

Association of earlier efgartigimod initiation with improved clinical outcomes in myasthenia gravis

Arjun Seth¹, Carolina Barnett-Tapia², Cynthia Qi³, Kristin Heerlein³, Rohit R Menon⁴, Jamie Aldridge³, Charan Atluri⁴, Helen Zhang⁵, Tobias Ruck⁶

¹Department of Neurology, Northwestern Medicine, Chicago, IL, USA; ²Department of Medicine, Division of Neurology, University of Toronto, Toronto, ON, Canada; ³argenx BVBA, Gent, Belgium; ⁴ZS Associates, Bengaluru, Karnataka, India; ⁵ZS Associates, New York, NY, USA; ⁶Department of Neurology with Heimer Institute for Muscle Research, University Hospital Bergmannsheil, Bochum, Nordrhein-Westfalen, Germany.

BACKGROUND

- Generalized myasthenia gravis (gMG) is a chronic autoimmune disease caused by pathogenic immunoglobulin G (IgG) autoantibodies disrupting neuromuscular transmission.¹
- Several targeted therapies, including the neonatal Fc receptor (FcRn) blocker efgartigimod, have been approved for gMG in the last decade. However, clinical research of these treatments has primarily focused on patients with longstanding disease and extensive prior treatment experience, limiting the evidence available for earlier treatment use.^{1,2}
- Efgartigimod has shown robust efficacy in gMG,^{3,4} with emerging clinical and real-world evidence suggesting improved clinical outcomes with earlier initiation of efgartigimod in the treatment paradigm.⁵⁻⁷
- Evidence remains limited regarding the impact of earlier efgartigimod initiation on outcomes in gMG, and “earlier” can be defined in multiple ways, including time from diagnosis and position in the treatment pathway.^{8,9}
- This study used claims data to evaluate outcomes across subgroups defined by time from diagnosis and prior treatment exposure to assess whether earlier efgartigimod initiation was associated with improved outcomes in gMG.

RESULTS

Patient characteristics

- Demographics and clinical characteristics of the overall cohort (N=1146) were consistent with expectations for a real-world MG population.
- Age and gender were comparable across all sub-cohorts, but significant differences emerged in other categories, notably insurance type, comorbidity burden (CCI), and baseline MG-ADL score (**Table 1**).

Table 1 Baseline demographics and characteristics

	Overall (n=1146)	By prior treatment exposure			By time from diagnosis		
		LoT ≤2 (n=335)	LoT ≥3 (n=811)	p-value ^c	<1 year (n=276)	1-3 years (n=306)	>3 years (n=564)
Age, mean (SD)	62.3 (15.2)	62.5 (15.3)	62.2 (15.1)	0.8	62.8 (14.6)	62.3 (14.7)	62 (15.7)
Gender, n (%)							
Female	533 (46.5)	155 (46.3)	378 (46.6)	0.9	114 (41.3)	143 (46.7)	276 (48.9)
Insurance type for first EFG claim, n (%) ^a							
Commercial	645 (56.3)	192 (57.3)	453 (55.9)	0.7	170 (61.6)	180 (58.8)	295 (52.3)
Medicare	470 (41.0)	132 (39.4)	338 (41.7)	0.5	95 (34.4)	118 (38.6)	257 (45.6)
Medicaid	52 (4.5)	>0, <20 ^d	34 (4.2)	0.4	>0, <20 ^d	>0, <20 ^d	22 (3.9)
CCI, mean (SD)	1.4 (1.9)	1.3 (1.7)	1.5 (2.0)	0.2	1.8 (2.3)	1.3 (2.0)	1.3 (1.6)
Common MG comorbidities, n (%) ^b							
Metabolic syndrome	718 (62.7)	209 (62.4)	509 (62.8)	0.9	188 (68.1)	178 (58.2)	352 (62.4)
Hyperlipidemia	422 (36.8)	126 (37.6)	296 (36.5)	0.7	122 (44.2)	101 (33.0)	199 (35.3)
Coronary artery diseases	139 (12.1)	46 (13.7)	93 (11.5)	0.3	48 (17.4)	31 (10.1)	60 (10.6)
Sleep disorder	316 (27.6)	69 (20.6)	247 (30.5)	<0.05	63 (22.8)	80 (26.1)	173 (30.7)
Malignancies	140 (12.2)	41 (12.2)	99 (12.2)	1.0	43 (15.6)	41 (13.4)	56 (9.9)
Baseline MG-ADL							
Available, n (%)	560 (48.9)	142 (42.4)	418 (51.5)		137 (49.6)	149 (48.7)	274 (48.6)
Mean (SD)	7.8 (3.8)	8.0 (3.6)	7.7 (3.9)	0.4	7.6 (3.8)	7.2 (4.0)	8.2 (3.7)
Time from initial Dx to EFG, months							
Mean (SD)	40.2 (29.3)	20.5 (23.9)	48.3 (27.4)	<0.05	5.6 (3.2)	23.1 (6.9)	66.4 (16.7)

