

Myasthenia Gravis

Clinical Research Highlights

Chilvana Patel, MD, FANA

Clinical Associate Professor;
University of Texas Medical Branch,
Galveston, TX

Ratna Bhavaraju-Sanka, MD, FAAN

Associate Professor
University of Texas Health,
San Antonio, TX

Disclosures



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Learning Objectives:

- Summarize key advances in MG research presented at AAN 2026 and discuss their potential implications for diagnosis, monitoring, and long-term management.
- Communicate clinically relevant updates in MG research to members of the multidisciplinary care team to support coordinated, evidence-based patient care.

Planner/Faculty Educator:

- Dr. Bhavaraju-Sanka discloses the following relevant financial relationships with ineligible companies:
 - Consultant: Amgen, argenx
- Dr. Patel has no relevant financial relationships with any ineligible companies.
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- All relevant financial relationships listed have been mitigated. This activity will discuss off-label uses and investigational agents.

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CLINICAL TRIAL DATA

Approved Indications

New indications for approved therapies

Discussion Points

- **Efgartigimod**
- **Efgartigimod – ocular and juvenile studies**
- **Zilucoplan**
- **Nipocalimab**
- **Inebilizumab**

Efgartigimod PH20 SC Long-term Safety: ADAPT-SC+

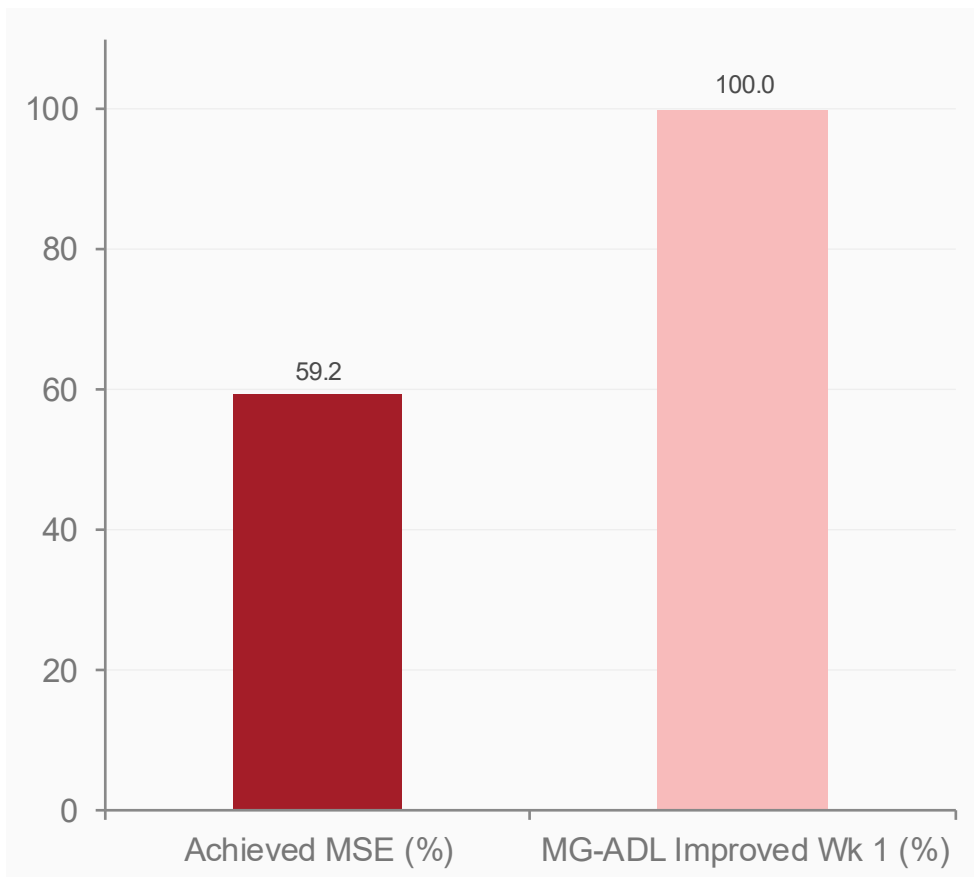
Bhavaraju-Sanka R et al. AAN 2026. Chicago, IL. April 18-22, 2026.

Objective: Evaluate long-term safety and efficacy of SC efgartigimod PH20 in gMG

Method: OLE study; N=180; mean 2.6 years follow-up (459 patient-years)

Conclusion: SC efgartigimod PH20 was well-tolerated long-term; 59.2% of AChR+ patients achieved MSE

Key Efficacy Outcomes (AChR-Ab+ Patients)



