

Supplementary Information

Title

Safety and effectiveness of efgartigimod in patients with generalized myasthenia gravis by autoantibody subtype: analysis of post-marketing surveillance in Japan

Authors

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Supplementary Table S1. Efgartigimod safety by autoantibody subtype during the observation period

Item	Overall		AChR-Ab+		MuSK-Ab+		Double-SN	
SAS	473		268		63		138	
M of ADR	231		133		47		51	
Person-year	354.4		193.5		50.9		106.9	
SOC and PT	M of ADR ^a	M of SADR ^a	M of ADR ^a	M of SADR ^a	M of ADR ^a	M of SADR ^a	M of ADR ^a	M of SADR ^a
General disorders and administration site conditions	97 (27.4)	11 (3.1)	70 (36.2)	7 (3.6)	12 (23.6)	1 (2.0)	15 (14.0)	3 (2.8)
Symptom recurrence	76 (21.4)	10 (2.8)	58 (30.0)	7 (3.6)	5 (9.8)	0 (0.0)	13 (12.2)	3 (2.8)
Malaise	8 (2.3)	0 (0.0)	6 (3.1)	0 (0.0)	1 (2.0)	0 (0.0)	1 (0.9)	0 (0.0)
Fatigue	3 (0.9)	0 (0.0)	2 (1.0)	0 (0.0)	1 (2.0)	0 (0.0)	0 (0.0)	0 (0.0)
Feeling abnormal	3 (0.9)	0 (0.0)	2 (1.0)	0 (0.0)	0 (0.0)	0 (0.0)	1 (0.9)	0 (0.0)
Oedema	2 (0.6)	0 (0.0)	0 (0.0)	0 (0.0)	2 (3.9)	0 (0.0)	0 (0.0)	0 (0.0)
Pyrexia	2 (0.6)	1 (0.3)	1 (0.5)	0 (0.0)	1 (2.0)	1 (2.0)	0 (0.0)	0 (0.0)
Asthenia	1 (0.3)	0 (0.0)	1 (0.5)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
Chest discomfort	1 (0.3)	0 (0.0)	0 (0.0)	0 (0.0)	1 (2.0)	0 (0.0)	0 (0.0)	0 (0.0)
Chest pain	1 (0.3)	0 (0.0)	0 (0.0)	0 (0.0)	1 (2.0)	0 (0.0)	0 (0.0)	0 (0.0)
Injury, poisoning and procedural complications	42 (11.9)	0 (0.0)	20 (10.3)	0 (0.0)	18 (35.4)	0 (0.0)	4 (3.7)	0 (0.0)
Procedural headache	42 (11.9)	0 (0.0)	20 (10.3)	0 (0.0)	18 (35.4)	0 (0.0)	4 (3.7)	0 (0.0)
Infections and infestations	23 (6.5)	3 (0.9)	10 (5.2)	1 (0.5)	5 (9.8)	0 (0.0)	8 (7.5)	2 (1.9)
Herpes zoster	4 (1.1)	0 (0.0)	2 (1.0)	0 (0.0)	2 (3.9)	0 (0.0)	0 (0.0)	0 (0.0)
Pneumonia	4 (1.1)	3 (0.9)	2 (1.0)	1 (0.5)	0 (0.0)	0 (0.0)	2 (1.9)	2 (1.9)
COVID-19	3 (0.9)	0 (0.0)	1 (0.5)	0 (0.0)	1 (2.0)	0 (0.0)	1 (0.9)	0 (0.0)
Bronchitis	1 (0.3)	0 (0.0)	1 (0.5)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)

Cellulitis	1 (0.3)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	1 (0.9)	0 (0.0)
Cystitis	1 (0.3)	0 (0.0)	0 (0.0)	0 (0.0)	1 (2.0)	0 (0.0)	0 (0.0)	0 (0.0)
Gastroenteritis	1 (0.3)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	1 (0.9)	0 (0.0)
Influenza	1 (0.3)	0 (0.0)	1 (0.5)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
Nasopharyngitis	1 (0.3)	0 (0.0)	1 (0.5)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
Periodontitis	1 (0.3)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	1 (0.9)	0 (0.0)
Pharyngitis	1 (0.3)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	1 (0.9)	0 (0.0)
Sinusitis	1 (0.3)	0 (0.0)	0 (0.0)	0 (0.0)	1 (2.0)	0 (0.0)	0 (0.0)	0 (0.0)
Urinary tract infection	1 (0.3)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	1 (0.9)	0 (0.0)
Malassezia infection	1 (0.3)	0 (0.0)	1 (0.5)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
Herpes zoster reactivation	1 (0.3)	0 (0.0)	1 (0.5)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
Gastrointestinal disorder	18 (5.1)	0 (0.0)	6 (3.1)	0 (0.0)	3 (5.9)	0 (0.0)	9 (8.4)	0 (0.0)
Diarrhoea	8 (2.3)	0 (0.0)	2 (1.0)	0 (0.0)	2 (3.9)	0 (0.0)	4 (3.7)	0 (0.0)
Nausea	5 (1.4)	0 (0.0)	2 (1.0)	0 (0.0)	1 (2.0)	0 (0.0)	2 (1.9)	0 (0.0)
Vomiting	2 (0.6)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	2 (1.9)	0 (0.0)
Abdominal pain upper	1 (0.3)	0 (0.0)	1 (0.5)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
Constipation	1 (0.3)	0 (0.0)	1 (0.5)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
Faeces soft	1 (0.3)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	1 (0.9)	0 (0.0)
Nervous system disorder	16 (4.5)	3 (0.9)	11 (5.7)	2 (1.0)	4 (7.9)	1 (2.0)	1 (0.9)	0 (0.0)
Myasthenia gravis	8 (2.3)	2 (0.6)	4 (2.1)	1 (0.5)	3 (5.9)	1 (2.0)	1 (0.9)	0 (0.0)
Dizziness	2 (0.6)	0 (0.0)	2 (1.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
Myasthenia gravis crisis	1 (0.3)	1 (0.3)	1 (0.5)	1 (0.5)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
Headache	1 (0.3)	0 (0.0)	1 (0.5)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
Migraine	1 (0.3)	0 (0.0)	1 (0.5)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)

