EGARTIGIMOD TREATMENT OF PATIENTS WITH GENERLIZED MYASTHENIA GRAVIS: **RESULTS OF THE PHASE 3 ADAPT STUDY**

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Disclosures

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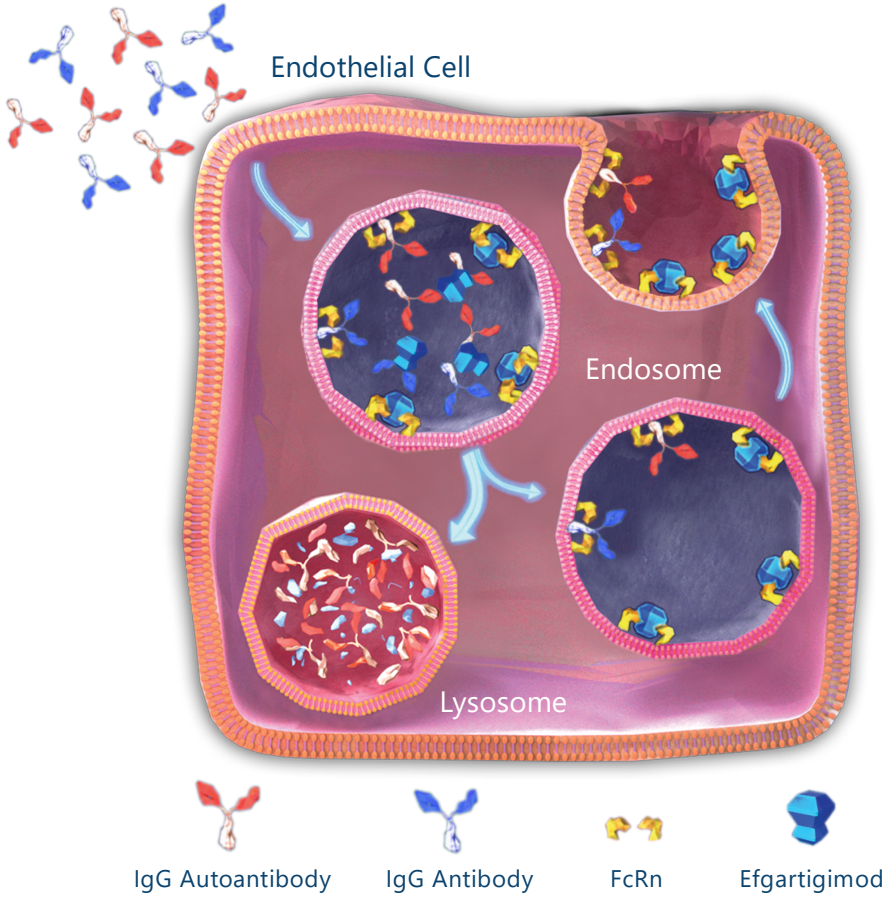
- Efgartigimod is an investigational agent that is not currently approved for use by any regulatory agency.

Efgartigimod Mechanism of Action: blocking neonatal Fc Receptor (FcRn)

- FcRn recycles IgG, extending its half life-life and abundancy
- Efgartigimod is a human IgG1 Fc fragment, a natural ligand of FcRn, engineered for increased affinity to FcRn
- Efgartigimod binds in the same way as endogenous IgG, preserving characteristic pH-dependent binding
- Efgartigimod was designed to outcompete endogenous IgG, preventing recycling, promoting IgG lysosomal degradation
  - Targeted reduction of all IgG subtypes
  - No impact on IgM, IgA or albumin



ADAPT Study Design

DESIGN

167 gMG patients

MGFA Class II, III, IV

AChR-antibody positive or negative

MG-ADL score ≥5*

On a minimum of one stable gMG treatment**

2 weeks screening

Patients randomized 1:1 to receive 10 mg/kg IV efgartigimod or placebo

26 weeks

Primary endpoint: MG-ADL responders in AChR-Ab+ patients in cycle 1 (8 weeks)