^aPercentages may not add up to 100 due to patients tagged to multiple payer channels. ^bPercentages might not add up to 100 due to patients having multiple comorbidities. ^cFor statistical testing: t-test and one-way ANOVA were used for continuous variables, Chi-square tests for categorical variables and Fisher's exact test for categorical variables with values <5. ^dPatient counts greater than 0 but less than 20 have been masked.

Steroid dosing

- At 12 months post-efgartigimod initiation, 60-79% of patients across all sub-cohorts were steroid-free (0 mg/day), and the proportion receiving >10 mg/day declined in every sub-cohort (**Table 2**).
- Among patients receiving ≥1 mg/day at baseline, achievement of ≥75% average daily dose (ADD) reduction was significantly greater with earlier efgartigimod initiation, both by prior treatment lines (LoT ≤2, 60% vs LoT ≥3, 49%; p<0.05) and by time from diagnosis (59%, 52%, and 46% for <1 year, 1-3 years, and >3 years, respectively; overall p<0.05) (**Table 2**).

Table 2 Steroid dosing

	By prior treatment exposure			By time from diagnosis		
	LoT ≤2 (335)	LoT ≥3 (811)	p-value ^b	<1 year (276)	1-3 years (306)	>3 years (564)
Baseline ^a steroid distribution, mg/day						
0 mg	223 (66.6)	314 (38.7)	<0.05	122 (44.2)	139 (45.4)	276 (48.9)
>0 mg and ≤5 mg	28 (8.4)	114 (14.1)	<0.05	25 (9.1)	43 (14.1)	74 (13.1)
>5 mg and ≤10 mg	26 (7.8)	131 (16.2)	<0.05	29 (10.5)	39 (12.7)	89 (15.8)
>10 mg	58 (17.4)	252 (31.1)	<0.05	100 (36.3)	85 (27.8)	125 (22.2)
12M steroid distribution, mg/day						
0 mg	263 (78.5)	485 (59.8)	<0.05	184 (66.7)	201 (65.7)	363 (64.4)
>0 mg and ≤5 mg	>0, <20 ^d	77 (9.5)	<0.05	21 (7.6)	21 (6.9)	52 (9.2)
>5 mg and ≤10 mg	>0, <20 ^d	92 (11.3)	<0.05	20 (7.2)	27 (8.8)	56 (9.9)
>10 mg	44 (13.2)	157 (19.3)	<0.05	51 (18.5)	57 (18.7)	93 (16.5)
Patients with ≥1 mg/day dosing at baseline	106 (31.6)	483 (59.6)	<0.05	150 (54.3)	162 (52.9)	277 (49.1)
Any ADD reduction	81 (76.4)	362 (74.9)	0.8	118 (78.7)	123 (75.9)	202 (72.9)
Achieved ≥50% ADD reduction	70 (66.0)	273 (56.5)	0.1	99 (66.0)	92 (56.8)	152 (54.9)
Achieved ≥75% ADD reduction	64 (60.4)	235 (48.7)	<0.05	89 (59.3)	84 (51.9)	126 (45.5)

^aBaseline period is the 90-day period before efgartigimod initiation. ^bFor statistical testing: Chi-square tests were used to compare sub-cohorts. ^cPatient counts greater than 0 but less than 20 have been masked.