Paraesthesia	1 (0.3)	0 (0.0)	1 (0.5)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
Somnolence	1 (0.3)	0 (0.0)	1 (0.5)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
Occipital neuralgia	1 (0.3)	0 (0.0)	0 (0.0)	0 (0.0)	1 (2.0)	0 (0.0)	0 (0.0)	0 (0.0)
Investigations	8 (2.3)	0 (0.0)	4 (2.1)	0 (0.0)	1 (2.0)	0 (0.0)	3 (2.8)	0 (0.0)
Gamma-glutamyltransferase increased	2 (0.6)	0 (0.0)	1 (0.5)	0 (0.0)	0 (0.0)	0 (0.0)	1 (0.9)	0 (0.0)
Alanine aminotransferase increased	1 (0.3)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	1 (0.9)	0 (0.0)
Aspartate aminotransferase increased	1 (0.3)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	1 (0.9)	0 (0.0)
Blood albumin increased	1 (0.3)	0 (0.0)	1 (0.5)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
Blood immunoglobulin G decreased	1 (0.3)	0 (0.0)	1 (0.5)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
C-reactive protein increased	1 (0.3)	0 (0.0)	1 (0.5)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
Lymphocyte count decreased	1 (0.3)	0 (0.0)	0 (0.0)	0 (0.0)	1 (2.0)	0 (0.0)	0 (0.0)	0 (0.0)
Skin and subcutaneous tissue disorders	6 (1.7)	0 (0.0)	3 (1.6)	0 (0.0)	1 (2.0)	0 (0.0)	2 (1.9)	0 (0.0)
Rash	3 (0.9)	0 (0.0)	1 (0.5)	0 (0.0)	0 (0.0)	0 (0.0)	2 (1.9)	0 (0.0)
Drug eruption	2 (0.6)	0 (0.0)	2 (1.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
Pruritus	1 (0.3)	0 (0.0)	0 (0.0)	0 (0.0)	1 (2.0)	0 (0.0)	0 (0.0)	0 (0.0)
Cardiac disorders	5 (1.4)	0 (0.0)	0 (0.0)	0 (0.0)	3 (5.9)	0 (0.0)	2 (1.9)	0 (0.0)
Palpitations	5 (1.4)	0 (0.0)	0 (0.0)	0 (0.0)	3 (5.9)	0 (0.0)	2 (1.9)	0 (0.0)
Immune system disorder	4 (1.1)	4 (1.1)	2 (1.0)	2 (1.0)	0 (0.0)	0 (0.0)	2 (1.9)	2 (1.9)
Hypogammaglobulinaemia	3 (0.9)	3 (0.9)	2 (1.0)	2 (1.0)	0 (0.0)	0 (0.0)	1 (0.9)	1 (0.9)
Anaphylactic shock	1 (0.3)	1 (0.3)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	1 (0.9)	1 (0.9)
Musculoskeletal and connective tissue disorders	4 (1.1)	0 (0.0)	1 (0.5)	0 (0.0)	0 (0.0)	0 (0.0)	3 (2.8)	0 (0.0)
Arthralgia	1 (0.3)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	1 (0.9)	0 (0.0)
Back pain	1 (0.3)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	1 (0.9)	0 (0.0)
Myalgia	1 (0.3)	0 (0.0)	1 (0.5)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)