Efgartigimod IV Long-term Dosing in gMG: ADAPT NXT Part B

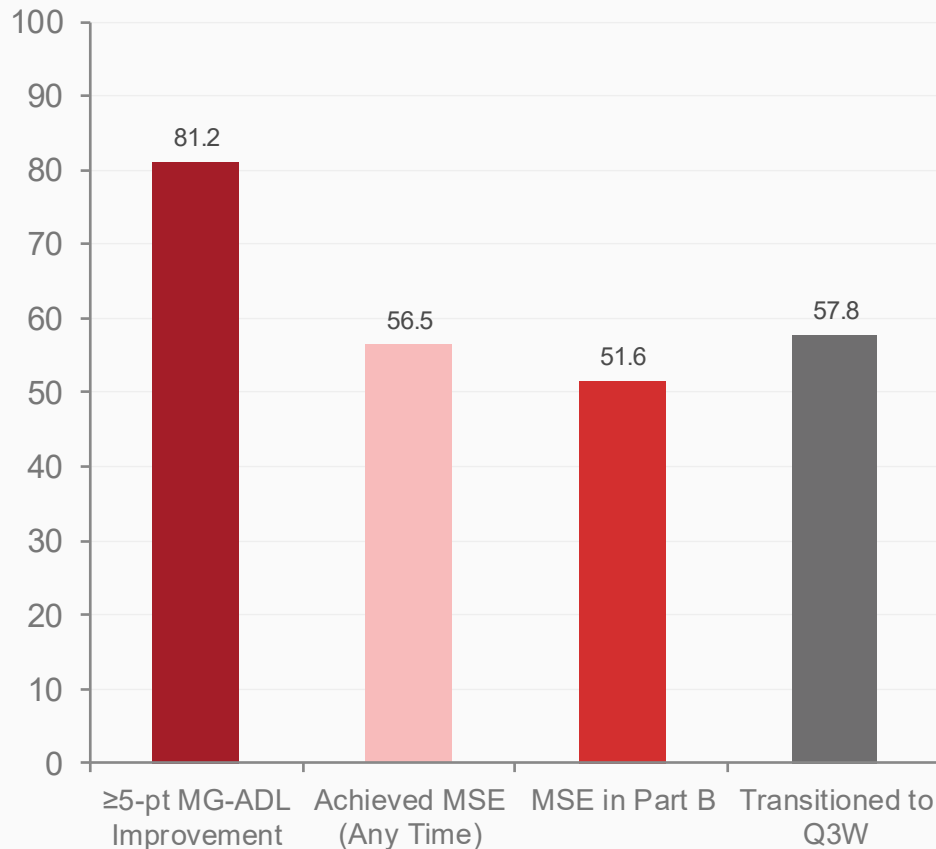
Seth A et al. AAN 2026. Chicago, IL. April 18-22, 2026.

Objective: Investigate long-term efficacy and safety of Q2W and Q3W efgartigimod dosing in gMG

Method: Phase 3b; N=65 continuing into 105-week Part B extension; Q2W with optional Q3W switch

Conclusion: Sustained MG-ADL improvements through Week 126; 56.5% achieved MSE; well-tolerated at all intervals

Clinical Outcomes During Study (% of Participants)



Efgartigimod PH20 SC in Ocular MG:
ADAPT OCULUS Interim

Juel V et al. AAN 2026. Chicago, IL. April 18-22, 2026.

Objective: Evaluate SC efgartigimod PH20 efficacy and safety in adults with ocular myasthenia gravis

Method: Phase 3 RCT; Part A: 1 cycle (4 weekly injections) + 4-week follow-up; primary endpoint MGII ocular PRO at Week 4

Conclusion: First Phase 3 trial in oMG; topline results pending — study ongoing; Part B OLE available

ADAPT OCULUS Study Design

Parameter	Detail
Population	Adults with confirmed oMG, MGII PRO ocular ≥6
Randomization	1:1 efgartigimod PH20 1000 mg vs placebo
Primary Endpoint	MGII PRO ocular score change at Week 4
Extension	Part B: ≤2-year open-label
Status	Topline results to be presented

Efgartigimod IV in AChR-Seronegative gMG (ADAPT SERON)

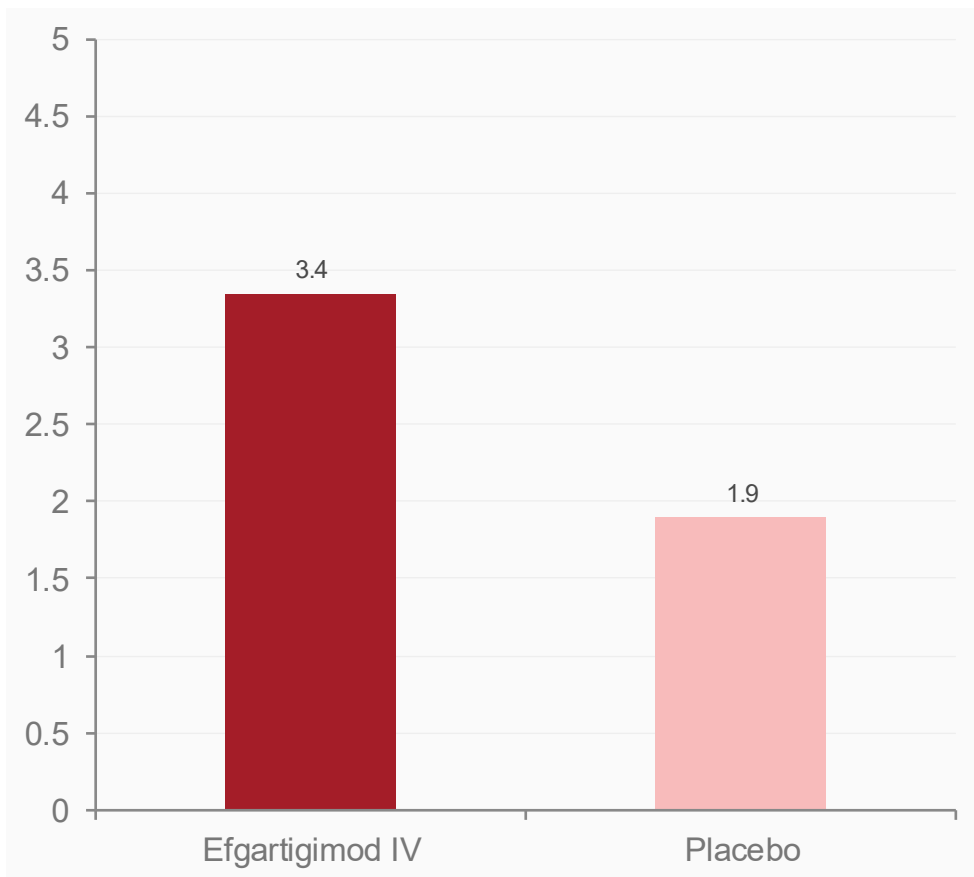
Howard J et al. AAN 2026. Chicago, IL April 18-22, 2026.

