



MGFA Study Web Content:

Title of Study here

Study Purpose: The primary objective of this study is to evaluate the efficacy of eculizumab in the treatment of pediatric refractory generalized myasthenia gravis (gMG) based on change from Baseline in the Quantitative Myasthenia Gravis score for disease severity (QMG).

Recruitment Information:

Study Type: An Open-Label, Multicenter Study to Evaluate the Efficacy, Safety, Pharmacokinetics, and Pharmacodynamics of Eculizumab in Pediatric Patients with Refractory Generalized Myasthenia Gravis

Status: Recruiting

Target Patient: Male or female between 6 and 18 years of age, with presence of refractory generalized Myasthenia Gravis (gMG), a positive serologic test for AChR Ab, and a MGFA Clinical Classification of Class II to IV.

Eligible Ages: 6 to < 18

Link to appropriate websites [Click here to enter text.](#)

Inclusion and Exclusion Criteria:

Inclusion Criteria: - Male or female pediatric participants 6 to <18 years of age at time of assent/consent. - Vaccinated against Neisseria meningitidis. - Documented vaccination against Haemophilus influenzae and Streptococcus pneumoniae infections prior to dosing as per local and country specific immunization guidelines for the appropriate age group. - Diagnosis of MG confirmed by positive serologic test for anti-acetylcholine receptor antibodies at Screening, and 1 of the following: (a) history of abnormal neuromuscular transmission test demonstrated by single-fiber electromyography or repetitive nerve stimulation; (b) history of positive anticholinesterase test (for example, edrophonium chloride or neostigmine test); or (c) participant demonstrated improvement in MG signs on oral acetylcholinesterase inhibitors, as assessed by the Investigator. - Presence of refractory gMG, defined



as participants with gMG who have 1 or more of the following: (a) failed treatment ≥ 1 year with at least 1 immunosuppressive therapies (IST), defined as follows: (1) persistent weakness with impairment of activities of daily living; (2) myasthenia gravis (MG) exacerbation and/or crisis while on treatment; or (3) intolerance to ISTs due to side effect or comorbid condition(s). (b) Require maintenance plasma exchange (PE) or intravenous immunoglobulin (IVIg) to control symptoms; and/or (c) in the opinion of the Investigator, MG poses a significant functional burden despite current MG treatment.

- MGFA Clinical Classification of Class II to IV at Screening. - QMG total score ≥ 12 at Screening. - All MG-specific treatment has been administered at a stable dosing regimen of adequate duration prior to Screening.

Exclusion Criteria: - Parent or legal guardian is an Alexion employee. - Any active or untreated thymoma. History of thymic carcinoma or thymic malignancy unless deemed cured by adequate treatment with no evidence of recurrence for ≥ 5 years before Screening. - History of thymectomy within 12 months prior to Screening.

- Are pregnant or lactating. - Any unresolved acute, or chronic, systemic bacterial or other infection, which is clinically significant in the opinion of the Investigator and has not been treated with appropriate antibiotics. - Use of PE within 4 weeks prior to first dose. - Use of rituximab within 6 months prior to first dose. - Participation in another interventional treatment study or use of any experimental therapy within 30 days before initiation of study drug on Day 1 in this study or within 5 half-lives of that investigational product, whichever is greater. - Have previously received treatment with eculizumab or other complement inhibitors

Study Information:

Sponsor: Alexion Pharmaceuticals

Principal Investigator: [Click here to enter text.](#)

Study Coordinator: [Click here to enter text.](#)

Type of Study: Interventional

Study Duration: 52 Weeks Primary; 104 Weeks Extension

Single Center/Multi-center: Multi-center

Travel Funds Available: Y N



Find A Center Near You:

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PI: Click here to enter text.

Coordinator: Click here to enter text.