Neck pain	1 (0.3)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	1 (0.9)	0 (0.0)
Respiratory, thoracic and mediastinal disorders	3 (0.9)	1 (0.3)	2 (1.0)	1 (0.5)	0 (0.0)	0 (0.0)	1 (0.9)	0 (0.0)
Upper respiratory tract inflammation	3 (0.9)	1 (0.3)	2 (1.0)	1 (0.5)	0 (0.0)	0 (0.0)	1 (0.9)	0 (0.0)
Vascular disorders	2 (0.6)	0 (0.0)	2 (1.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
Hypertension	1 (0.3)	0 (0.0)	1 (0.5)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
Vasculitis	1 (0.3)	0 (0.0)	1 (0.5)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
Neoplasms benign, malignant and unspecified (incl cysts and polyps)	1 (0.3)	1 (0.3)	1 (0.5)	1 (0.5)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
Lymphoproliferative disorder	1 (0.3)	1 (0.3)	1 (0.5)	1 (0.5)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
Metabolism and nutrition disorders	1 (0.3)	0 (0.0)	1 (0.5)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
Decreased appetite	1 (0.3)	0 (0.0)	1 (0.5)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
Ear and labyrinth disorders	1 (0.3)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	1 (0.9)	0 (0.0)
Motion sickness	1 (0.3)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	1 (0.9)	0 (0.0)

Abbreviations: Ab = antibody, AChR = acetylcholine receptor, ADR = adverse drug reaction, M = number of events, MuSK = muscle-specific kinase, N = number of patients, PT = preferred term, SADR = serious adverse drug reaction, SAS = safety analysis set, SD = standard deviation, SN = seronegative, SOC = system organ class

a. Events per year

The autoantibody subtype for four patients was unknown.

Supplementary Table S2. MG-ADL total and subdomain scores by autoantibody subtype (Cycle 1)

Analysis population	Item	Score	Cycle	Administration period	Value			Change from the first administration in the cycle			
					N	Mean	SD	N	Mean	SD	Test ^a
Overall	MG-ADL total score	0 - 24	Cycle 1	1 st	290	7.2	4.0	-	-	-	-
				2 nd	159	5.8	4.1	159	-1.6	2.2	< 0.001
				3 rd	165	5.0	4.2	165	-2.4	2.7	< 0.001
				4 th	206	4.2	3.7	204	-3.0	3.0	< 0.001
	Ocular	0 - 6	Cycle 1	1 st	293	2.9	1.9	-	-	-	-
				2 nd	162	2.4	1.9	162	-0.6	1.0	< 0.001
				3 rd	168	2.1	1.8	168	-0.9	1.3	< 0.001
				4 th	207	1.9	1.8	207	-1.0	1.4	< 0.001
	Bulbar	0 - 9	Cycle 1	1 st	290	1.9	1.9	-	-	-	-
				2 nd	160	1.4	1.8	159	-0.6	1.2	< 0.001
				3 rd	166	1.2	1.7	165	-0.9	1.3	< 0.001
				4 th	206	0.9	1.4	204	-1.1	1.5	< 0.001
	Limb/Gross motor	0 - 6	Cycle 1	1 st	291	1.7	1.5	-	-	-	-
				2 nd	160	1.4	1.4	160	-0.3	0.8	< 0.001
				3 rd	166	1.1	1.5	166	-0.5	1.0	< 0.001
				4 th	207	1.0	1.3	205	-0.7	1.1	< 0.001
	Respiratory	0 - 3	Cycle 1	1 st	292	0.7	0.7	-	-	-	-
				2 nd	162	0.5	0.7	161	-0.1	0.4	< 0.001
				3 rd	168	0.6	0.7	167	-0.1	0.4	< 0.001
				4 th	207	0.4	0.6	206	-0.2	0.6	< 0.001

Analysis population	Item	Score	Cycle	Administration period	Value			Change from the first administration in the cycle			
					N	Mean	SD	N	Mean	SD	Test ^a
AChR-Ab+	MG-ADL total score	0 - 24	Cycle 1	1 st	170	6.6	3.9	-	-	-	-
				2 nd	100	5.0	3.7	100	-1.8	2.3	< 0.001
				3 rd	98	4.4	3.8	98	-2.5	2.7	< 0.001
				4 th	124	3.8	3.4	123	-3.0	3.1	< 0.001
	Ocular	0 - 6	Cycle 1	1 st	172	2.8	2.0	-	-	-	-
				2 nd	102	2.3	1.9	102	-0.6	1.1	< 0.001
				3 rd	100	2.0	1.8	100	-0.9	1.3	< 0.001
				4 th	125	1.9	1.9	125	-1.1	1.4	< 0.001
	Bulbar	0 - 9	Cycle 1	1 st	170	1.7	1.8	-	-	-	-
				2 nd	100	1.1	1.6	100	-0.8	1.3	< 0.001
				3 rd	98	0.9	1.5	98	-0.9	1.3	< 0.001
				4 th	124	0.7	1.3	123	-1.1	1.6	< 0.001
	Limb/Gross motor	0 - 6	Cycle 1	1 st	171	1.5	1.3	-	-	-	-
				2 nd	101	1.1	1.2	101	-0.3	0.8	< 0.001
				3 rd	99	0.9	1.2	99	-0.5	1.0	< 0.001
				4 th	125	0.8	1.1	124	-0.7	1.1	< 0.001
	Respiratory	0 - 3	Cycle 1	1 st	172	0.6	0.7	-	-	-	-
				2 nd	102	0.5	0.7	102	-0.1	0.4	p=0.013
				3 rd	100	0.5	0.7	100	-0.1	0.5	p=0.004
				4 th	125	0.4	0.6	125	-0.1	0.5	p=0.002
MuSK-Ab+	MG-ADL total score	0 - 24	Cycle 1	1 st	36	7.5	3.7	-	-	-	-

