Results From the ADAPT Jr Study Investigating Intravenous Efgartigimod in Juvenile Generalized Myasthenia Gravis

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PRESENTED AT THE MYASTHENIA GRAVIS FOUNDATION OF AMERICA (MGFA) SCIENTIFIC SESSION
AT THE AMERICAN ASSOCIATION OF NEUROMUSCULAR & ELECTRODIAGNOSTIC MEDICINE (AANEM) ANNUAL MEETING;
OCTOBER 29, 2025; SAN FRANCISCO, CA, USA

Financial Disclosures

The ADAPT JR trial was funded by argenx

ANS received research support from Alexion, argenx, and NS pharma; served on medical advisory boards for Johnson & Johnson and Sarepta; and participated in speaking engagements for Biogen

Introduction and Objective

- Previous Phase 3 trials (ADAPT/ADAPT+) have demonstrated that IV efgartigimod is efficacious and well tolerated in adults with gMG[1,2]
- The incidence of juvenile patients with gMG (1-5 cases per million person-years) is considerably lower than adult patients with gMG (~4-30 cases per million person-years)[3]
- The course of gMG for juvenile patients may be associated with exacerbations, such as myasthenic crisis, and standard treatments can be associated with adverse effects[3,4]
- There remains an unmet need for safe and effective treatments in this juvenile population[4]
- **Here we present interim results from an ongoing Phase 2/3 clinical trial assessing efgartigimod IV in juvenile participants with AChR-Ab+ gMG (ADAPT JR; NCT04833894) that aims to confirm an age-adjusted optimum dose of efgartigimod IV in a cohort of adolescent participants (aged 12-17 years)**

AChR-Ab+, acetylcholine receptor antibody positive; gMG, generalized myasthenia gravis; IV, intravenous.

1. Howard JF Jr, et al. *Lancet Neurol*. 2021;20(7):526-536. **2.** Howard JF Jr, et al. *Front Neurol*. 2024;14:1284444. **3.** Dresser L, et al. *J Clin Med.* 2021;10(11):2235. **4.** O'Connell K, et al. *Front Neurol.* 2020;11:734.

Efgartigimod Blocks FcRn and Reduces IgG Levels

- Efgartigimod is an IgG1 antibody Fc fragment that has been engineered for increased affinity to FcRn compared to endogenous IgG and is uniquely composed of the only part of the IgG antibody that normally binds FcRn[1]
- Efgartigimod selectively reduces IgG by blocking FcRn-mediated IgG recycling without impacting antibody production or other parts of the immune system, and does not decrease albumin[1-3]

1. Efgartigimod and IgG are internalized[1,4]
2. Efgartigimod competes with endogenous IgG for binding to FcRn[1]
3. Unbound IgG enters the degradation pathway[1,4]
4. Efgartigimod and fewer IgGs are recycled back into the bloodstream[1]

Fc, fragment crystallizable (region); FcRn, neonatal Fc receptor; IgG, immunoglobulin G.