Open – label Extension

- 151 patients who completed ADAPT entered the ADAPT+ Study
- Open label, efgartigimod treatment cycles
- **Up to 3 years treatment**

DOSING

Treatment Cycles of 4 weekly IV infusions
(1 hour infusion)

All patients receive initial treatment cycle

Individualized treatment cycles
(*up to 3 cycles in 26 weeks*)

Time between cycles determined by duration of clinically meaningful improvement (CMI)

Retreatment criteria:

- ≥8 weeks since initiation of previous cycle
- Total MG-ADL ≥5 points*
- For MG-ADL responders, no CMI in MG-ADL (i.e., <2-point reduction compared to start of cycle)

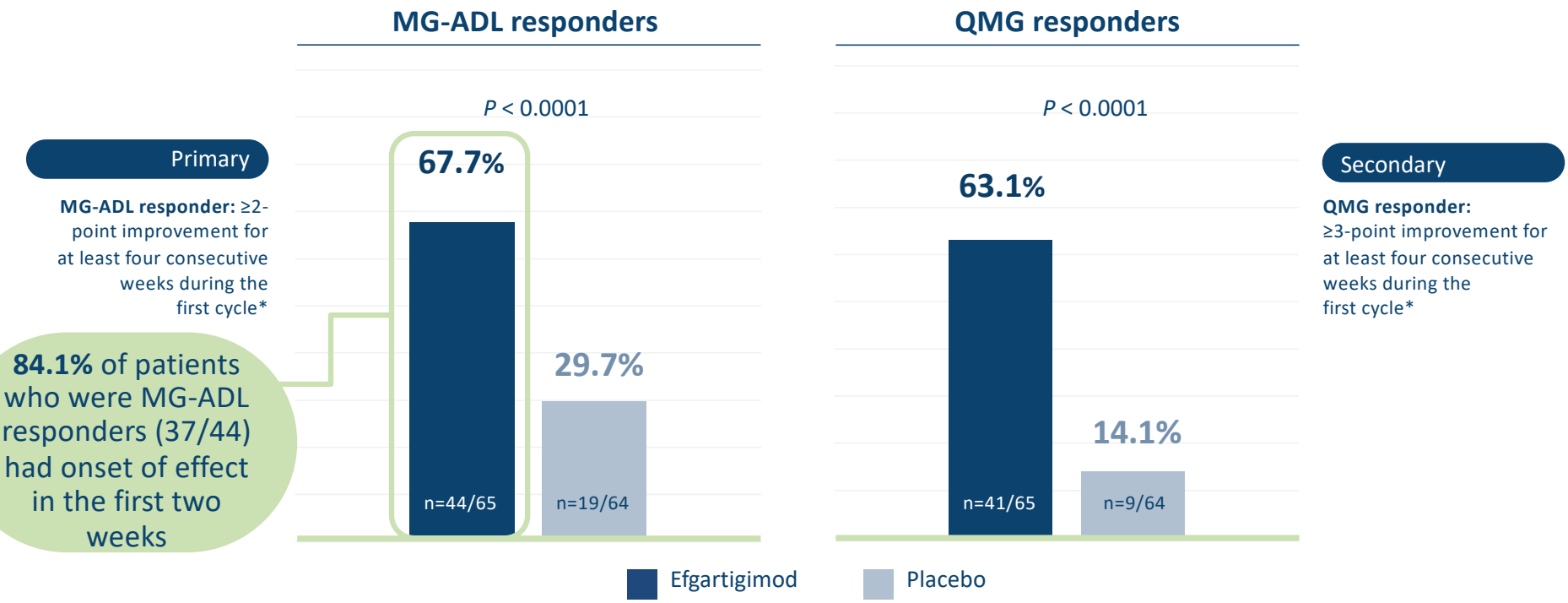
*50% of the score attributed to non-ocular items; **(Acetylcholinesterase inhibitor, Steroid +/or Non-steroidal immunosuppressive therapy)
gMG, generalized myasthenia gravis; IV, intravenous
Note: Patients requiring rescue therapy discontinued from the study treatment