Analysis population	Item	Score	Cycle	Administraction period	Value		Change from the first administration in the cycle				
					N	Mean	SD	N	Mean	SD	Test ^a
Double-SN			Cycle 1	2 nd	17	6.8	3.4	17	-1.0	2.2	p=0.109
				3 rd	18	4.9	3.6	18	-2.9	2.7	< 0.001
				4 th	24	2.9	3.4	23	-3.7	3.1	< 0.001
	Ocular	0 - 6	Cycle 1	1 st	37	2.6	1.6	-	-	-	-
				2 nd	18	2.2	1.6	18	-0.7	1.2	p=0.047
				3 rd	19	1.5	1.3	19	-1.2	1.4	< 0.001
				4 th	24	1.0	1.2	24	-1.3	1.4	< 0.001
	Bulbar	0 - 9	Cycle 1	1 st	36	2.8	2.2	-	-	-	-
				2 nd	18	2.1	1.5	17	-0.4	1.5	p=0.422
				3 rd	19	1.8	2.1	18	-1.1	1.6	p=0.017
				4 th	24	1.1	1.5	23	-1.4	1.6	< 0.001
	Limb/Gross motor	0 - 6	Cycle 1	1 st	36	1.4	1.4	-	-	-	-
				2 nd	17	1.5	1.5	17	-0.1	0.5	p=0.625
				3 rd	18	0.9	1.4	18	-0.5	0.9	p=0.031
				4 th	24	0.5	1.2	23	-0.7	1.1	p=0.004
	Respiratory	0 - 3	Cycle 1	1 st	36	0.8	0.7	-	-	-	-
				2 nd	18	0.7	0.6	17	0.0	0.0	-
				3 rd	19	0.4	0.6	18	-0.2	0.4	p=0.250
4 th				24	0.3	0.5	23	-0.3	0.5	p=0.008	
Double-SN	MG-ADL total score	0 - 24	Cycle 1	1 st	80	8.3	4.3	-	-	-	-
				2 nd	38	7.4	4.9	38	-1.4	2.3	< 0.001

Analysis population	Item	Score	Cycle	Administration period	Value			Change from the first administration in the cycle			
					N	Mean	SD	N	Mean	SD	Test ^a
Analysis population	Ocular	0 - 6	Cycle 1	3 rd	47	6.2	4.9	47	-2.0	2.6	< 0.001
				4 th	56	5.5	3.9	56	-2.8	2.8	< 0.001
				1 st	80	3.1	1.8	-	-	-	-
				2 nd	38	2.9	1.9	38	-0.5	0.8	< 0.001
				3 rd	47	2.3	1.9	47	-0.8	1.2	< 0.001
				4 th	56	2.1	1.9	56	-0.8	1.3	< 0.001
	Bulbar	0 - 9	Cycle 1	1 st	80	2.0	1.9	-	-	-	-
				2 nd	38	1.8	2.2	38	-0.4	0.9	< 0.001
				3 rd	47	1.5	1.8	47	-0.6	1.1	< 0.001
				4 th	56	1.2	1.5	56	-0.9	1.1	< 0.001
	Limb/Gross motor	0 - 6	Cycle 1	1 st	80	2.3	1.6	-	-	-	-
				2 nd	38	2.1	1.7	38	-0.4	0.9	p=0.024
				3 rd	47	1.7	1.8	47	-0.5	1.1	p=0.001
				4 th	56	1.6	1.4	56	-0.8	1.1	< 0.001
	Respiratory	0 - 3	Cycle 1	1 st	80	0.9	0.7	-	-	-	-
				2 nd	38	0.7	0.7	38	-0.1	0.5	p=0.188
				3 rd	47	0.7	0.7	47	-0.1	0.4	p=0.031
				4 th	56	0.5	0.6	56	-0.3	0.6	< 0.001

Abbreviations: Ab = antibody, AChR = acetylcholine receptor, MG-ADL = Myasthenia Gravis-Activities of Daily Living, MuSK = muscle-specific kinase, N = number of patients, SD = standard deviation, SN = seronegative

a. Wilcoxon signed-rank sum test

Supplementary Table S3. MG-ADL total score by autoantibody subtype (Cycle 1-3)