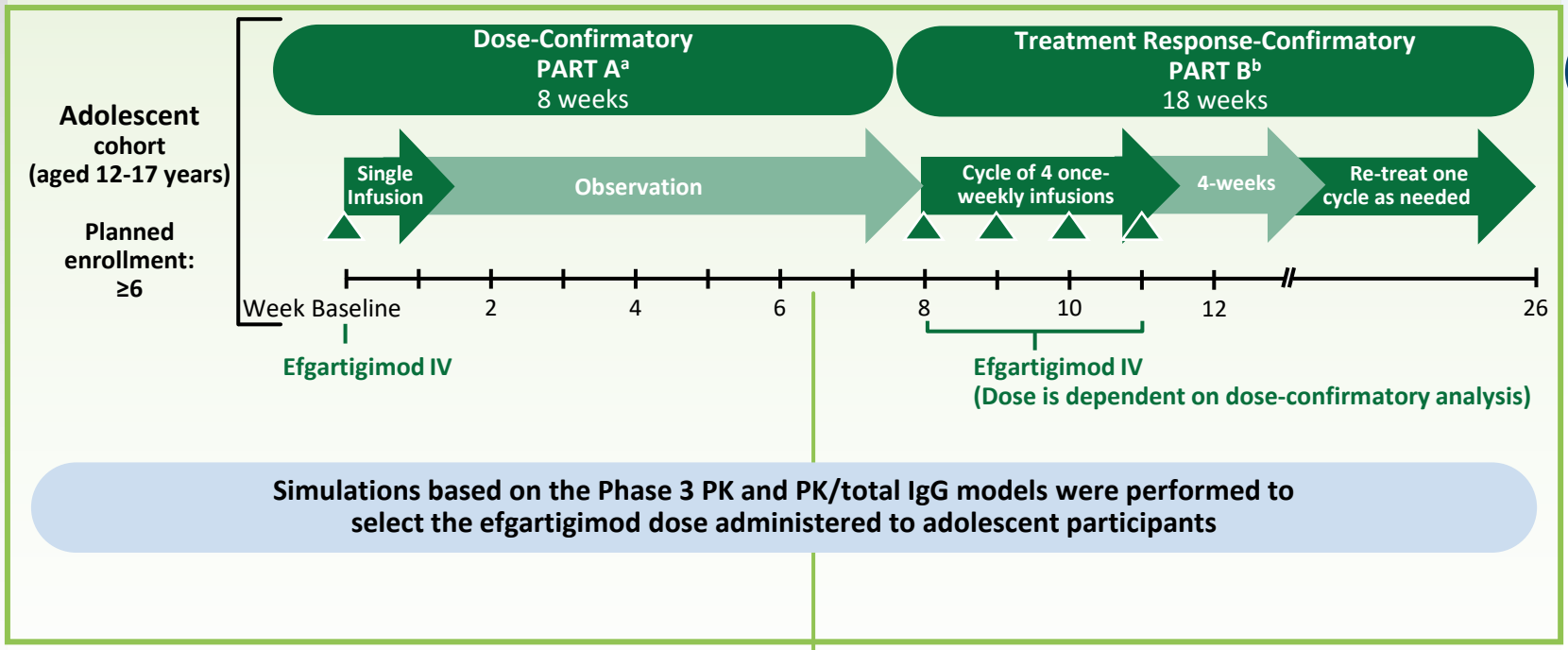
1. Ulrichts P, et al. *J Clin Invest.* 2018(10);128:4372-4386. **2.** Howard JF Jr, et al. *Lancet Neurol.* 2021;20(7):526-536. **3.** Guptill JT, et al. *Autoimmunity.* 2022;55(8):620-631. **4.** Sesarman A, et al. *Cell Mol Life Sci.* 2010;67(15):2533-2550.

ADAPT JR Study Design

SCREENING
+/-2 weeks

Key Inclusion Criteria

- Male or female participants aged 2-17 years
- Confirmed diagnosis of gMG
- MGFA Class II, III, or IVa
- AChR-Ab+
- Unsatisfactory response to immunosuppressants, steroids, or AChEIs
- Stable concomitant gMG therapy
- Total IgG level above the lower limit of normal



- After interim PK/PD analyses of adolescent data from Parts A and B, the age-appropriate dose for the child cohort is selected and enrollment of the child cohort (n=≥6, aged 2-11 years) begins
- Enrollment for the child cohort is ongoing

ADAPT JR+
(ARGX-113-2008)

- Adolescent and child cohorts may rollover into the long-term, single-arm, open-label, multicenter, extension study

AChEI, acetylcholinesterase inhibitor; AChR-Ab+, acetylcholine receptor antibody positive; DSMB, Data Safety Monitoring Board; gMG, generalized myasthenia gravis; IgG, immunoglobulin G; IV, intravenous; MGFA, Myasthenia Gravis Foundation of America; PD, pharmacodynamics; PK, pharmacokinetics. [a]Confirmation of age-appropriate dose of efgartigimod (predicted based on modeling) based on PK and PD. Participants who discontinue early from Part A may be replaced upon consultation with the sponsor. [b]Participants who complete Part A but do not roll over to Part B may be replaced by new participants who can start immediately in Part B.

Baseline Demographics and Clinical Characteristics of Adolescent Participants

Demographics

Adolescent Cohort (aged 12-17 years)	Efgartigimod IV[a] (N=11)
Age, years	
Mean (SD)	14.8 (1.6)
Median (min, max)	15.0 (12.0, 17.0)
Female sex, n (%)	11 (100)
Race, n (%)	
Black or African American	1 (9.1)
White	8 (72.7)
Other	2 (18.2)
Ethnicity, n (%)	
Hispanic or Latino	2 (18.2)
Not Hispanic or Latino	9 (81.8)
Weight, kg	
Mean (SD)	69.8 (16.8)
Median (min, max)	63.60 (47.3, 100.0)