Objective: Evaluate efgartigimod IV efficacy and safety in AChR-Ab seronegative gMG

Method: Phase 3 RCT; N=119 (efgartigimod n=58, placebo n=61); primary endpoint MG-ADL at Week 4

Conclusion: Efgartigimod significantly improved MG-ADL vs placebo ($p=0.0068$) in seronegative gMG

LS Mean Change in MG-ADL Score at Week 4



Efgartigimod IV in Juvenile gMG:ADAPT JR Interim

Schwaede A et al. AAN 2026. Chicago, IL. April 18-22, 2026.

Objective: Confirm age-appropriate dosing and assess PK/PD, safety, and efficacy in juvenile gMG

Method: Phase 2/3 trial; adolescent cohort (12–17 y), N=11; Part A (dose-confirm) + Part B (treatment)

Conclusion: Pharmacodynamics, safety, and MG-ADL improvements in adolescents were consistent with adult data. This is an ongoing study.

Adolescent vs Adult Outcomes Comparison

Endpoint	ADAPT JR (Adolescent)	Adult ADAPT Studies
IgG Reduction	Similar	Well established
AChR-Ab Reduction	Similar	Well established
MG-ADL Improvement	Consistent	Consistent
Safety Profile	Consistent	Consistent

Zilucoplan: 120-Week MSE Durability in gMG (RAISE-XT)

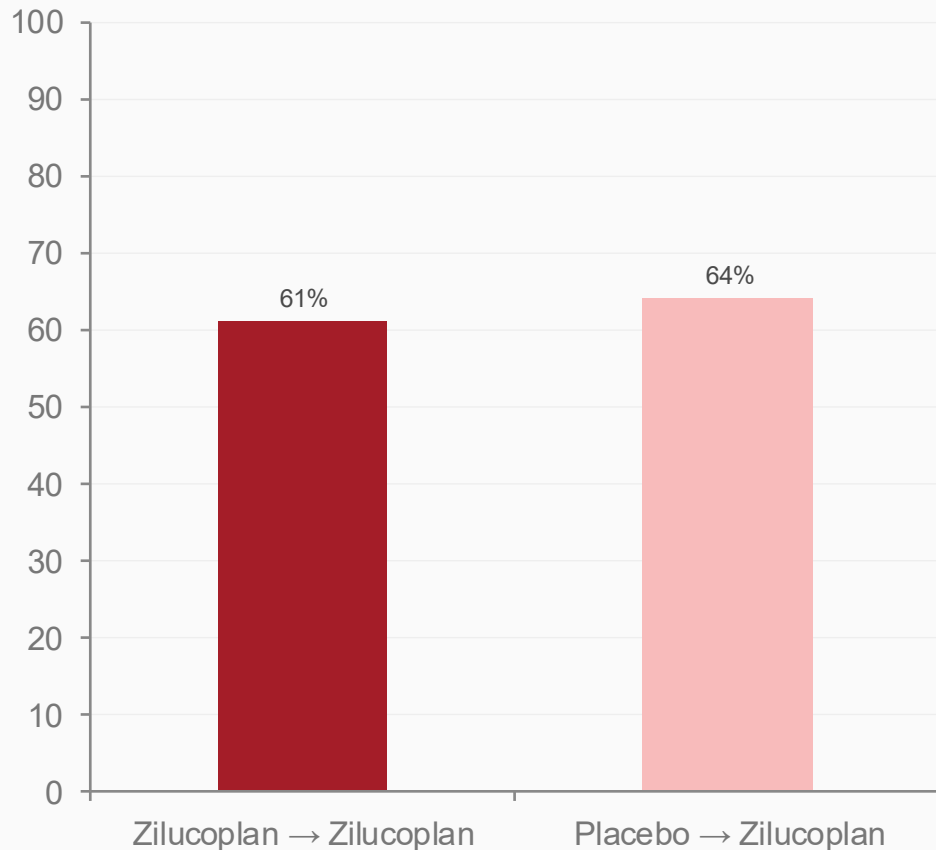
Howard Jr JF et al. AAN 2026. Chicago, IL. April 18-22, 2026.