Analysis population	Item	Cycle	Administration period	Value			Change from the first administration in the cycle			
				N	Mean	SD	N	Mean	SD	Test ^a
Overall	MG-ADL total score	Cycle 1	1 st	290	7.2	4.0	-	-	-	-
			2 nd	159	5.8	4.1	159	-1.6	2.2	< 0.001
			3 rd	165	5.0	4.2	165	-2.4	2.7	< 0.001
			4 th	206	4.2	3.7	204	-3.0	3.0	< 0.001
		Cycle 2	1 st	181	6.8	4.0	-	-	-	-
			2 nd	107	5.2	3.8	100	-1.5	2.4	< 0.001
			3 rd	111	4.4	3.8	101	-2.3	2.5	< 0.001
			4 th	121	4.0	3.8	107	-2.6	2.9	< 0.001
		Cycle 3	1 st	129	7.1	4.0	-	-	-	-
			2 nd	76	5.3	3.9	65	-1.3	2.5	< 0.001
			3 rd	79	4.6	3.8	70	-1.9	2.3	< 0.001
			4 th	92	4.4	4.1	82	-2.8	3.0	< 0.001
AChR-Ab+	MG-ADL total score	Cycle 1	1 st	170	6.6	3.9	-	-	-	-
			2 nd	100	5.0	3.7	100	-1.8	2.3	< 0.001
			3 rd	98	4.4	3.8	98	-2.5	2.7	< 0.001
			4 th	124	3.8	3.4	123	-3.0	3.1	< 0.001
		Cycle 2	1 st	104	6.5	4.0	-	-	-	-
			2 nd	63	4.3	3.5	61	-1.6	2.6	< 0.001
			3 rd	66	3.9	3.6	60	-2.1	2.5	< 0.001
			4 th	73	3.9	3.9	66	-2.3	2.9	< 0.001
		Cycle 3	1 st	76	6.4	3.9	-	-	-	-

Analysis population	Item	Cycle	Administration period	Value			Change from the first administration in the cycle			
				N	Mean	SD	N	Mean	SD	Test ^a
MuSK-Ab +	MG-ADL total score	Cycle 1	2 nd	41	4.9	3.9	38	-1.4	2.4	< 0.001
			3 rd	44	3.8	3.5	41	-2.2	2.3	< 0.001
			4 th	54	3.9	4.3	50	-2.6	2.6	< 0.001
			1 st	36	7.5	3.7	-	-	-	-
		Cycle 2	2 nd	17	6.8	3.4	17	-1.0	2.2	p=0.109
			3 rd	18	4.9	3.6	18	-2.9	2.7	< 0.001
			4 th	24	2.9	3.4	23	-3.7	3.1	< 0.001
			1 st	24	6.1	3.8	-	-	-	-
		Cycle 3	2 nd	12	6.3	3.6	11	-1.4	2.5	p=0.102
			3 rd	16	3.8	3.3	15	-3.5	2.8	< 0.001
			4 th	16	2.8	2.5	14	-4.1	2.8	< 0.001
			1 st	16	7.8	4.2	-	-	-	-
Double-SN	MG-ADL total score	Cycle 1	2 nd	14	4.9	4.1	9	-1.2	1.9	p=0.188
			3 rd	14	4.3	3.8	11	-2.5	2.7	p=0.020
			4 th	13	3.2	2.4	11	-4.9	3.4	p=0.002
		Cycle 2	1 st	80	8.3	4.3	-	-	-	-
			2 nd	38	7.4	4.9	38	-1.4	2.3	< 0.001
			3 rd	47	6.2	4.9	47	-2.0	2.6	< 0.001
		Cycle 2	4 th	56	5.5	3.9	56	-2.8	2.8	< 0.001
			1 st	51	7.8	4.0	-	-	-	-
			2 nd	29	6.6	4.2	26	-1.2	1.9	p=0.004
			3 rd	28	6.0	4.3	25	-2.0	2.3	< 0.001

Analysis population	Item	Cycle	Administration period	Value			Change from the first administration in the cycle			
				N	Mean	SD	N	Mean	SD	Test ^a
			4 th	30	4.9	4.2	26	-2.4	2.7	< 0.001
		Cycle 3	1 st	34	8.4	3.9	-	-	-	-
			2 nd	18	6.7	3.9	15	-1.2	3.3	p=0.221
			3 rd	18	7.2	3.7	15	-0.7	1.9	p=0.199
			4 th	23	6.4	3.9	19	-2.0	3.4	p=0.022

Abbreviations: Ab = antibody, AChR = acetylcholine receptor, MG-ADL = Myasthenia Gravis-Activities of Daily Living, MuSK = muscle-specific kinase, N = number of patients, SD = standard deviation, SN = seronegative

a. Wilcoxon signed-rank sum test

Supplementary Table S4. Patient and disease characteristics by oral glucocorticoid dose after one year

