Supplementary Online Content

Howard JF Jr, Nowak RJ, Wolfe GI, et al; the Zilucoplan MG Study Group. Clinical effects of the self-administered subcutaneous complement inhibitor zilucoplan in patients with moderate to severe generalized myasthenia gravis: results of a phase 2 randomized, double-blind, placebo-controlled, multicenter clinical trial. Published online February 17, 2020. *JAMA Neurol*. doi:10.1001/jamaneurol.2019.5125

This supplementary material has been provided by the authors to give readers additional information about their work.

eTable 1. Efficacy of Zilucoplan Is Independent of Prior Therapies

Prior Therapy	Treatment Interaction *P* Value (zilucoplan 0.3 mg/kg vs placebo)			
	QMG	MG-ADL	MGC	MG-QoL15r
IST	0.59	0.43	0.62	0.62
IVIg	0.74	0.68	0.64	0.77
PLEX	0.21	0.49	0.29	0.45

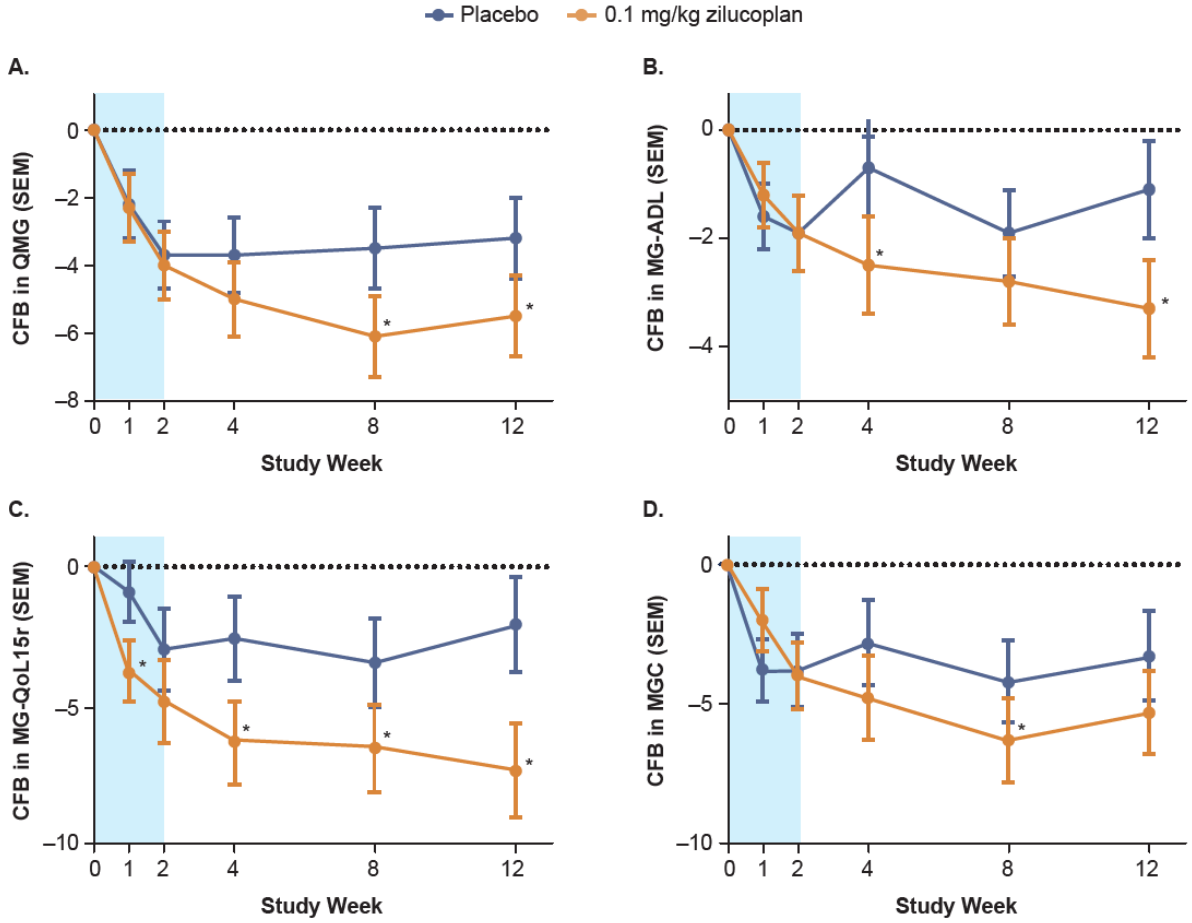
Abbreviations: IST, immunosuppressive therapy; IVIg, intravenous immunoglobulin; MG-ADL, Myasthenia Gravis Activities of Daily Living; MGC, Myasthenia Gravis Composite; MG-QoL15r, Myasthenia Gravis Quality-of-Life revised scale; PLEX, plasma exchange; QMG, Quantitative Myasthenia Gravis.