Objective: Assess durability of minimal symptom expression (MSE) with zilucoplan in gMG over 120 weeks

Method: Post hoc analysis; N=183 gMG patients from RAISE-XT open-label extension

Conclusion: Zilucoplan demonstrated sustained MSE response up to 120 weeks of treatment

Patients Achieving MSE (Cumulative %)



Nipocalimab: Sustained vs Transient MSE and QoL (Vivacity-MG3)

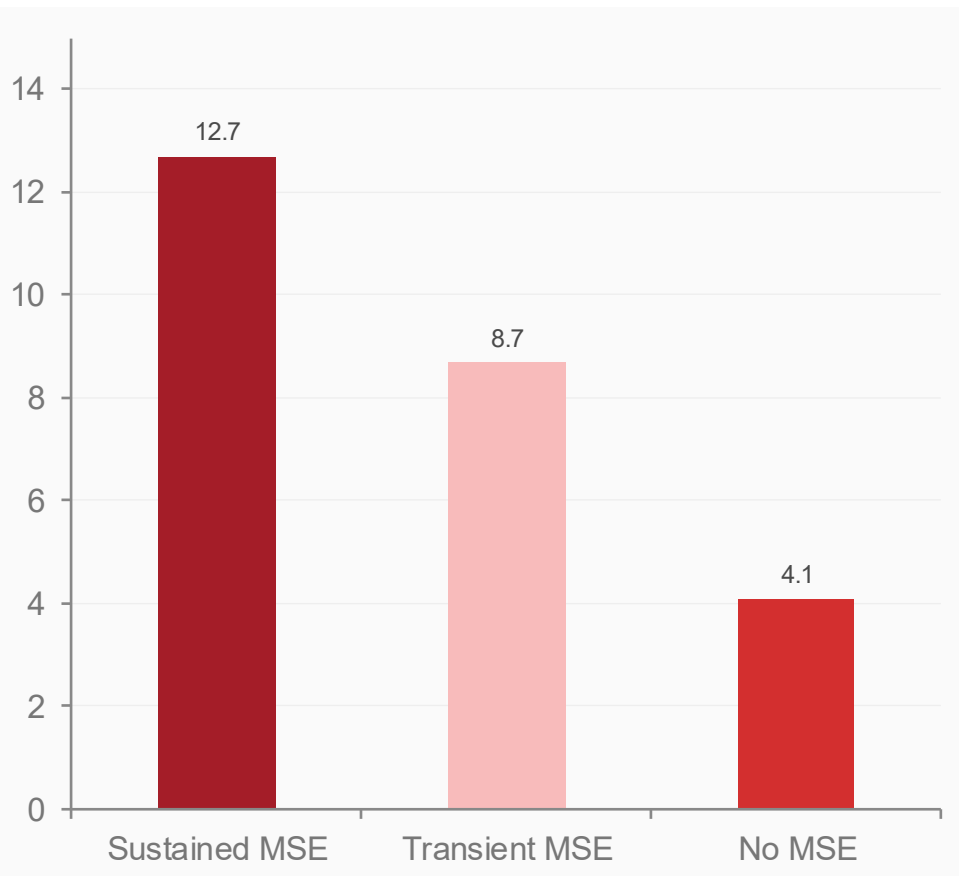
Cortés Vicente E et al. AAN 2026. Chicago, IL. April 18-22, 2026.

Objective: Compare QoL outcomes in patients with sustained vs transient MSE over 24 weeks

Method: Phase 3 post hoc; N=153 (nipocalimab+SOC n=77, placebo+SOC n=76)

Conclusion: Nipocalimab patients 4× more likely to achieve sustained MSE; sustained MSE yielded greater MG-QoL-15r benefit

MG-QoL-15r Change from Baseline at Week 24 (LSM)



Inebilizumab (Anti-CD 19) in AChR+ gMG: MINT Week 52

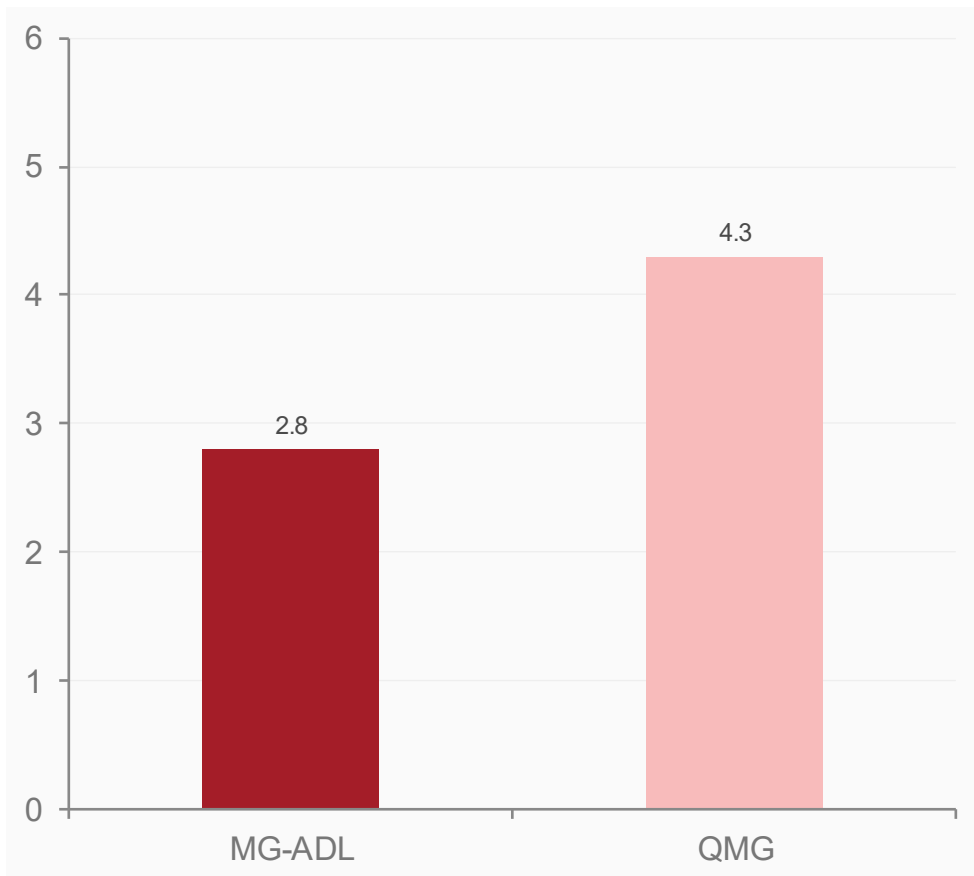
Clarkson K et al. AAN 2026. Chicago, IL April 18-22, 2026.