Item	Classification	Overall		Oral glucocorticoid dose after one year $\leq 5\text{mg/day}$		Oral glucocorticoid dose after one year $> 5\text{mg/day}$		Test
		N / Summary	(%)	N / Summary	(%)	N / Summary	(%)	
		statistics		statistics		statistics		
		309	-	130	(42.1)	179	(57.9)	-
Sex	Male	96	(31.1)	35	(26.9)	61	(34.1)	$p = 0.171^a$
	Female	210	(68.0)	95	(73.1)	115	(64.3)	
	Not available	3	(1.0)	0	(0.0)	3	(1.7)	
Age (years)	N	214		93		121		$p = 0.587^b$
	Mean $\pm$ SD	48.5 $\pm$ 15.3		49.2 $\pm$ 16.3		48.0 $\pm$ 14.6		
	Median	49.5		51.0		46.0		
	Min - Max	18 - 91		18 - 84		18 - 91		
	< 18	0	(0.0)	0	(0.0)	0	(0.0)	$p = 0.115^c$
	$18 \leq < 65$	182	(58.9)	75	(57.7)	107	(59.8)	
	$65 \leq$	32	(10.4)	18	(13.9)	14	(7.8)	
	Not available	95	(30.7)	37	(28.5)	58	(32.4)	
Disease duration of gMG (years)	N	303		127		176		$p = 0.007^b$
	Mean $\pm$ SD	8.1 $\pm$ 8.8		6.5 $\pm$ 7.1		9.3 $\pm$ 9.7		
	Median	5.1		4.0		5.8		
	Min - Max	0.1 - 46.2		0.1 - 33.1		0.1 - 46.2		
Past/concomitant treatment for gMG	Oral glucocorticoid	300	(97.1)	122	(93.9)	178	(99.4)	-

	IVIg ^d	268	(86.7)	110	(84.6)	158	(88.3)	
	Cholinesterase inhibitor ^d	266	(86.1)	105	(80.8)	161	(89.9)	
	NSIST ^d	266	(86.1)	107	(82.3)	159	(88.8)	
	IVMP ^d	194	(62.8)	85	(65.4)	109	(60.9)	
	PLEX ^d	130	(42.1)	50	(38.5)	80	(44.7)	
	Thymectomy ^d	108	(35.0)	41	(31.5)	67	(37.4)	
	Eculizumab ^d	34	(11.0)	11	(8.5)	23	(12.9)	
	Oral immunosuppressive drugs (oral glucocorticoid / NSIST / cholinesterase inhibitor) only ^e	19	(6.2)	13	(10.0)	6	(3.4)	-
	Any of IVIg / IVMP / PLEX / Eculizumab ^e	290	(93.9)	117	(90.0)	173	(96.7)	
MGFA classification	IIa-IVa	245	(79.3)	112	(86.2)	133	(74.3)	p = 0.010 ^a
	IVb-V	62	(20.1)	17	(13.1)	45	(25.1)	
	Not available	2	(0.7)	1	(0.8)	1	(0.6)	
MG-ADL total score at the first administration	N	208		84		124		
	Mean ± SD	7.4 ± 3.8		6.8 ± 3.6		7.7 ± 3.9		p = 0.110 ^b
	Median	7.0		6.5		7.0		
	Min - Max	0 - 19		0 - 18		0 - 19		
Oral glucocorticoid	0	27	(8.7)	26	(20.0)	1	(0.6)	p < 0.001 ^c

dose at baseline (mg/day)						
0 < ≤ 5	68	(22.0)	66	(50.8)	2	(1.1)
5 < ≤ 10	106	(34.3)	23	(17.7)	83	(46.4)
10 <	108	(35.0)	15	(11.5)	93	(52.0)
Not available	0	(0.0)	0	(0.0)	0	(0.0)
Treatment	Mean ± SD	322.9 ± 54.5	323.1 ± 52.3	322.8 ± 56.2	p = 0.965 ^b	
duration with efgartigimod (days)						
	Median	360.0	360.0	360.0		
	Min - Max	184 - 463	190 - 360	184 - 463		

Abbreviations: Ab = antibody, AChR = acetylcholine receptor, BMI = body mass index, gMG = generalized myasthenia gravis, IVIg = intravenous immunoglobulin, IVMP = intravenous methylprednisolone, MG-ADL = Myasthenia Gravis-Activities of Daily Living, MGFA = Myasthenia Gravis Foundation of America, MuSK = muscle-specific kinase, N = number of patients, NSIST = nonsteroidal immunosuppressant therapy, PLEX = plasma exchange, SD = standard deviation, SN = seronegative

- Fisher's exact test
- Two sample t-test
- Wilcoxon rank sum test
- Duplicate count was allowed.
- Duplicate count was not allowed.