METHODS

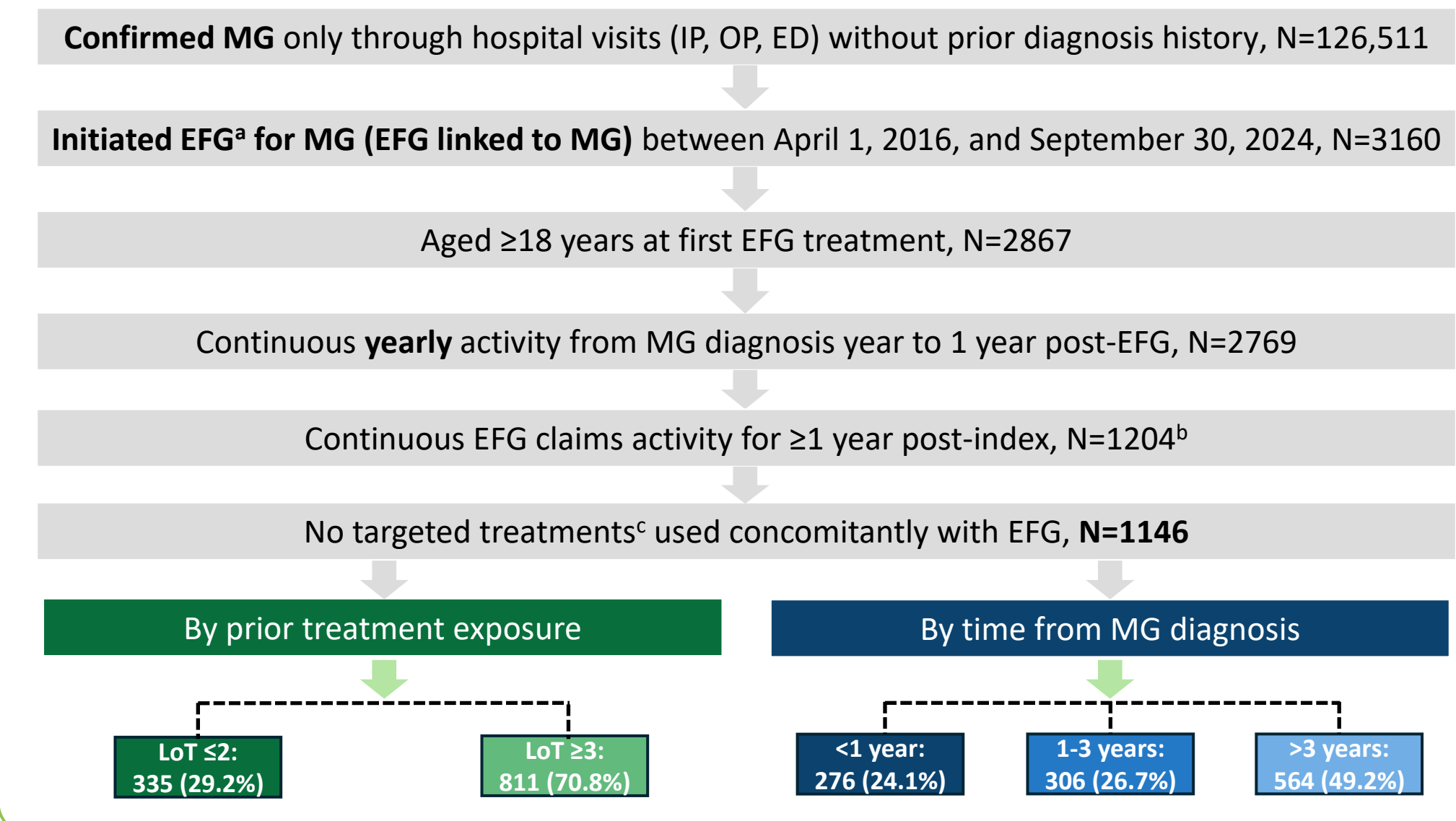
Database used

- Adults with confirmed MG (≥2 MG diagnoses separated by ≥30 days and within 2 years) receiving efgartigimod for at least 1 year were identified using United States medical and pharmacy claims data, based on information licensed from IQVIA: Longitudinal Access and Adjudication Data (LAAD) for the period April 2016-September 2025, reflecting estimates of real-world activity (all rights reserved).
- Myasthenia Gravis Activities of Daily Living (MG-ADL) scores were recorded for a subset of patients with linked MG-ADL scores from the My VVGART Path patient support program (PSP).