Baseline Patient Characteristics

	AChR Ab+ patients		Overall Population	
	Efgartigimod (n=65)	Placebo (n=64)	Efgartigimod (n=84)	Placebo (n=83)
Age *Mean years (SD)*	44.7 (15.0)	49.2 (15.5)	45.9 (14.4)	48.2 (15.0)
Female *n (%)*	46 (70.8)	40 (62.5)	63 (75.0)	55 (66.3)
Time since diagnosis *Mean years (SD)*	9.68 (8.3)	8.93 (8.2)	10.13 (9.0)	8.83 (7.6)
MG-ADL score *Mean (SD)*	9.0 (2.5)	8.6 (2.1)	9.2 (2.6)	8.8 (2.3)
QMG score *Mean (SD)*	16.0 (5.1)	15.2 (4.4)	16.2 (5.0)	15.5 (4.6)
MGFA class at screening *n (%)*				
Class II	28 (43.1)	25 (39.1)	34 (40.5)	31 (37.3)
Class III	35 (53.8)	36 (56.3)	47 (56.0)	49 (59.0)
Class IV	2 (3.1)	3 (4.7)	3 (3.6)	3 (3.6)
Prior treatment with NSIST *n (%)*	47 (72.3)	43 (67.2)	62 (73.8)	57 (68.7)
MG therapies at baseline* *n (%)*				
Any NSIST	40 (61.5)	37 (57.8)	51 (60.7)	51 (61.4)
Any Steroid	46 (70.8)	51 (79.7)	60 (71.4)	67 (80.7)
AChR Ab status *(n AChR Ab+ / n AChR Ab-)*	65 / 0	64 / 0	65 / 19	64 / 19

*Non-steroidal immunosuppressive therapy (NSIST)): Azathioprine, Cyclosporin, Cyclophosphamide, Methotrexate, Mycophenolate, Tacrolimus (in mono- or combination therapy)

Efgartigimod provided statistically significant clinical benefit (AChR-Ab+ patients, Cycle 1)



Primary

MG-ADL responder: ≥2-point improvement for at least four consecutive weeks during the first cycle*

84.1% of patients who were MG-ADL responders (37/44) had onset of effect in the first two weeks

Secondary

QMG responder: ≥3-point improvement for at least four consecutive weeks during the first cycle*