Clinical Characteristics

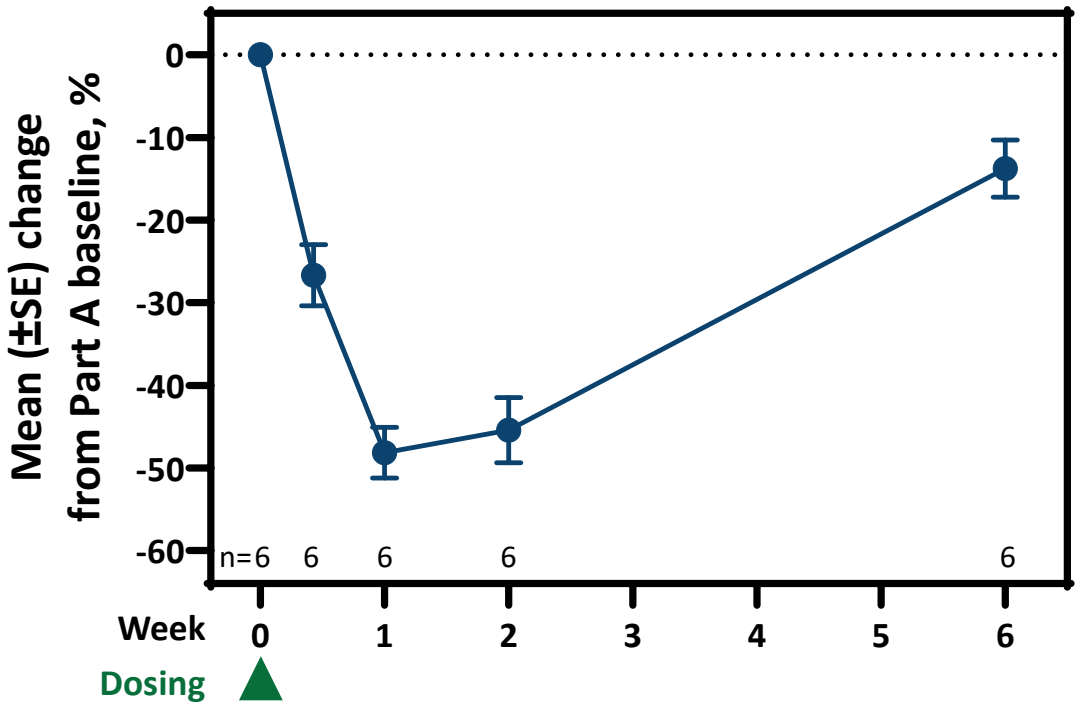
Adolescent Cohort (aged 12-17 years)	Efgartigimod IV[a] (N=11)
Time since diagnosis, years	
Mean (SD)	4.6 (3.2)
Median (min, max)	3.8 (0.7, 11.5)
MG-ADL total score at baseline	
Mean (SD)	6.3 (3.2)
Median (min, max)	7.0 (2.0, 13.0)

MG-ADL, Myasthenia Gravis Activities of Daily Living; SD, standard deviation.
[a]Safety analysis B and safety analysis set overall.

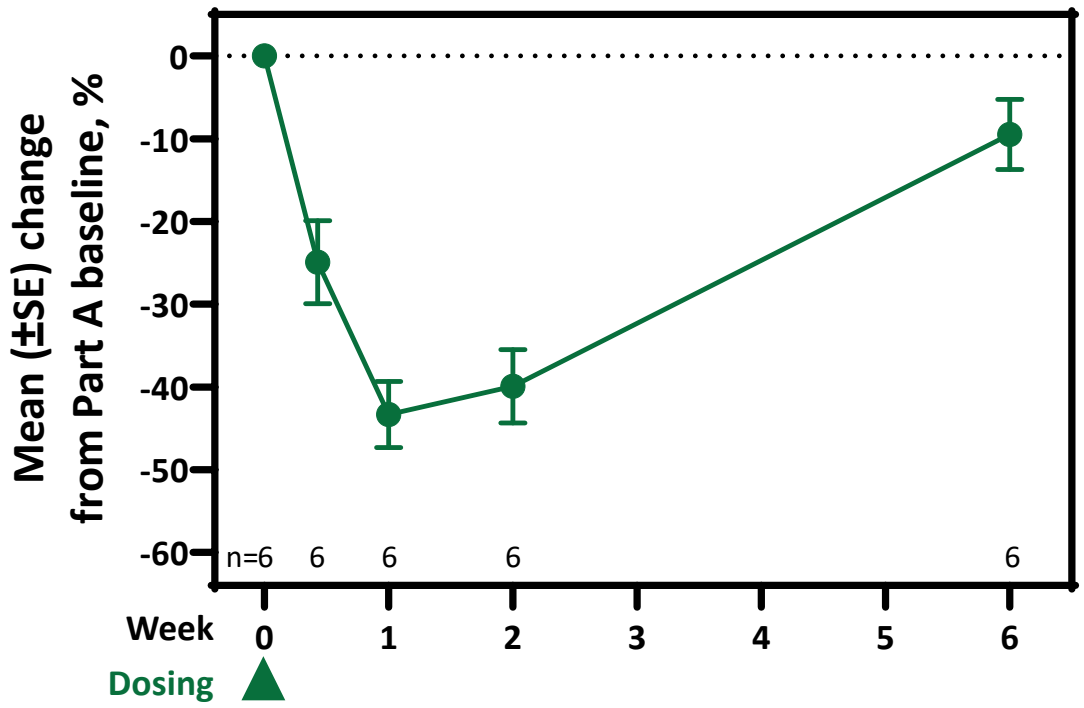
Part A

A Single Infusion of Efgartigimod Resulted in Decreases of Both Total IgG and AChR-Ab Levels in Adolescent Participants