- Index date was defined as the first efgartigimod claim (**Figure 1**).

Patient selection

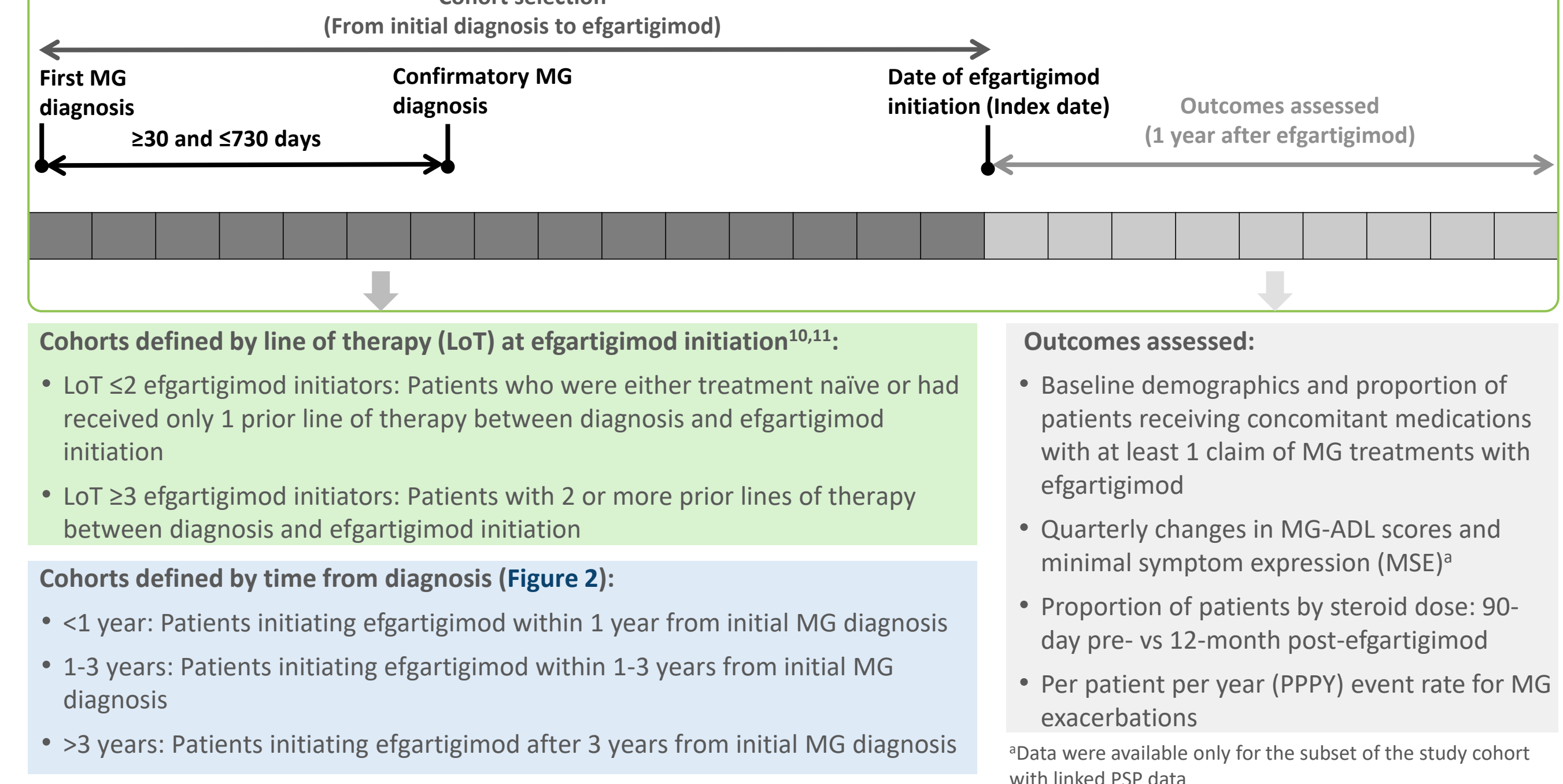
Figure 1 Patient selection



^aVVGART and VVGART Hytrulo claims were considered. ^bOpen claims data are subject to incomplete capture of healthcare encounters. To support reliable 1-year outcome assessment, analyses were restricted to patients with continuously observable efgartigimod claims. This criterion reflected data completeness requirements for