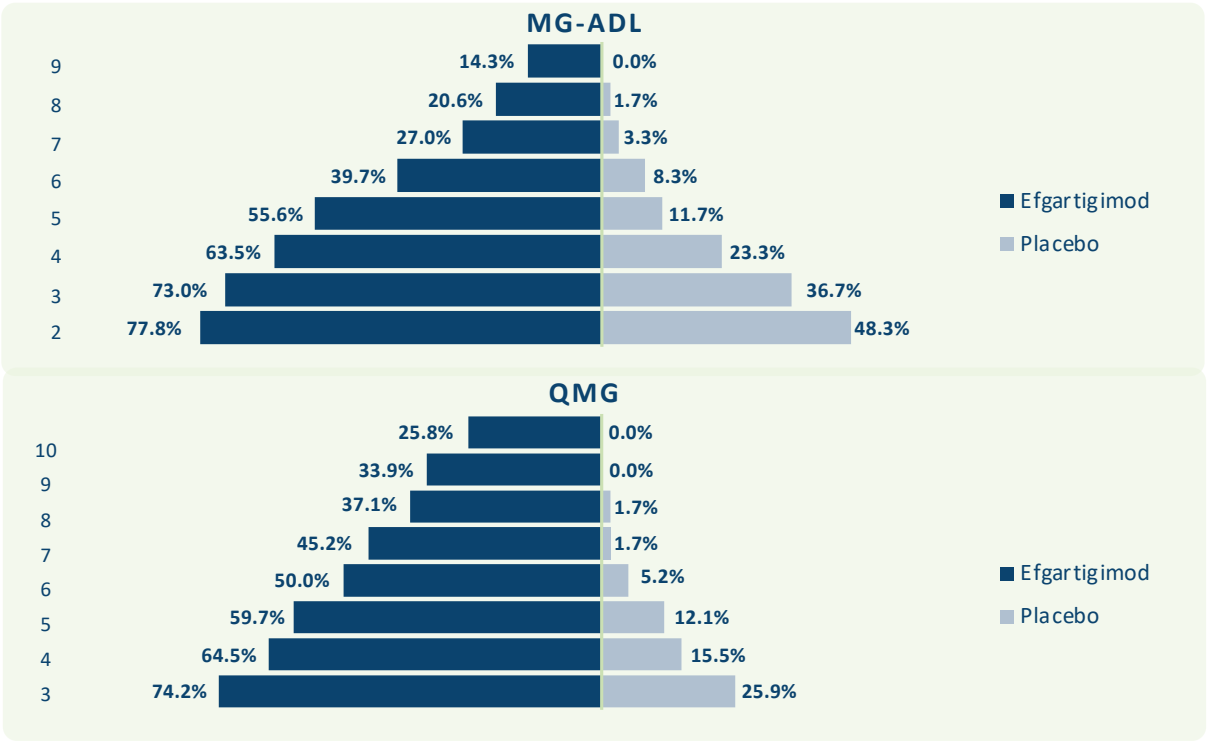
Significantly more efgartigimod treated patients had clinically meaningful improvement in function and strength

* The first reduction had to occur no later than 1 week after the last infusion

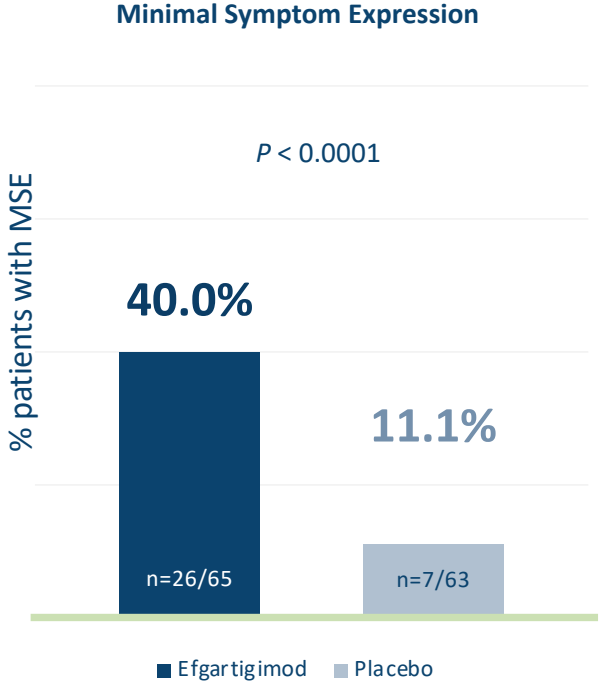
MG-ADL, Myasthenia Gravis Activities of Daily Living; QMG, Quantitative Myasthenia Gravis Score

Proportion of patients with increasing MG-ADL and QMG improvement and achieving Minimal Symptom Expression (AChR-Ab+ patients, Cycle 1)

Proportion of patients with increasing thresholds of MG-ADL and QMG improvement at week 4*