Percent Change in Total IgG Levels



Percent Change in AChR-Ab Levels

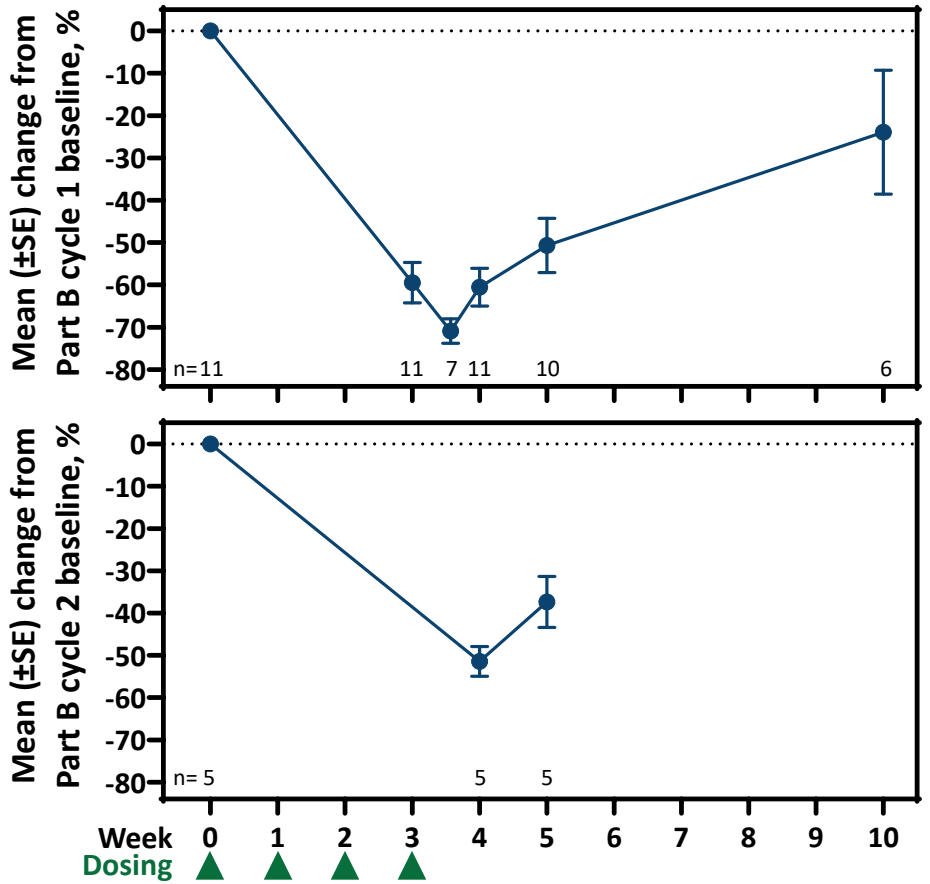


AChR-Ab, acetylcholine receptor antibody, IgG, immunoglobulin G.