Supplementary Table S5. Oral glucocorticoid dosage description

	Overall	AChR-Ab+	MuSK-Ab+	Double-SN
N	206	118	27	58
Baseline (mg/day), Mean (SD)	10.7 (7.4)	11.1 (7.7)	13.3 (6.5)	8.9 (6.9)
At one year (mg/day), Mean (SD)	7.7 (5.4)	8.0 (5.6)	9.3 (5.0)	6.6 (5.0)
Difference (p-value ^a)	−3.0 (p < 0.001)	−3.1 (p < 0.001)	−4.0 (p < 0.001)	−2.3 (p < 0.001)

Abbreviations: Ab = antibody, AChR = acetylcholine receptor, MuSK = muscle-specific kinase, N = number of patients, SD = standard deviation, SN = seronegative

a. Wilcoxon signed-rank sum test

The 1-year assessment was defined as 12 months after efgartigimod initiation for patients treated for ≥ 12 months, and for those treated for <12 months, defined as the earliest of 120 days after the last efgartigimod administration or initiation of another targeted therapy as defined in the Methods.

Supplementary Table S6. MG-ADL total score in patients who had ≤ 5 mg/day or > 5 mg/day of oral glucocorticoid after one-year

Item	Cycle	Administration period	Oral glucocorticoid dose after one year							Oral glucocorticoid dose after one year						
			≤ 5 mg/day							> 5 mg/day						
			Value			Change from the first administration in the cycle				Value			Change from the first administration in the cycle			
			N	Mean	SD	N	Mean	SD	Test ^a	N	Mean	SD	N	Mean	SD	Test ^a
MG-ADL total score	Cycle 1	1 st	75	6.7	3.4	-	-	-	-	114	7.5	3.7	-	-	-	-
		At the best response	64	3.5	3.1	64	-3.2	2.7	< 0.001	83	4.5	3.6	83	-3.3	2.9	< 0.001
	Cycle 2	1 st	62	5.8	3.7	-	-	-	-	93	7.1	4.0	-	-	-	-
		At the best response	50	3.0	3.0	50	-2.8	2.8	< 0.001	67	4.6	4.1	67	-2.6	2.4	< 0.001
	Cycle 3	1 st	51	5.9	3.6	-	-	-	-	61	8.1	4.1	-	-	-	-
		At the best response	35	3.4	3.2	35	-2.0	2.5	< 0.001	48	4.6	4.2	48	-3.5	2.8	< 0.001

Abbreviations: MG-ADL = Myasthenia Gravis-Activities of Daily Living, N = number of patients, SD = standard deviation

a. Wilcoxon signed-rank sum test

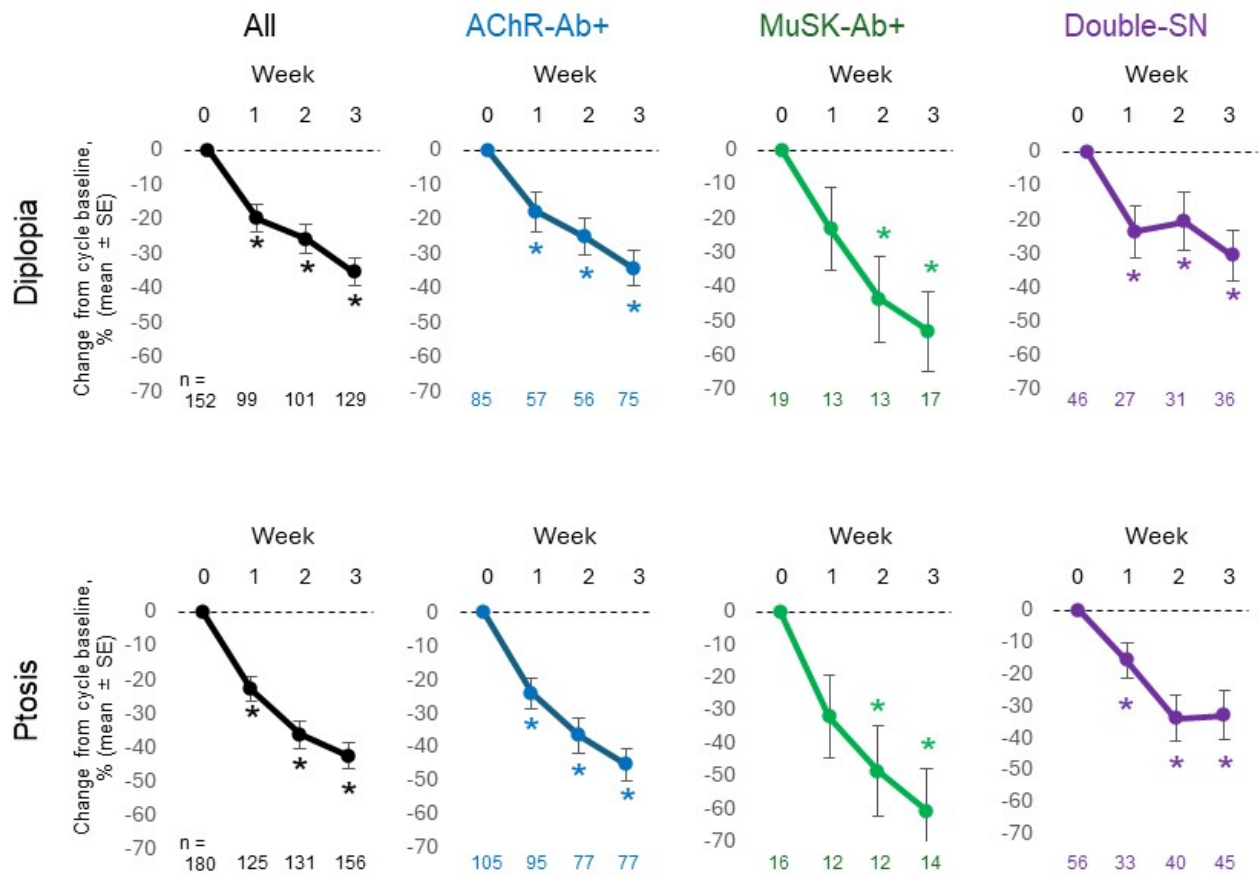
Supplementary Table S7. MG-ADL total score in patients who had ≤ 5 mg/day or > 5 mg/day of oral glucocorticoid after one-year by autoantibody subtype