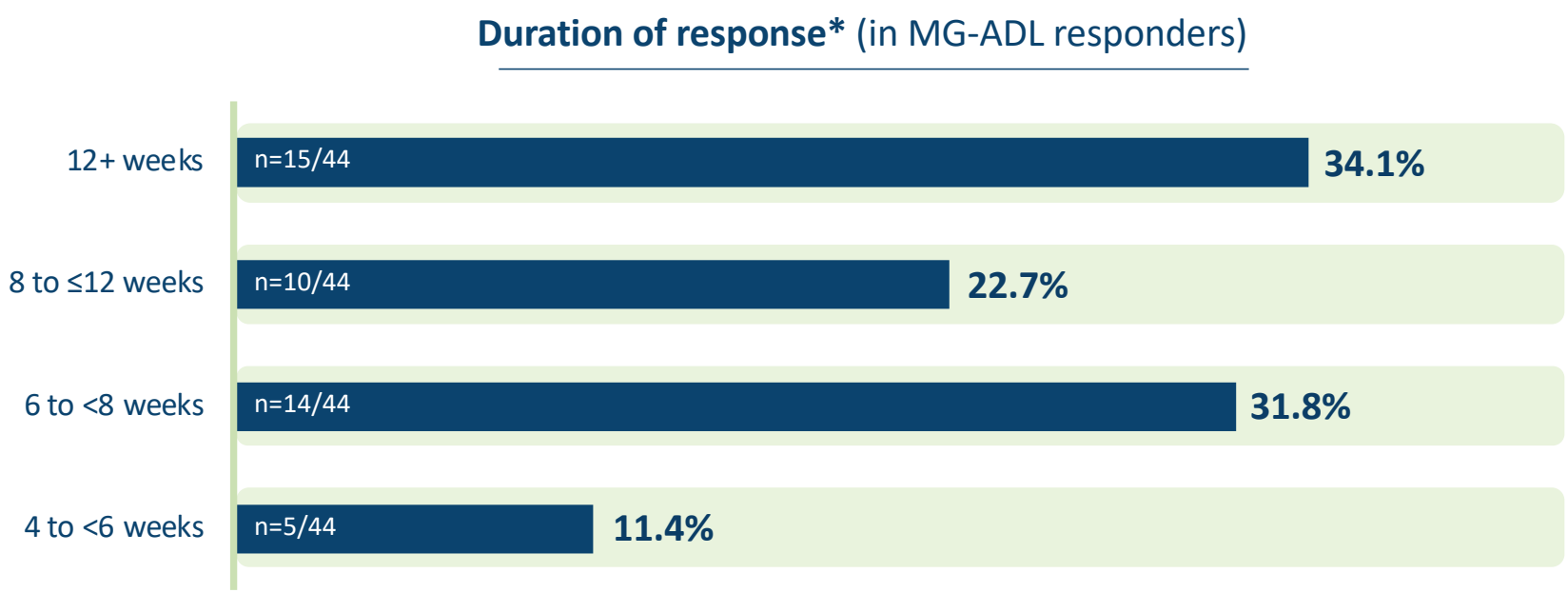


Proportion of patients with MSE (MG-ADL 0 or 1) any time during cycle 1



MSE, Minimal Symptom Expression
*One week after last infusion of cycle

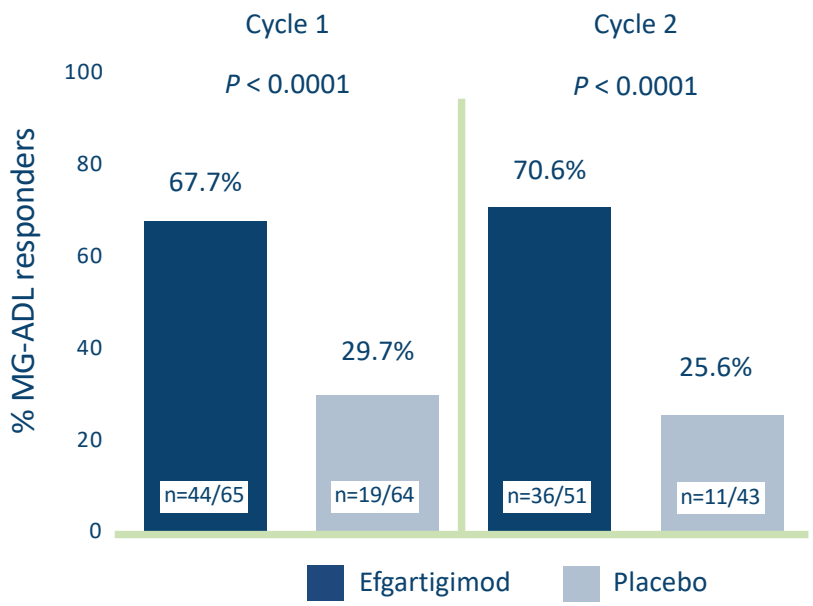
Duration of MG-ADL response (AChR-Ab+ patients, Cycle 1)