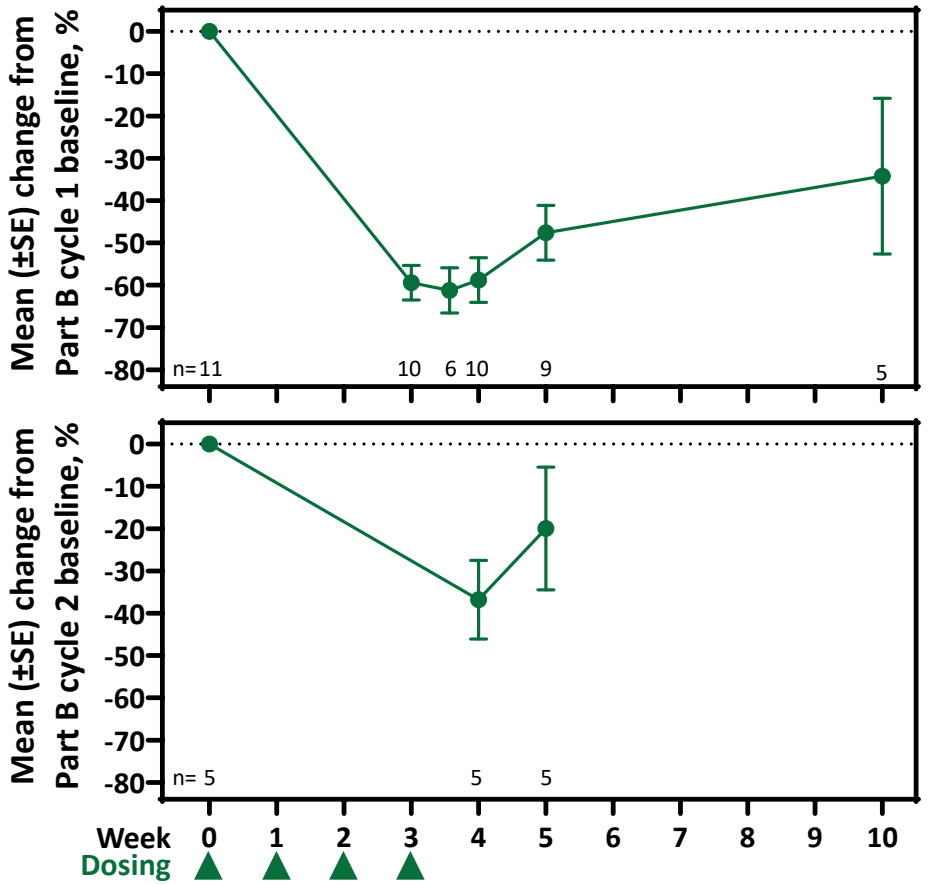
Part B

Efgartigimod Treatment Resulted in Decreases of Both Total IgG and AChR-Ab Levels in Adolescent Participants

Percent Change in Total IgG Levels



Percent Change in AChR-Ab Levels



- Reductions in total IgG and AChR-Ab levels were similar to reductions observed with efgartigimod treatment in phase 3 adult studies

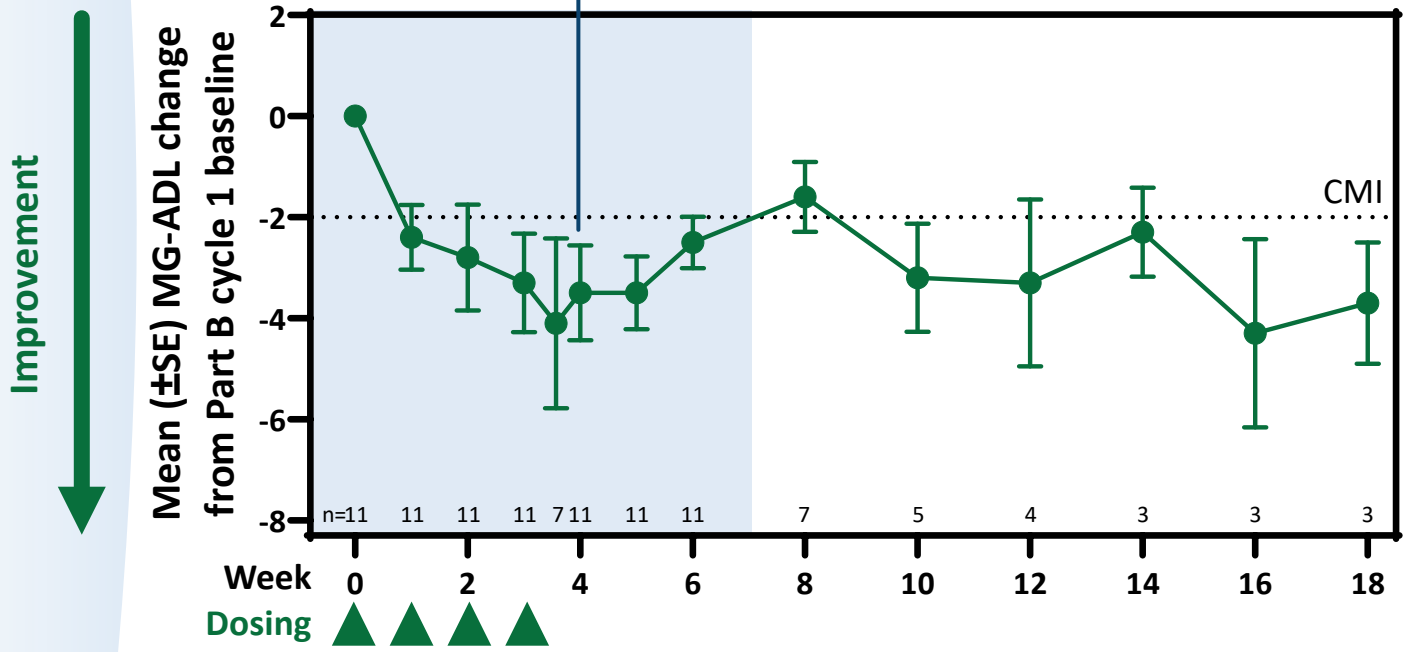
AChR-Ab, acetylcholine receptor antibody, IgG, immunoglobulin G.