Objective: Evaluate 52-week efficacy and pharmacodynamics of inebilizumab in AChR+ gMG

Method: Phase 3 RCT (MINT); N=190 AChR+ (inebilizumab n=95, placebo n=95); 52-week RCP

Conclusion: Inebilizumab produced sustained MG-ADL and QMG improvement through Week 52 with 93% B-cell depletion

Adjusted Score Difference vs Placebo at Week 52



Evolving Therapies

- **CART-cell therapies**
- **Gefurulimab**
- **Claseprubart**
- **Cemdisiran, Pozelimab**

Rese-cel CAR T-Cell Therapy in gMG
(RESET-MG)

Habib A et al. AAN 2026. Chicago, IL. April 18-22, 2026.

Objective: Evaluate safety and efficacy of rese-cel (CD19 CAR T) in AChR+ and AChR– gMG

Method: Phase I/2 trial; 4 patients (2/cohort) with ≥1 month follow-up as of Sept 2025

Conclusion: Rese-cel well-tolerated; produced immune reset and MG-ADL improvements off all gMG therapies

MG-ADL Outcomes at Follow-up

Patient	Baseline MG-ADL	Follow-up MG-ADL	Weeks Post-Infusion	Off gMG Meds
Patient 1	17	0 (MSE)	20 wks	Yes
Patient 2	14	7	8 wks	Yes
Patient 3	Patient not evaluable			
Patient 4	Insufficient follow-up			

KYV-101 CD19 CAR T-Cell Therapy in gMG (KYSA-6)

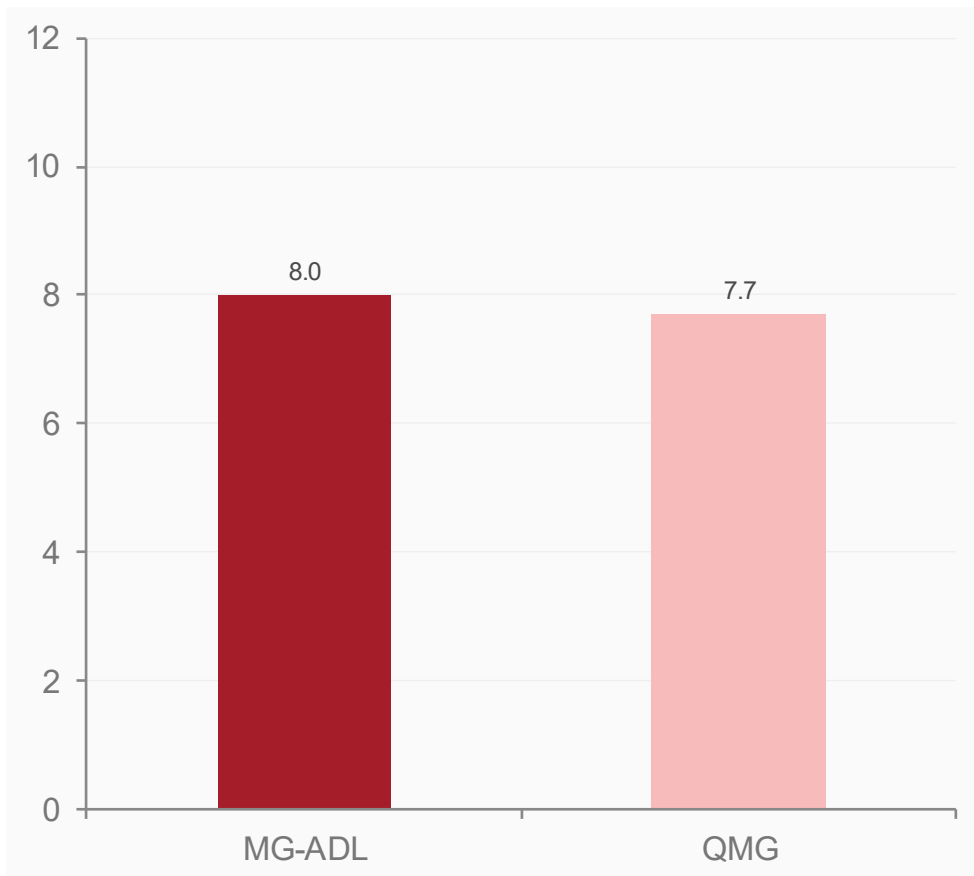
Muppidi S et al. AAN 2026. Chicago, IL. April 18-22, 2026.