Analysis population	Cycle	Administration period	Oral glucocorticoid dose after one year							Oral glucocorticoid dose after one year						
			≤ 5 mg/day							> 5 mg/day						
			Value			Change from the first administration in the cycle				Value			Change from the first administration in the cycle			
			N	Mean	SD	N	Mean	SD	Test ^a	N	Mean	SD	N	Mean	SD	Test ^a
AChR-Ab+	Cycle 1	1 st	38	6.1	3.3	-	-	-	-	71	7.2	3.9	-	-	-	-
		At the best response	33	3.3	3.3	33	-3.2	2.8	< 0.001	53	4.1	3.7	53	-3.5	3.1	< 0.001
	Cycle 2	1 st	30	5.5	3.6	-	-	-	-	56	6.7	4.0	-	-	-	-
		At the best response	27	2.7	2.8	27	-2.7	2.9	< 0.001	43	4.5	4.2	43	-2.4	2.2	< 0.001
	Cycle 3	1 st	27	5.1	3.0	-	-	-	-	38	7.4	4.2	-	-	-	-
		At the best response	18	3.2	3.4	18	-1.4	1.7	$p = 0.004$	30	4.1	4.4	30	-3.3	2.5	< 0.001
MuSK-Ab +	Cycle 1	1 st	8	7.0	3.1	-	-	-	-	16	7.6	3.8	-	-	-	-
		At the best response	5	3.6	3.8	5	-2.8	1.5	$p = 0.063$	10	3.0	3.8	10	-4.3	3.2	$p = 0.008$
	Cycle 2	1 st	8	4.9	3.2	-	-	-	-	14	6.4	4.2	-	-	-	-
		At the best response	4	3.3	2.5	4	-3.0	2.2	$p = 0.250$	11	2.2	2.8	11	-4.4	2.8	$p = 0.002$
	Cycle 3	1 st	6	6.5	3.8	-	-	-	-	8	8.4	5.0	-	-	-	-

		At the best response	4	3.5	2.4	4	-3.3	3.9	p = 0.125	7	2.7	2.7	7	-5.1	3.1	p = 0.016
Double-SN	Cycle 1	1 st	27	7.9	3.6	-	-	-	-	26	8.1	2.9	-	-	-	-
		At the best response	24	3.9	3.0	24	-3.4	2.9	< 0.001	19	6.2	2.9	19	-2.2	2.0	< 0.001
	Cycle 2	1 st	22	6.7	4.1	-	-	-	-	23	8.5	3.4	-	-	-	-
		At the best response	17	3.4	3.7	17	-3.0	3.1	< 0.001	13	7.1	3.5	13	-1.6	2.1	p = 0.027
	Cycle 3	1 st	16	7.1	4.3	-	-	-	-	14	10.0	2.9	-	-	-	-
		At the best response	11	4.1	3.6	11	-2.4	3.3	p = 0.031	10	7.7	3.3	10	-2.7	3.1	p = 0.047

Abbreviations: Ab = antibody, AChR = acetylcholine receptor, MG-ADL = Myasthenia Gravis-Activities of Daily Living, MuSK = muscle-specific kinase, N = number of patients, SD = standard deviation, SN = seronegative

a. Wilcoxon signed-rank sum test



Supplementary Figure S1. Percentage change from baseline in MG-ADL ocular subdomain scores during Cycle 1.

Abbreviations: Ab = antibody, AChR = acetylcholine receptor, MG-ADL = Myasthenia Gravis-Activities of Daily Living, MuSK = muscle-specific receptor kinase, n = number of patients, SE = standard error, SN = seronegative. *p-value < 0.05