Part B

Efgartigimod Treatment Resulted in MG-ADL Score Decreases in Adolescent Participants

Cycle 1

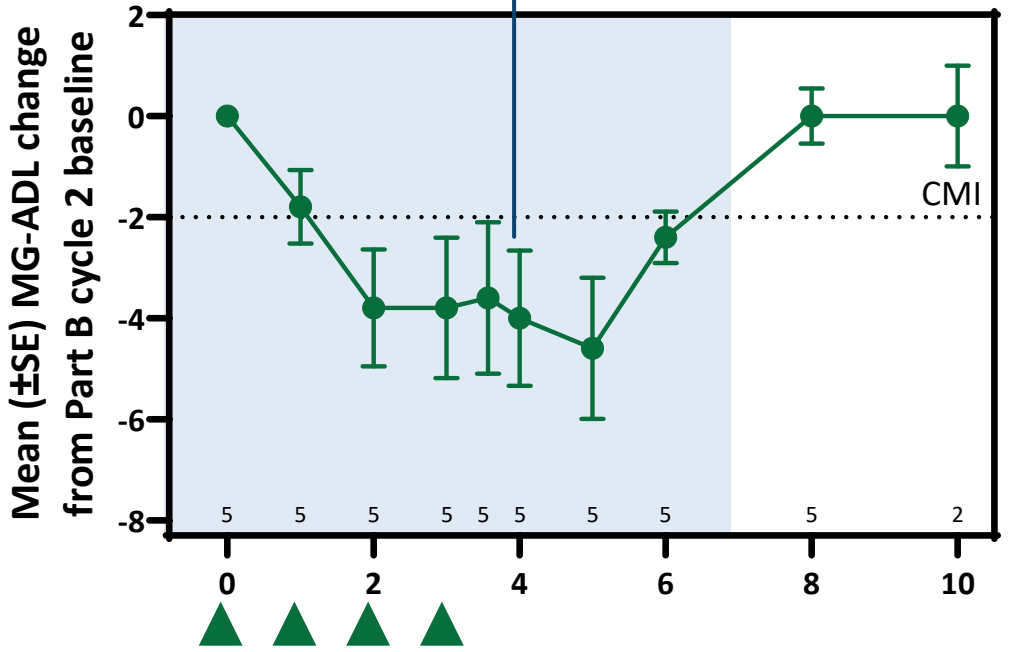
Median (min, max) MG-ADL score change at Week 4: **-2 (-11, -1)**



- MG-ADL score improvements were sustained for several weeks
- MSE (MG-ADL score of 0 or 1) was reached by 8 of 11 (72.7%) participants