Objective: Evaluate clinical outcomes of KYV-101 (CD19 CAR T) in patients with gMG

Method: Phase 2 open-label single-arm study; N=6 dosed; mean age 45.5y; MG-ADL 11.2 at baseline

Conclusion: 100% ≥ 3 -pt MG-ADL improvement; 50% achieved MSE; 5/6 patients became immunosuppressant-free. Trial is ongoing.

Mean Score Reduction from Baseline at 24 Weeks



Gefurulimab in AChR+ gMG: PREVAIL Phase 3

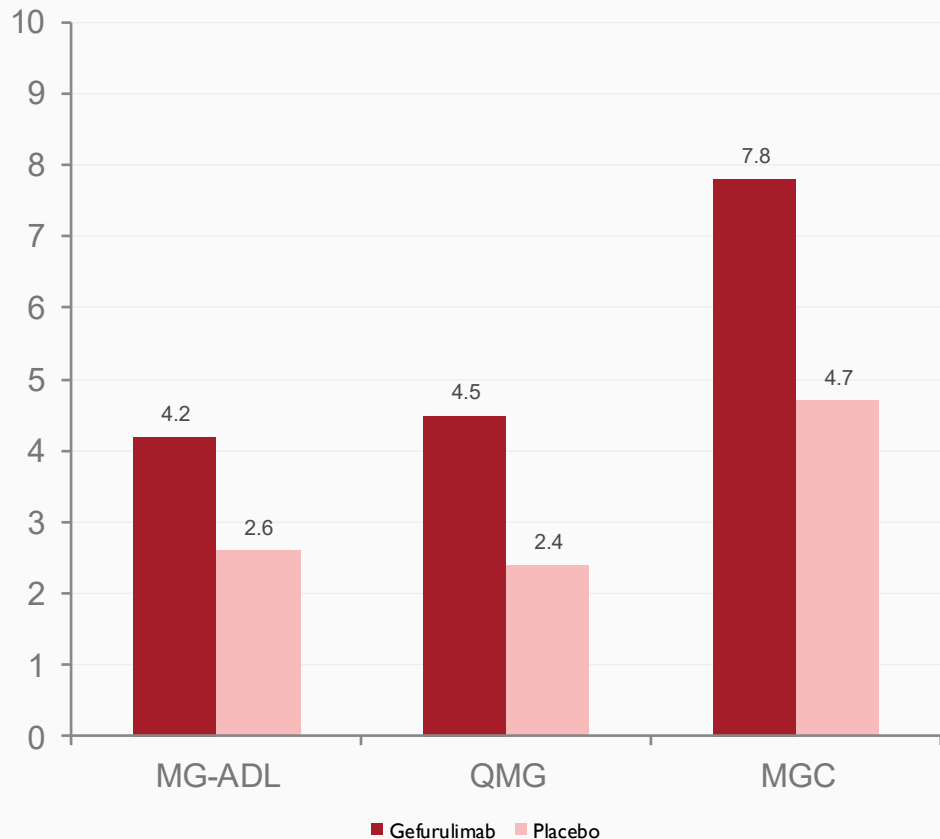
Gwathmey K et al. AAN 2026. Chicago, IL April 18-22, 2026.

Objective: Evaluate efficacy and safety of subcutaneous gefurulimab (C5 nanobody) in AChR+ gMG

Method: RCT; N=260 (gefurulimab n=131, placebo n=129); primary endpoint MG-ADL change at Week 26

Conclusion: All endpoints met; rapid, sustained improvement in MG-ADL, QMG, and MGC vs placebo (all $p < 0.0001$)

