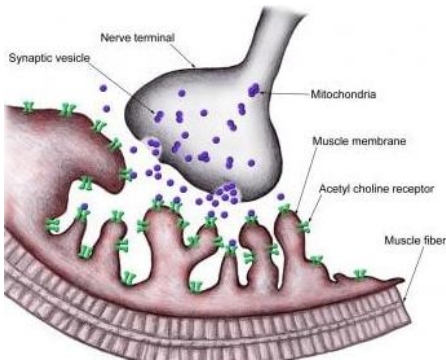
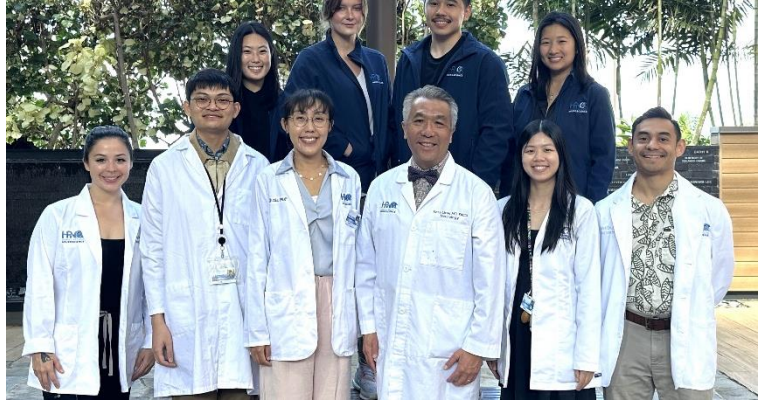




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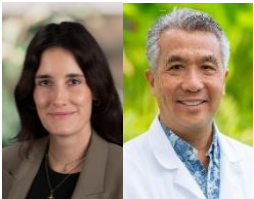
According to [Myasthenia Gravis Foundation](#), Generalized myasthenia gravis is a form of MG with generalized muscle weakness, approximately 85% of all MG patients. Symptoms may include droopy eyelids (ptosis) and/ or double vision, difficulty speaking, difficulty breathing, problems chewing and swallowing, trouble performing everyday tasks, or generalized muscle weakness.

Iptacopan (LNP023) is a small molecule that inhibits complement factor B (FB). When taken orally, iptacopan binds to FB and prevents the formation of the alternative pathway (AP) C3-convertase (C3bBb). This limits the cleavage of C3 to the active fragment C3b, which may prevent C3b-mediated extravascular hemolysis (EVH) in certain disorders. Iptacopan also prevents the formation of AP C5 convertase, which prevents downstream cell destruction, inflammation, and excessive complement deposition.



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 Standard of Care 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 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. For more information, call (808) 564-6141 or [NIH Info](#).



“Our Hawaii patients, caregivers, families, neurologists & researchers are honored to contribute to the development of Novel Neuromuscular Therapy” [Natalia Gonzalez, MD](#), Director of [Hawaii ALS and Neuromuscular Center](#) and Sub investigator [Neuromuscular Research Unit](#), who is dual fellowship trained in Neuromuscular and

Neuroimmunology and Kore Kai Liow, MD, Neurologist & Principal Investigator, [Neuromuscular Research Unit](#) Clinical Professor of Medicine (Neurology), Graduate Faculty, Clinical & Translational Research, University of Hawai‘i John Burns School of Medicine.

[Hawaii ALS and Neuromuscular Center](#) is a member of MG Foundation of America Partners in MG Care. **Dedicated Neuromuscular Research Hotline (808) 564-6141**



CLINICAL TRIALS