Cycle 2

Median (min, max) MG-ADL score change at Week 4: **-3 (-9, -1)**



- MSE (MG-ADL score of 0 or 1) was reached by 4 of 5 (80.0%) participants

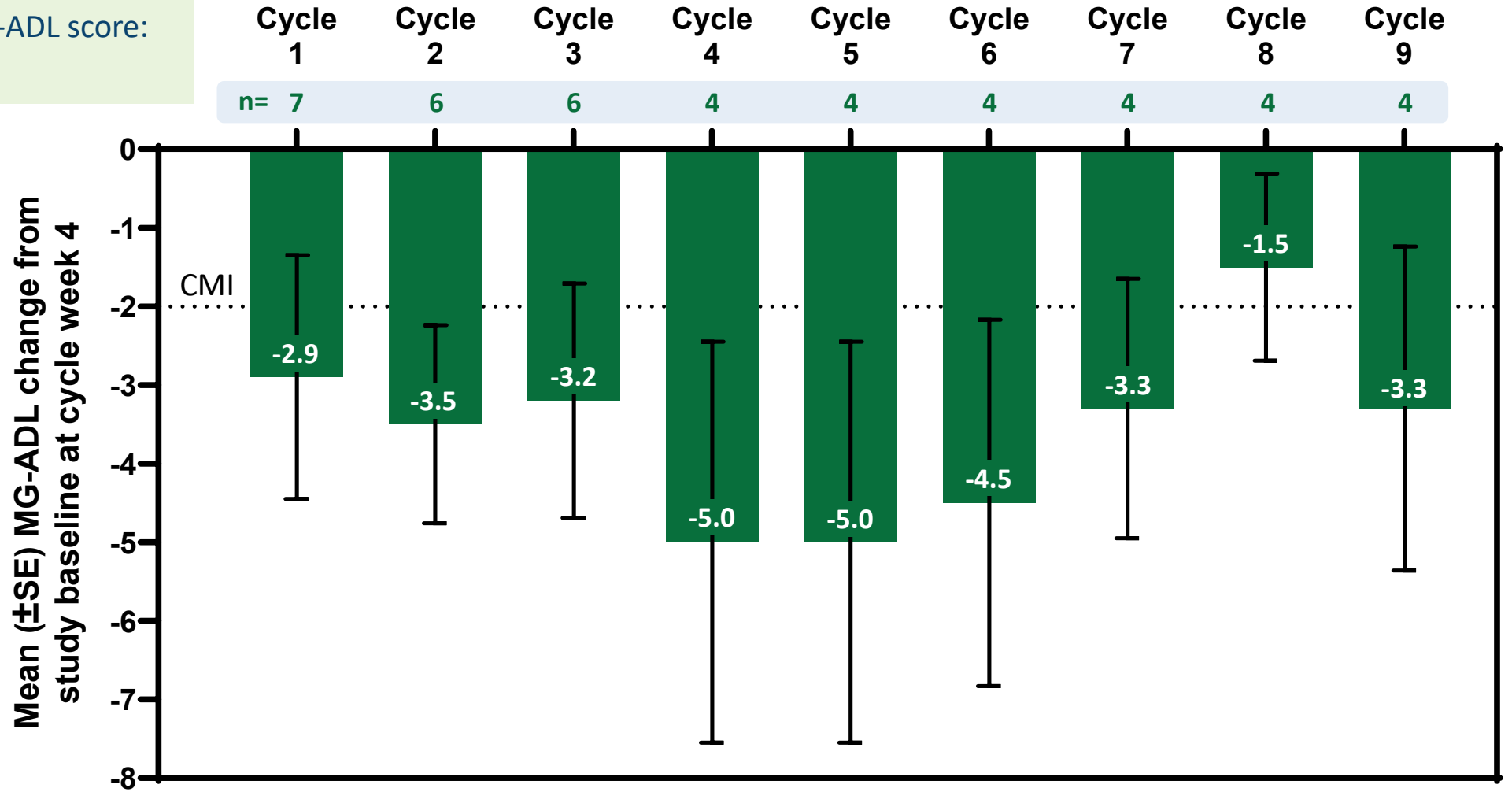
CMI, clinically meaningful improvement; MG-ADL, Myasthenia Gravis Activities of Daily Living; MSE, minimal symptom expression.
Shaded area (baseline to week 6) has a fixed # of participants because they were not allowed to move to next treatment cycle during this period. Four patients had missing data on Day 25 of Cycle 1.

ADAPT JR+

Multiple Efgartigimod Treatment Cycles in the Ongoing OLE Resulted in Repeatable MG-ADL Score Decreases in Adolescent Participants

Study baseline mean (SE) MG-ADL score: **4.9 (1.4)**

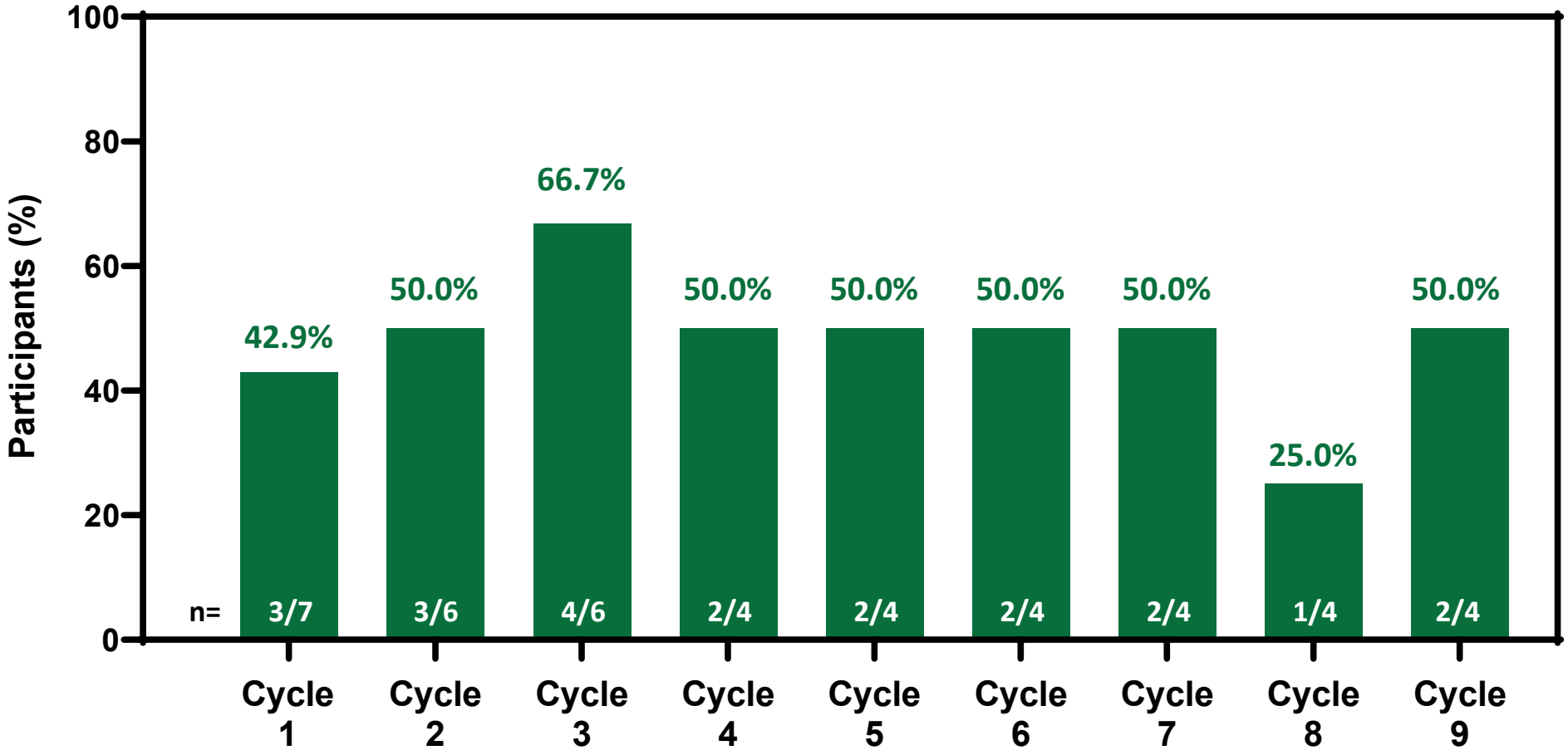
MG-ADL Change From Study Baseline at Week 4 of Each Cycle