***Duration of response:**
Defined as duration of MG-ADL improvement ≥2 points

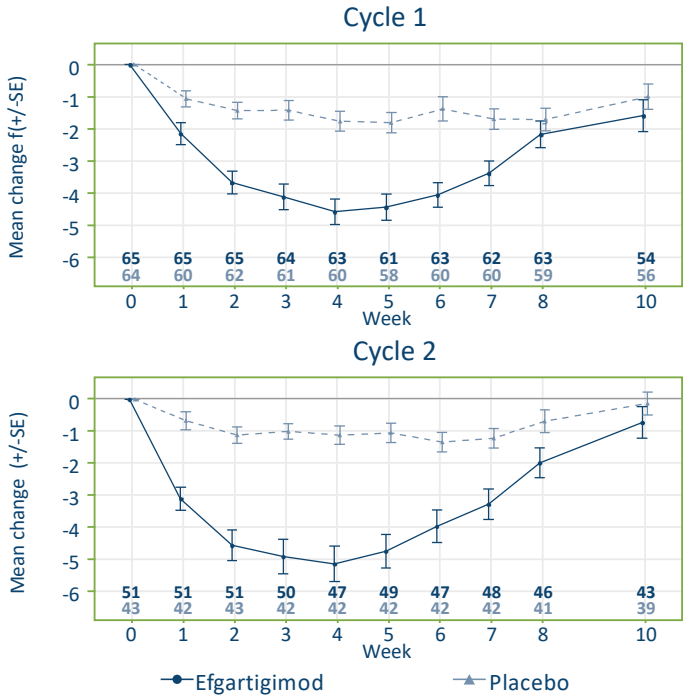
Repeatability of response: MG-ADL responders and change in MG-ADL (AChR-Ab+ patients, Cycles 1 and 2)

MG-ADL responders



36.8% (7/19) efgartigimod patients who were not MG-ADL responders in cycle 1 and were retreated achieved MG-ADL responder for the first time in cycle 2

Total MG-ADL: Mean change from cycle baseline



Across cycles 1 and 2 **78.5% (51/65)** efgartigimod patients were MG-ADL responders

The number of patients in cycle 2 is smaller as some patients only required one treatment cycle during the study
The numbers below trend lines indicate the number of patient measurements for each data set

