activity of Daily Living (MG-ADL) total score

Momentos temporales de evaluación primaria

No aportado

Objetivo Secundario

To assess whether iptacopan is superior to placebo in patients with gMG in reducing Quantitative MG (QMG) total score at 6 months of treatment
To assess whether iptacopan is superior to placebo on the proportion of study participants achieving a reduction from baseline to Month 6 of QMG total score 5 points without rescue medication and/or strongly confounding prohibited medication
To assess whether iptacopan is superior to placebo on the proportion of study participants achieving a reduction from baseline to Month 6 of MG-ADL total score 3 points without rescue medication and/or strongly confounding prohibited medication
To assess whether iptacopan is superior to placebo in reducing MGC total score at Month 6 of treatment
To assess whether iptacopan is superior to placebo in reducing MG-QOL15r survey score at Month 6 of treatment
To evaluate the safety and tolerability of Iptacopan compared to placebo in patients with gMG
To evaluate the efficacy of iptacopan compared to placebo in patients with gMG on other efficacy endpoints
To assess long-term effect of iptacopan in patients with gMG in reducing the total score of the MG-ADL during the Extension Part
To assess long-term effect of iptacopan in patients with gMG on the proportion of participants achieving a reduction in oral corticosteroids (OCS) dose compared to Core Part that was maintained up to end of Extension Part
To assess long-term safety and tolerability of iIptacopan in patients with gMG
To assess whether iptacopan is superior to placebo in achieving Minimal Symptom Expression (MSE) at Month 6, defined as an MG-ADL of 0 or 1 at Month 6 without rescue therapy and/or strongly confounding prohibited medication

Variables de Evaluación Secundaria

Change from baseline to Month 6 in Quantitative MG (QMG) total score
Proportion of participants with 5 points reduction from baseline to Month 6 of QMG total score without rescue

medication and/or strongly confounding prohibited medication
Proportion of participants with 3 points reduction from baseline to Month 6 of MG-ADL total score without rescue medication and/or strongly confounding prohibited medication
Change from baseline to Month 6 in Myasthenia Gravis Composite (MGC) total score
Change from baseline to Month 6 in revised MG Quality of Life Questionnaire (MG-QOL15r) survey score
Incidence of adverse events and changes in clinical laboratory values, vital signs, and electrocardiograms, Columbia Suicide Severity Rating
Proportion of time during which participants showed a reduction of 2 points in MG-ADL total score that was maintained up to Month 6
Proportion of early MG-ADL responders during treatment (early responders with first MG-ADL improvement from baseline of 2 points occurring by week 4
Change from baseline to Month 6 in EuroQol-5 Dimensions-5 Level (EQ-5D-5L)
Change from baseline in MG-ADL total score
Proportion of participants achieving a reduction in oral corticosteroids (OCS) dose compared to Core Part that was maintained up to end of Extension Part
Incidence of adverse events and changes in clinical laboratory values, vital signs, and electrocardiograms, Columbia Suicide Severity Rating
Proportion of participants achieving MSE at Month 6, defined as MG-ADL score of 0 or 1 at Month 6 without rescue therapy and/or strongly confounding prohibited medication
Proportion of participants with reduction of 3 points from baseline to Month 6 in MGC total score

Momentos temporales de evaluación secundaria

No aportado

Criterios de Inclusión

Adult patients with gMG (age 18-85 years) at screening
Positive serology testing for AChR+ antibody at screening
Myasthenia Gravis Foundation of America (MGFA) Class II-IV gMG at screening and likely not in need 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 test with short-acting acetylcholinesterase inhibitors (e.g. neostigmine or edrophonium chloride) 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 receiving at least one of the following treatments for gMG for 6 months prior to baseline One or more NSISTs; or plasmapheresis, plasma exchange, or intravenous immunoglobulin (at least quarterly) to control symptoms despite treatment with steroids and NSISTs; or an approved FcRN antagonist; or rituximab; or other approved gMG disease modifying therapies excluding complement inhibitors
Vaccination against Neisseria meningitidis and Streptococcus pneumoniae infection is required prior to the start of study treatment. If the participant has not been previously vaccinated, or if a booster was required, the vaccine should be given according to local guidelines at least 2 weeks prior to first study drug administration. If study treatment has to start earlier than 2 weeks post-vaccination, prophylactic antibiotic treatment should be initiated at the start of the study treatment and continued until at least 2 weeks after vaccination or booster was completed..
Note: for US sites participating in Study CLNP023Q12301, the completion of the meningococcal vaccination or booster is required for patients with gMG prior to initiating study treatment, irrespective of prophylactic antibiotic use.
If not previously vaccinated, vaccination against Haemophilus influenzae infections should be given, if available and according to local guidelines, at least 2 weeks prior to first study drug administration.