CMI, clinically meaningful improvement; MG-ADL, Myasthenia Gravis Activities of Daily Living; OLE, open-label extension.

ADAPT JR+

Minimal Symptom Expression (MG-ADL score 0 or 1) Was Achieved by Many of the Adolescent Participants in the Ongoing OLE



- MSE was observed across up to 9 cycles of ADAPT JR+, with as many as 66.7% reaching MSE in Cycle 3

CMI, clinically meaningful improvement; MG-ADL, Myasthenia Gravis Activities of Daily Living; MSE, minimal symptom expression; OLE, open-label extension.

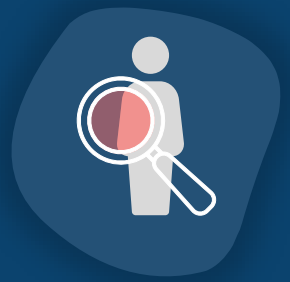
Parts A+B

Efgartigimod Treatment Was Well Tolerated in Adolescent Participants

Adolescent Cohort (aged 12-17 years)	Efgartigimod IV (N=11)	
	n (%)	ER[a]
≥1 TEAE	11 (100.0)	16.3
≥1 Serious TEAE[b]	2 (18.2)	0.7
≥1 Fatal TEAE	0	-
≥1 Grade ≥3 TEAE[c]	3 (27.3)	1.2
≥1 Infection event[d]	4 (36.4)	1.9
≥1 IRR event	1 (9.1)	0.2
≥1 TEAE leading to efgartigimod discontinuation	0	-
Most common adverse events[e]		
Headache	7 (63.6)	2.1
Oropharyngeal pain	3 (27.3)	0.7
Lymphopenia	2 (18.2)	0.7
Nausea	2 (18.2)	0.7
Diarrhea	2 (18.2)	0.5
Nasal congestion	2 (18.2)	0.5
Rhinorrhea	2 (18.2)	0.5

ER, event rate; IRR, infusion-related reaction; MG, myasthenia gravis; PYFU, participant years of follow up; TEAE, treatment-emergent adverse event.
[a]ER was calculated as the number of events/PYFU. [b]Serious TEAEs included one event each of general physical health deterioration, fibrin D-dimer increase, and MG worsening. [c]Grade ≥3 TEAEs included one event each of general physical health deterioration, vascular device occlusion, fibrin D-dimer increase, hypertriglyceridemia, and MG worsening. [d]Infection events included one event each of asymptomatic bacteriuria, COVID-19, nail infection, pulpitis dental, and two events each of upper respiratory tract infection and urinary tract infection. [e]Reported in ≥2 participants.

KEY TAKEAWAYS



In adolescent participants, treatment with efgartigimod was well tolerated and resulted in decreased levels of both total IgG and AChR-Abs

In both the ADAPT JR and the ADAPT JR+ studies, efgartigimod treatment resulted in consistent and repeatable MG-ADL score decreases in adolescent participants with many reaching MSE (MG-ADL 0-1)

Pharmacodynamics, efficacy, and overall safety was similar in adolescents treated with efgartigimod compared with previous studies in adults

Efgartigimod IV may address an unmet need for the treatment of gMG in juvenile patients