Secondary Endpoint Overview

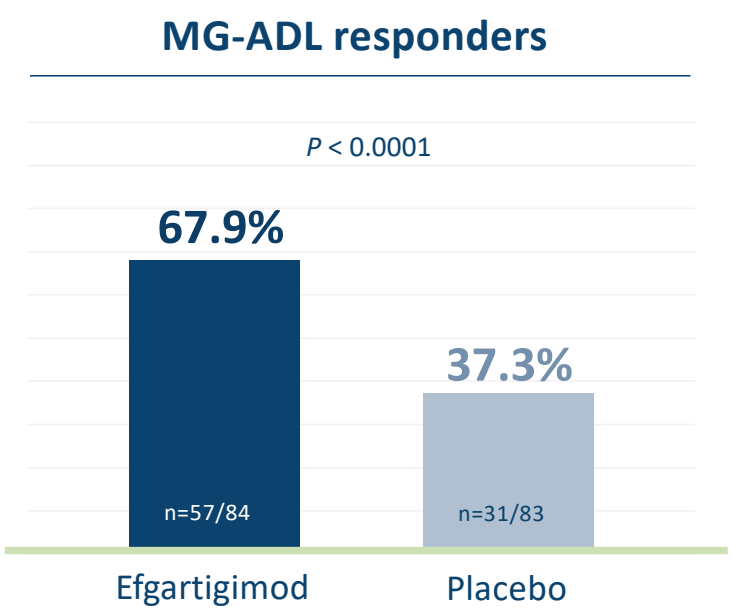
Secondary Endpoints	Measure	Population	Time	Efgartigimod	Placebo	P-value
Response	*QMG responder*	*AChR Ab +*	*Cycle 1*	63.1% (41/65)	14.1% (9/64)	<0.0001
Response	*MG-ADL responder*	*Overall Population*	*Cycle 1*	67.9% (57/84)	37.3% (31/83)	<0.0001
Duration	*% of study duration ≥ 2-point improvement in MG-ADL*	*AChR Ab +*	*Until day 126**	48.7%	26.6%	0.0001
Duration	*Days until qualification for retreatment, measured from day 28 until no CMI*	*AChR Ab +*	*Full study*	Median 35 days	Median 8 days	0.2604
Onset	*MG-ADL responder, with onset within first 2 weeks*	*AChR Ab +*	*Cycle 1*	56.9% (37/65)	25.0% (16/64)	--**

*Day 126 was the last day it was possible to start and complete a retreatment cycle within the study
**Not formally tested per hierarchical testing as prior endpoint did not meet significance
CMI, Clinically Meaningful Impact

Overall population and AChR-Ab seronegative treatment effect (Cycle 1)