LS Mean Change from Baseline at Week 26



Claseprubart (CIs Inhibitor) in gMG: MaGic Phase 2

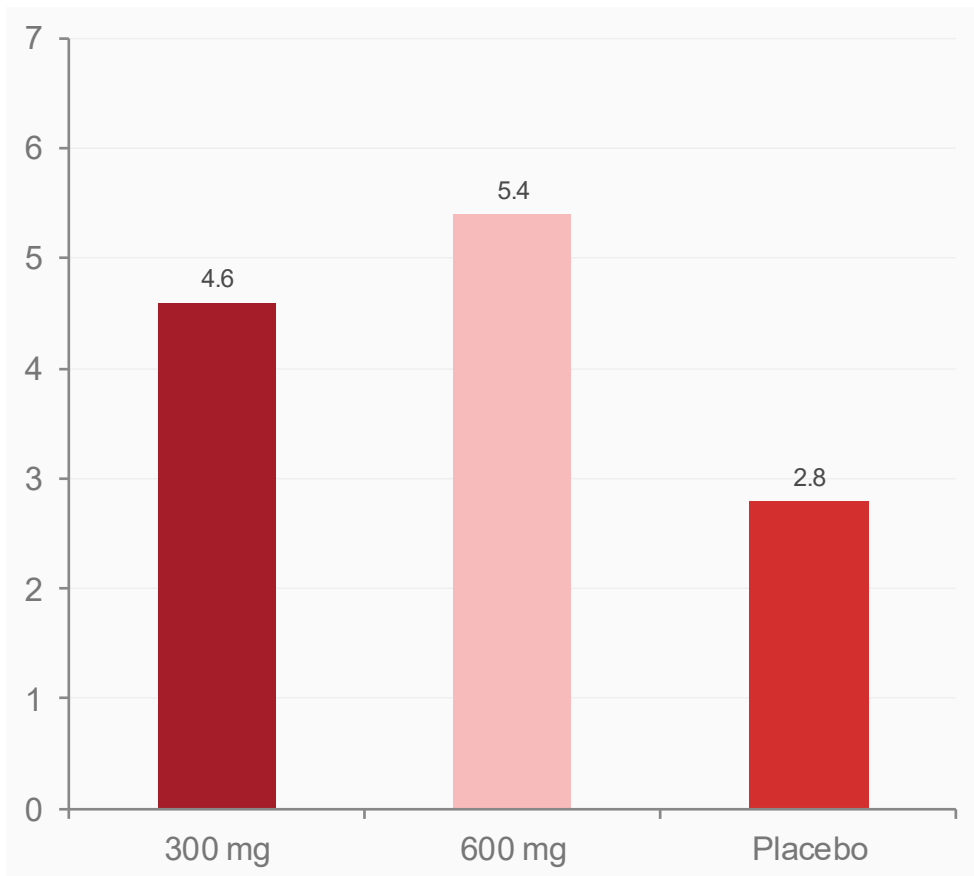
Peric S et al. AAN 2026. Chicago, IL April 18-22, 2026.

Objective: Assess safety and efficacy of claseprubart (aCIs inhibitor) in AChR Ab+ gMG

Method: RCT; N=65 randomized 1:1:1 (300 mg, 600 mg, placebo Q2W) for 13 weeks

Conclusion: Both doses improved MG-ADL significantly vs placebo from Week 1; Phase 3 planned

MG-ADL Improvement from Baseline at Week 13



Cemdisiran $\pm$ Pozelimab in gMG: NIMBLE Phase 3

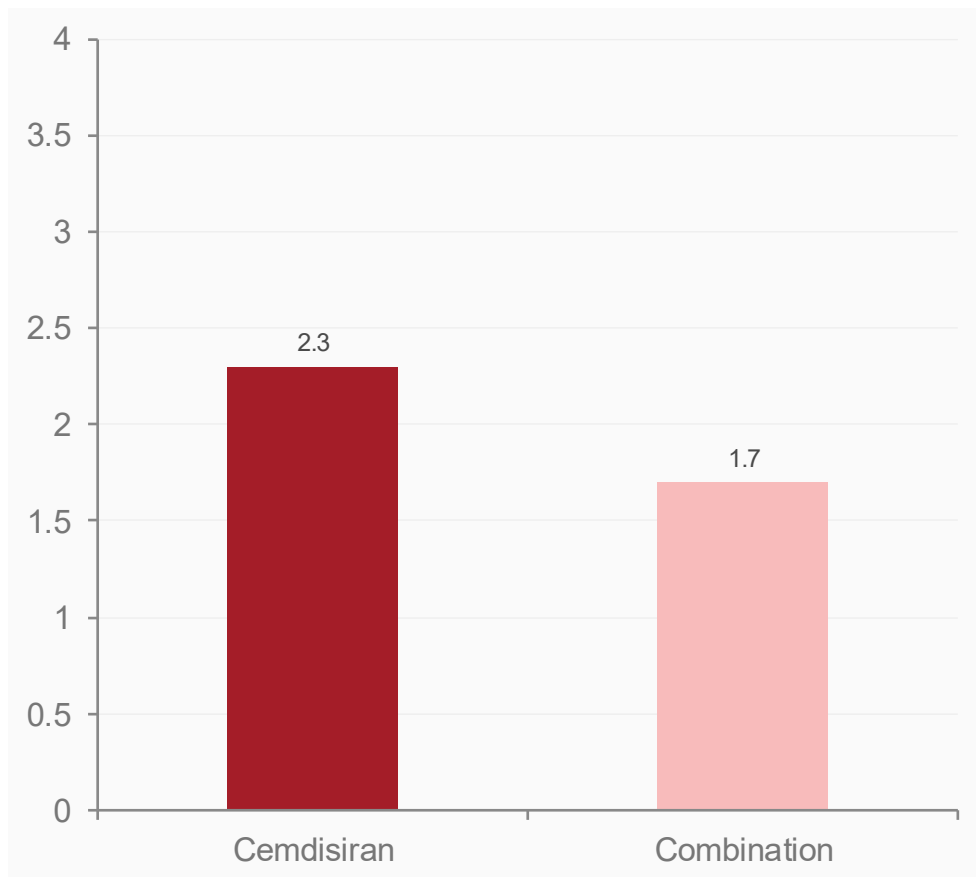
Vu T et al. AAN 2026. Chicago, IL April 18-22, 2026.