eTable 2. Treatment-Emergent AEs and Injection-Site Reactions

Patients, n	Placebo (n = 15)	Zilucoplan 0.1 mg/kg (n = 15)	Zilucoplan 0.3 mg/kg (n = 14)
Patients with AEs	12	15	12
Patients with treatment-related AEs	3	8	3
Patients with serious AEs	3	0	5
Patients with treatment-related serious AEs	0	0	0
Patients with most common treatment-related AEs[a]			
Nausea	0	2	0
Injection-site bruising	1	2	0
Injection-site scab	0	3	0
Contusion	0	1	1
Headache	1	4	2
Patients with injection-site reactions	2	4	3

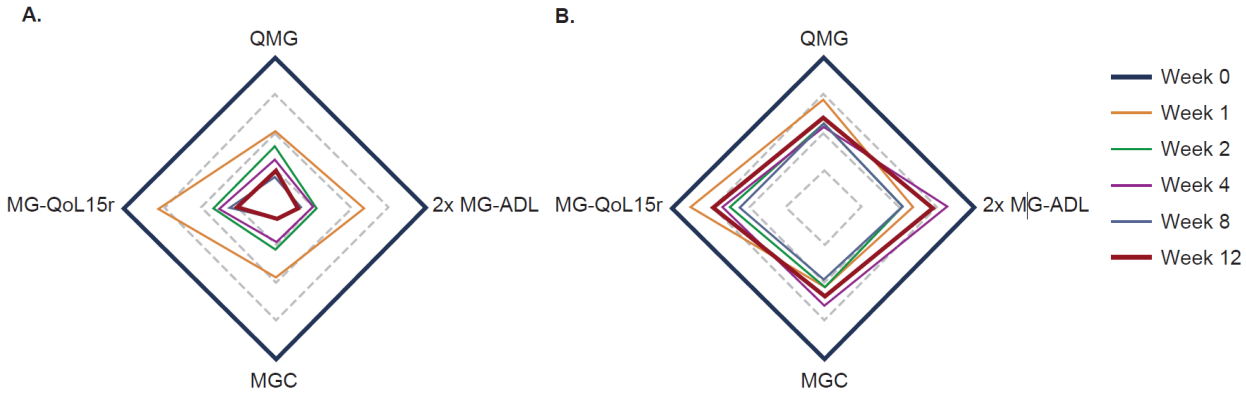
[a]Occurring in >1 patient.
Abbreviation: AE, adverse event.

eFigure 1. Change From Baseline Over 12 Weeks for 0.1 mg/kg Zilucoplan and Placebo in (A) QMG (B) MG-ADL, (C) MG-QoL15r, and (D) MGC Scores



