

AAN 2019 Emerging Science Session

Zilucoplan, a Subcutaneously Self-Administered Peptide Inhibitor of Complement Component 5 (C5), for the Treatment of Generalized Myasthenia Gravis: Results of a Phase 2 Randomized, Double-Blind, Placebo-Controlled Trial and Open-Label Long-Term Extension

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May 7, 2019, Philadelphia, PA

Randomized, Double-Blind, Placebo-Controlled Phase 2 Study of Zilucoplan in a Broad Generalized Myasthenia Gravis Population

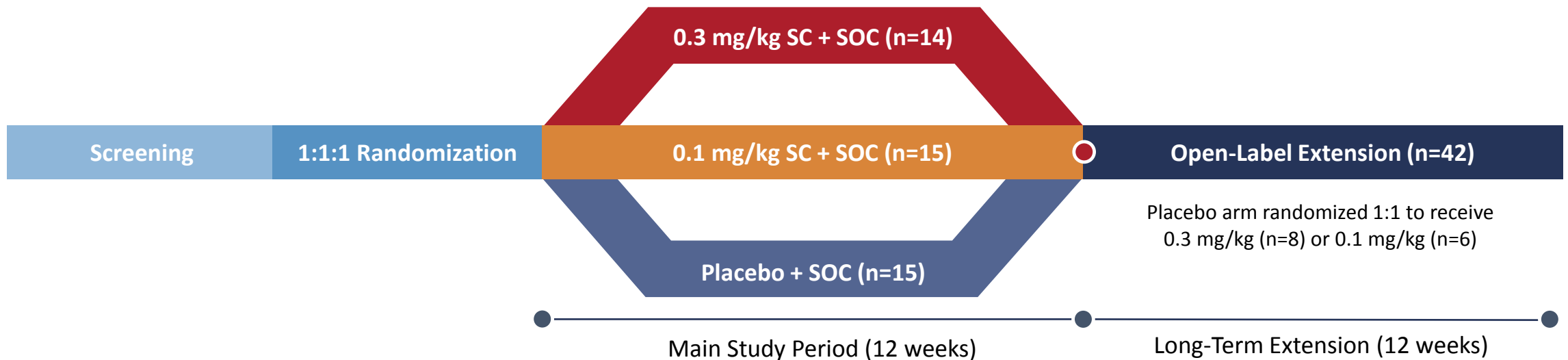
Broad Patient Population:

- Generalized myasthenia gravis (MGFA class II-IVa)
- Acetylcholine receptor (AChR) antibody positive
- QMG score of ≥ 12
- Stable doses of corticosteroids and/or immunosuppressants
- No requirement to be “refractory” or to have failed multiple prior therapies
- Patients must be vaccinated against meningococcus

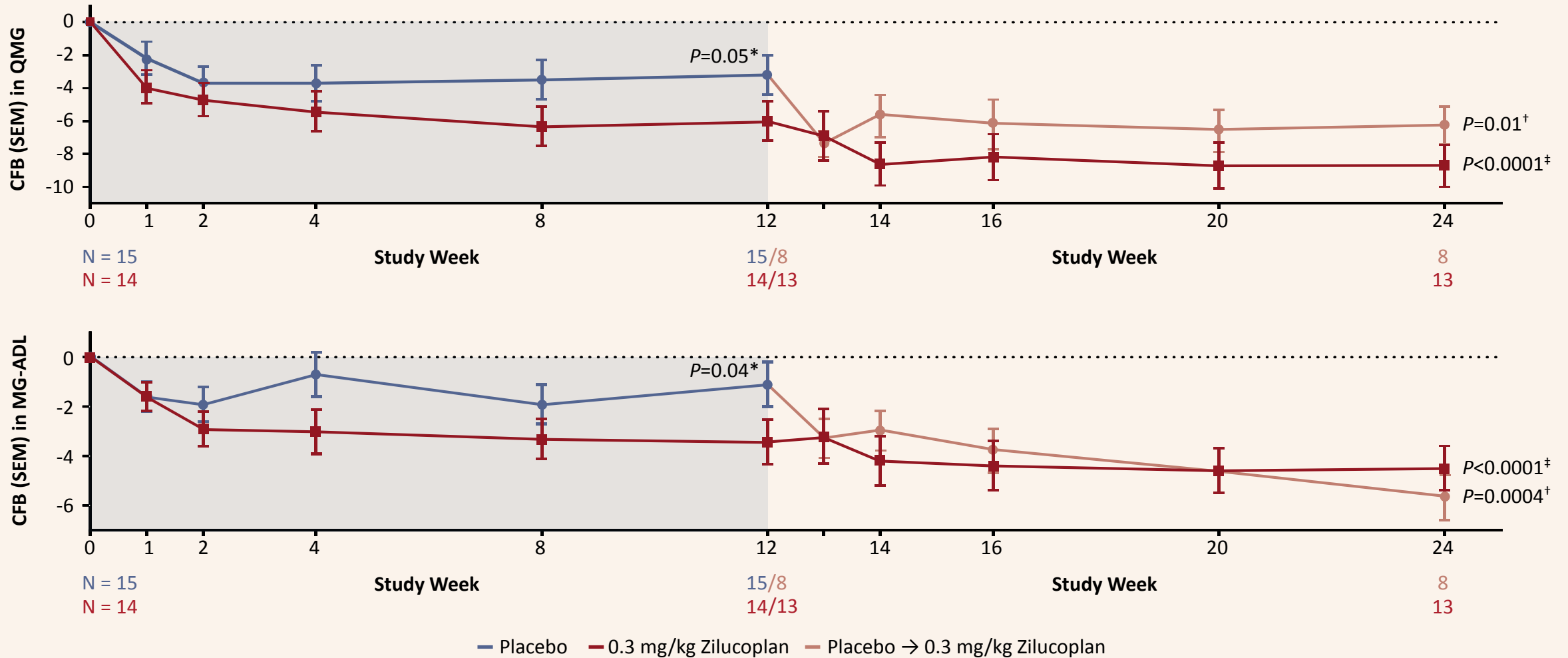
Endpoints:

- Primary: Change in QMG score from baseline to week 12
- Secondary: Change in MG-ADL, MG Composite, and MGQoL15r scores from baseline to week 12
- Pre-specified significance testing at a 1-sided alpha of 0.1

Enrollment: 44 patients (vs. target of 36)



Zilucoplan Achieved Rapid, Clinically Meaningful, Statistically Significant, and Sustained Reductions in QMG and MG-ADL



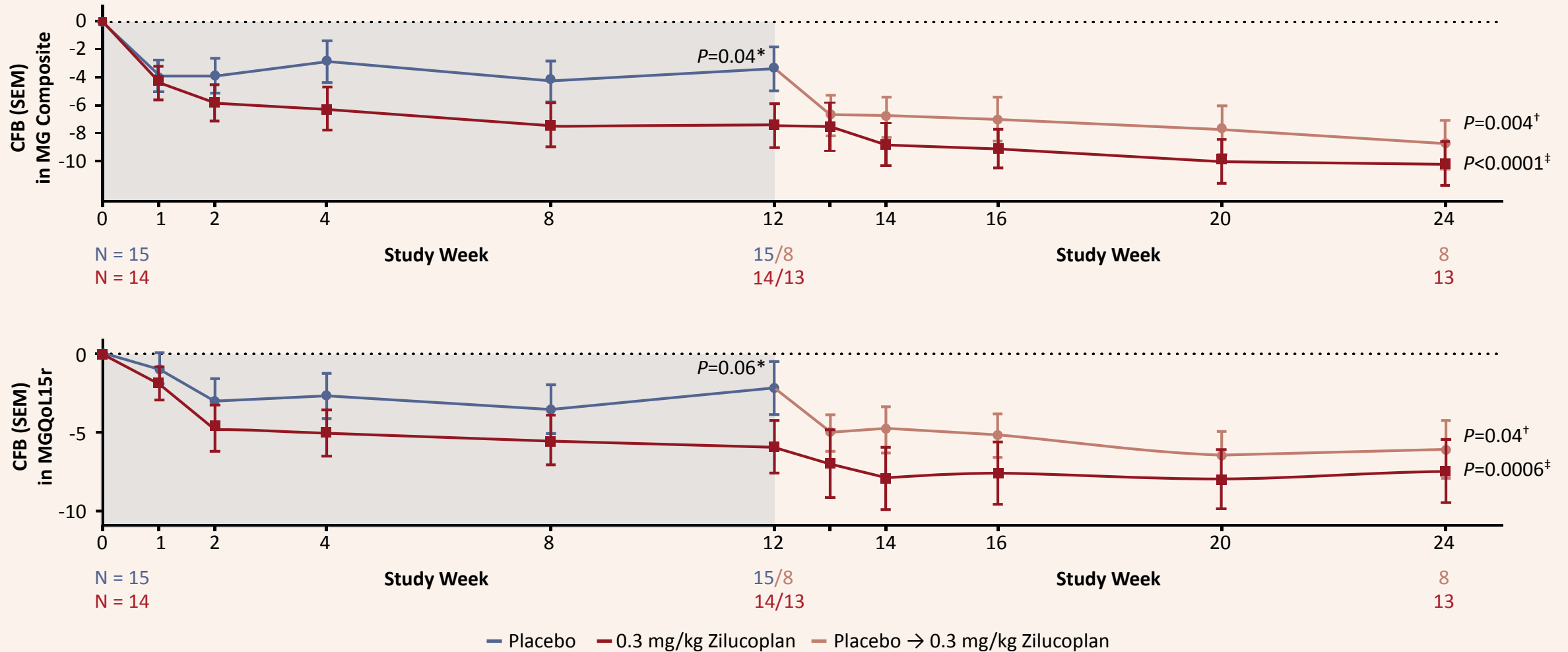
*1-sided analysis of covariance for LS mean change from baseline for 0.3 mg/kg arm vs. placebo; placebo patients re-baselined to zero upon completion of 12-week main study.

†2-sided t test for LS mean change from week 12 to week 24 for placebo patients crossing over to 0.3 mg/kg (n=8).

‡2-sided t test for LS mean change from week 0 to week 24 for 0.3 mg/kg arm.

CFB, change from baseline; LS, least squares; MG-ADL, Myasthenia Gravis Activities of Daily Living; QMG, Quantitative Myasthenia Gravis; SEM, standard error of the mean.

Zilucoplan Achieved Rapid, Clinically Meaningful, Statistically Significant, and Sustained Reductions in MG Composite and MGQoL15r



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CFB, change from baseline; LS, least squares; MGQoL15r, 15-item Myasthenia Gravis Quality-of-Life revised scale; SEM, standard error of the mean.