Objective: Compare subcutaneous cemdisiran (siRNA) $\pm$ pozelimab vs placebo in AChR+/LRP4+ gMG

Method: 4-arm RCT; N=284 randomized; primary endpoint MG-ADL change at Week 24

Conclusion: Cemdisiran monotherapy met primary/secondary endpoints; pozelimab monotherapy did not improve vs placebo

Placebo-Adjusted MG-ADL Change at Week 24



META-ANALYSES AND REAL WORLD DATA

Retrospective cohorts & registry studies

Predictors of Early Mortality in ICI-Induced Myositis/Myasthenia

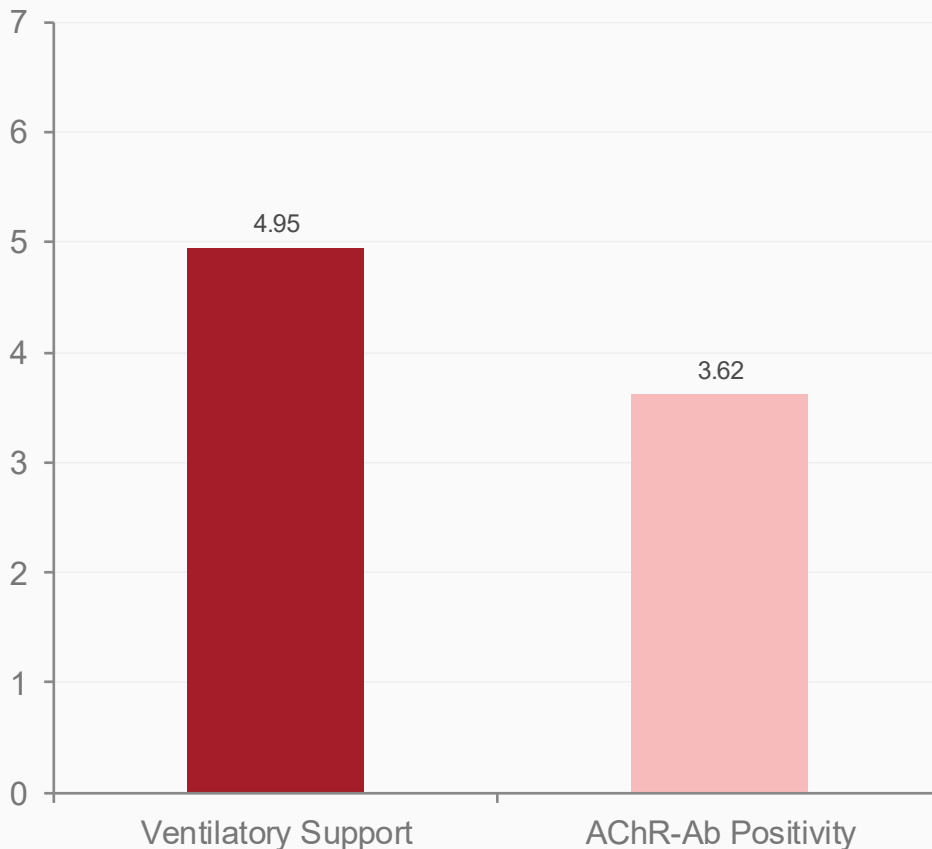
Velasco Fargas R et al. AAN 2026. Chicago, IL April 18-22, 2026.

Objective: Identify predictors of 30-day mortality in immune checkpoint inhibitor–related myositis/myasthenia

Method: Nationwide retrospective cohort; N=153 patients from 36 hospitals in Spain through March 2025

Conclusion: Ventilatory support and AChR-Ab positivity independently predicted early mortality; TnT correlated with MGFA severity

Mortality Predictors: OR (Multivariable Analysis)



Network Meta-analysis: Monoclonal Antibodies in Myasthenia Gravis

Waseem H et al. AAN 2026. Chicago, IL. April 18-22, 2026.

Objective: Compare safety and effectiveness of various monoclonal antibody doses in MG treatment

Method: Network meta-analysis of 18 RCTs; databases searched through June 2025

Conclusion: Rozanolixizumab 10 mg/kg ranked best for MG-ADL; batoclimab 680 mg best for QMG; eculizumab best for QoL

Top-Ranked Agents by Outcome (Network Meta-Analysis)

Outcome	Top-Ranked Agent	P-Score
MG-ADL Score	Rozanolixizumab 10 mg/kg	0.77
QMG Score	Batodimab 680 mg	0.96
MGC Score	Batodimab 340 mg	0.82
MG-QoL 15r	Eculizumab	0.93
Adverse Events	Belimumab 10 mg/kg	0.87

CLINICAL PEARLS

Key Takeaways from AAN 2026 Myasthenia Gravis Research

Complement Inhibitors

Zilucoplan

- Approved therapy showing long term safety and efficacy
- In Development - Newer therapies (Claseprubart, Gefurulimab, Cemdisiran/Pozelimab) showing promise

FcRn Inhibitors

Efgartigimod

- Continued long term safety and efficacy
- New indication for Seronegative
- Promising data in Ocular MG
- Ongoing study in Juvenile MG

Nipocalimab (Vivacity) - continuing data on safety and efficacy

CD19-Targeted B-Cell Depletion

- Inebilizumab (MINT, AChR+) – continued safety and efficacy data

CAR-T therapies

Single dose after lymphodepletion shows safety and efficacy in refractory MG in Phase 1/2 (Rese-cel CAR-T) and Phase 2 (KYV-101) trials

Network Meta-Analysis Rankings

- Different molecules (Efgartigimod, Rozanolixizumab, Batoclimab) showing better efficacy in different end points like MGADL, QMG, MG-QOL
- No head-to-head comparison for different therapies

REFLECTIVE QUESTION

How will the data discussed during this activity impact your clinical decision-making?