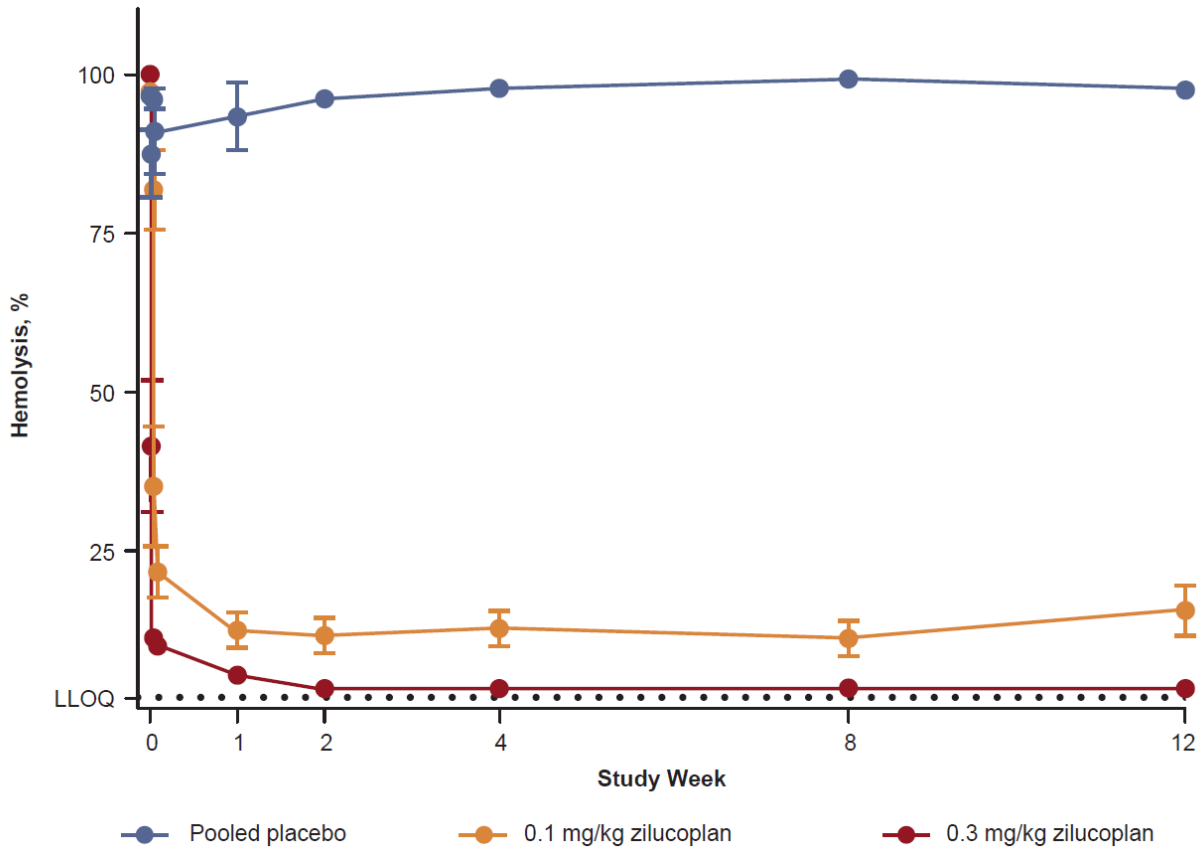
Blue shading highlights the delayed onset of clinical effect of the 0.1-mg/kg dose of zilucoplan as compared to the 0.3-mg/kg dose (Figure 2). *P < 0.10. CFB, change from baseline; MG-ADL, Myasthenia Gravis Activities of Daily Living; MGC, Myasthenia Gravis Composite; MG-QoL15r, Myasthenia Gravis Quality-of-Life revised scale; QMG, Quantitative Myasthenia Gravis; SEM, standard error of the mean.

eFigure 2. Radar Plots Suggest Consistency of Improvement Across All 4 End Points When Treated With (A) 0.3 mg/kg Zilucoplan Daily Subcutaneously vs (B) Placebo.



The outermost line on each radar plot represents the baseline (bold dark blue; ie, “0’ for all end points), and each concentric dashed square represents a reduction from baseline by 2 points. The center of each plot represents a reduction of 8 points. Mean CFB values for QMG, MGC, and MG-QoL15r are shown. Given the lower dynamic range of the MG-ADL, 2x MG-ADL is represented on the graphs for illustrative purposes. CFB at 12 weeks (bold red) and other time points are as indicated. 2x MG-ADL, 2-fold mean MG-ADL; CFB, change from baseline; MG-ADL, Myasthenia Gravis Activities of Daily Living; MGC, Myasthenia Gravis Composite; MG-QoL15r, Myasthenia Gravis Quality-of-Life revised scale; QMG, Quantitative Myasthenia Gravis.

eFigure 3. Mean Complement Activity as Measured by Sheep Red Blood Cell Assay (% Hemolysis)



LLOQ, lower limit of quantification.