Overall population* similar results to AChR-Ab+ patients

AChR-Ab- patients
Patient responder analyses

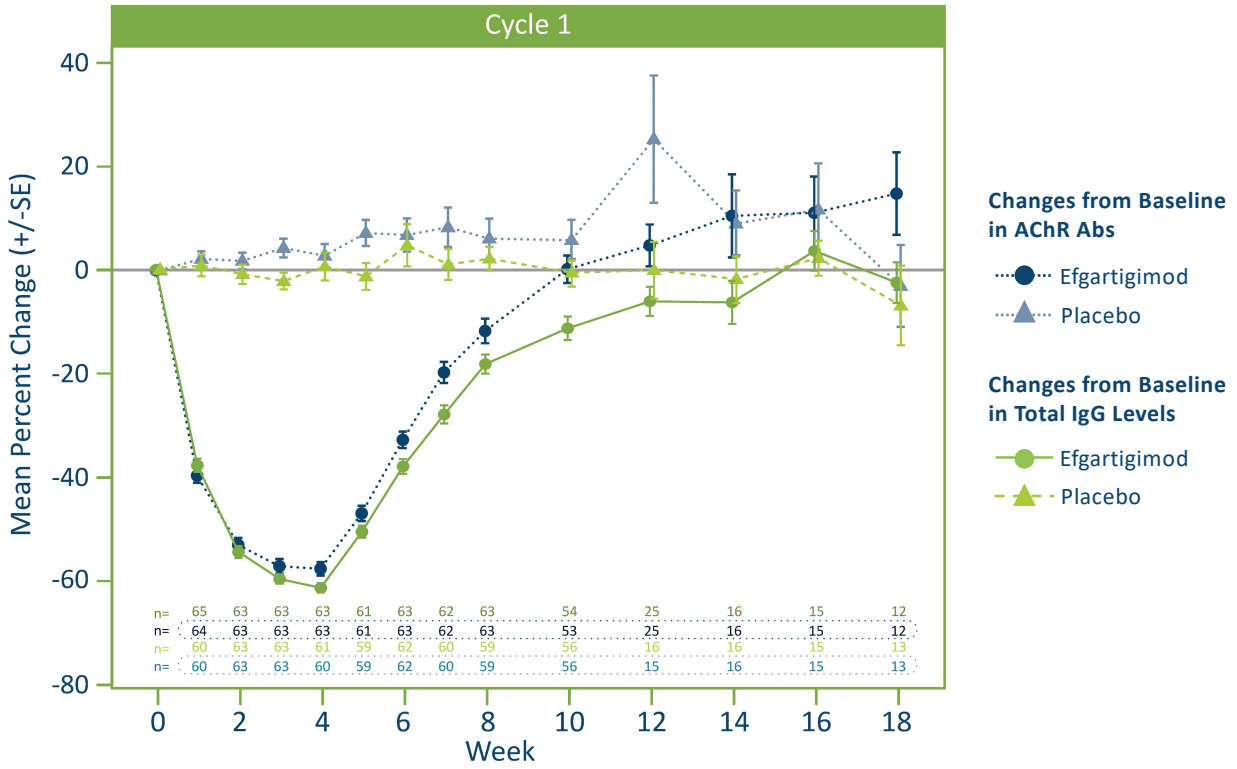


	Efgartigimod (n = 19)	Placebo (n = 19)
MG-ADL responders	**13** (68.4%)	**12** (63.2%)
QMG responders	**10** (52.6%)	**7** (36.8%)
MG-ADL responder **AND** QMG responder**	**9** (47.4%)	**4** (21.1%)

*AChR-Ab+ and AChR-Ab negative
**Post-hoc analysis

Pharmacodynamic effect: change in total IgG and AChR-Ab (AChR-Ab+ patients, Cycle 1)

- Maximum mean reduction at week 4: Total IgG 61.3%, AChR-Ab 57.6%
- Similar reduction across subtypes (IgG1, 2, 3, 4)
- Overall population experienced similar reductions (AChR-Ab+ and AChR-Ab negative)
- Albumin levels did not change



The numbers below trend lines indicate the number of patient measurements for each data set

Safety: Summary of Adverse events (AEs)

AEs were predominantly **mild or moderate in severity**. Efgartigimod was generally **well tolerated**.

	Efgartigimod (n=84)	Placebo (n=83)
AEs *n (%)*	65 (77.4)	70 (84.3)
SAEs *n (%)*	4 (4.8)	7 (8.4)
Most frequent AEs *n (%)*		
Headache	24 (28.6)	23 (27.7)
Nasopharyngitis	10 (11.9)	15 (18.1)
Nausea	7 (8.3)	9 (10.8)
Diarrhea	6 (7.1)	9 (10.8)
Upper respiratory tract infection	9 (10.7)	4 (4.8)
Urinary tract infection	8 (9.5)	4 (4.8)
≥1 Infusion-related reaction event	3 (3.6)	8 (9.6)
Infection AEs* *n (%)*	39 (46.4)	31 (37.3)
Discontinued study treatment due to AEs** *n (%)*	3 (3.6)	3 (3.6)

All AEs were treatment emergent AEs
Adverse Event (AE); Serious Adverse Event (SAE)
*3 severe infections: Influenza, pharyngitis (efgartigimod); Urinary tract infection (placebo); **Efgartigimod treated patients: depression, rectal adenocarcinoma, thrombocytosis

Summary of ADAPT results

- Clinically and statistically significant improvements in function (MG-ADL responders, 67.7%) and strength (QMG responders, 63.1%) observed in efgartigimod treated AChR-Ab+ patients compared to placebo (29.7% and 14.1%, respectively).
- Similar improvements were seen in the overall population (AChR-Ab+ and AChR-Ab- patients).
- Efgartigimod was well tolerated and most AEs were mild or moderate in severity. No infusion-related reaction AE signal.
- 91% of patients have enrolled in the OLE, ADAPT+.