Criterios de Exclusión

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); Active Hepatitis C Virus (HCV); 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 and an additional one week following cessation 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

Calendario

(Última actualización: 27/01/2026)

Autorización	Inicio de Ensayo	Inclusión Primer Paciente	Interrumpido	Reiniciado
26/06/2024	16/10/2024	16/10/2024	No aportado	No aportado

Fin de reclutamiento	Fin prematuro (España)	Fin prematuro (Global)	Fin del ensayo en España	Fin del ensayo global
No aportado	No aportado	No aportado	No aportado	No aportado

Promotor

Novartis Pharma AG Switzerland

Lichtstrasse 35

Contact Person

Novartis Pharma Arzneimittel GmbH

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Monetary support: N/A

Tipo de promotor: Comercial

Ceim

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Centros

Activo (26/08/2025)

Bellvitge University Hospital

L'Hospitalet De Llobregat

BARCELONA

Neurology

Activo (11/08/2025)

Complexo Hospitalario Universitario De Santiago

Santiago De Compostela

CORUÑA

Neurology

Activo (03/09/2024)

Hospital Clinic De Barcelona

Barcelona

BARCELONA

Neurology

Activo (23/07/2024)

Hospital General Universitario Dr. Balmis

Alicante

ALICANTE

Neurology

Activo (30/07/2024)

Hospital Ruber Juan Bravo

Madrid

MADRID

Neurology

Activo (27/08/2025)

Hospital Universitari Arnau De Vilanova De La Gerencia Territorial De Lleida

Lleida

LÉRIDA

Neurology

Activo (04/07/2025)

Hospital Universitario Regional De Malaga

Malaga

MÁLAGA

Neurology

Activo (28/07/2025)

Hospital Universitario Y Politecnico La Fe

Valencia

VALENCIA

Neurology

Medicamentos

IPTACOPAN
HARD GELATIN CAPSULES
ORAL

-

Principios Activos: N/A

Experimental

Sin resultados

A randomized, double-blind, placebo-controlled phase III study to evaluate the efficacy, safety, and tolerability of iptacopan in patients with generalized Myasthenia Gravis, followed by an open label extension phase

State	Type of participants	Age Ranges
Recruiting	Not provided	Older than 64 , Adults (18-64 years)
Gender	**Phases**	**Expected Participants**
Both	Phase III	81
Results	**Low level of intervention**	**Rare disease**
No results	No	Yes
Geographic coverage	**Areas of the study**	**Advanced therapy**
Undetermined	treatment, safety, effectiveness, pharmacokinetics, pharmacodynamics, pharmacogenetics, pharmacoeconomic	NO

Information

Identifier
2023-507064-39-00 (CTIS)

Protocol Code
CLNP023Q12301

Therapeutic area
Diseases [C] - Nervous System Diseases [C10]|

Investigated Disease
generalized Myasthenia Gravis

Scientific Title
A randomized, double-blind, placebo-controlled phase III study to evaluate the efficacy, safety, and tolerability of iptacopan in patients with generalized Myasthenia Gravis, followed by an open label extension phase

Rationale

Not provided

Main Objective

To demonstrate efficacy of iptacopan compared to placebo in patients with gMG on stable SOC in reducing the total score of the Myasthenia Gravis Activity of Daily Living (MG-ADL) scale at 6 months (Day 180) of treatment

Primary Endpoints

Change from baseline to Month 6 in Myasthenia Gravis Activity of Daily Living (MG-ADL) total score

Temporary moments of secondary assessment

Not provided

Secondary Objective

To assess whether iptacopan is superior to placebo in patients with gMG in reducing Quantitative MG (QMG) total score at 6 months of treatment
To assess whether iptacopan is superior to placebo on the proportion of study participants achieving a reduction from baseline to Month 6 of QMG total score 5 points without rescue medication and/or strongly confounding prohibited medication
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Secondary Endpoints

Change from baseline to Month 6 in Quantitative MG (QMG) total score
Proportion of participants with 5 points reduction from baseline to Month 6 of QMG total score without rescue

medication and/or strongly confounding prohibited medication
Proportion of participants with 3 points reduction from baseline to Month 6 of MG-ADL total score without rescue medication and/or strongly confounding prohibited medication
Change from baseline to Month 6 in Myasthenia Gravis Composite (MGC) total score
Change from baseline to Month 6 in revised MG Quality of Life Questionnaire (MG-QOL15r) survey score
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Proportion of time during which participants showed a reduction of 2 points in MG-ADL total score that was maintained up to Month 6
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Proportion of participants with reduction of 3 points from baseline to Month 6 in MGC total score

Temporary moments of secondary assessment

Not provided

Inclusion criteria

Adult patients with gMG (age 18-85 years) at screening
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Exclusion criteria

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Calendar

(Last Update: 27/01/2026)

Authorization	Start of Trial	First patient inclusion	Halted	Restarted
26/06/2024	16/10/2024	16/10/2024	Not aported	Not aported

End of recruitment	Premature end (Spain)	Premature End (Global)	Trial end (Spain)	Trial end (Global)
Not aported	Not aported	Not aported	Not aported	Not aported

Sponsor

Novartis Pharma AG Switzerland

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Contact Person

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Monetary support: N/A

Sponsor type: Commercial

Ceim

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Sites

Active (26/08/2025)

Bellvitge University Hospital

L'Hospitalet De Llobregat

BARCELONA

Neurology

Active (11/08/2025)

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Neurology

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MADRID

Neurology

Active (27/08/2025)

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Lleida

LÉRIDA

Neurology

Active (04/07/2025)

Hospital Universitario Regional De Malaga

Malaga

MÁLAGA

Neurology

Active (28/07/2025)

Hospital Universitario Y Politecnico La Fe

Valencia

VALENCIA

Neurology

Medication

IPTACOPAN
HARD GELATIN CAPSULES
ORAL

-

Active Principles: N/A

Experimental

No results