outcome measurement and was not an assessment of treatment persistence. ^cEculizumab, rituximab, ravulizumab, zilucoplan, rozanolixumab.

Study design

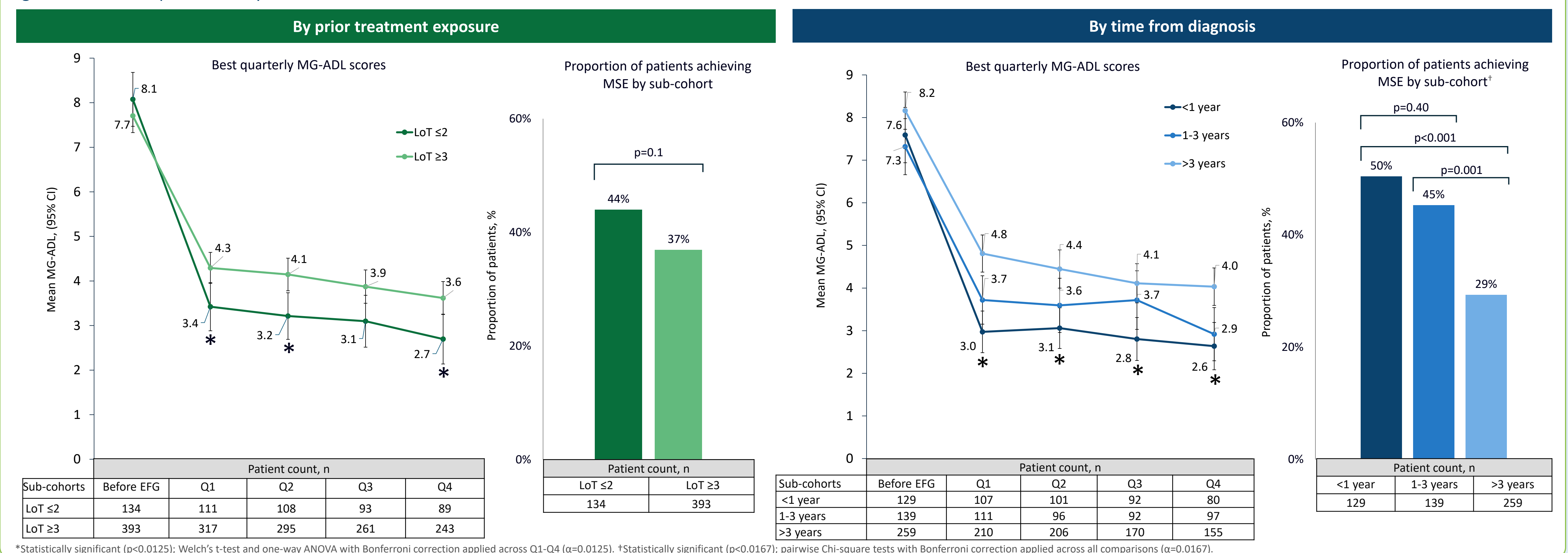
Figure 2 Study design



MG-ADL and MSE outcomes after efgartigimod initiation

- Rapid and substantial MG-ADL improvement was observed across all sub-cohorts after efgartigimod initiation, with the largest reductions among patients treated earlier in their disease course (LoT ≤2: 8.1 to 2.7; <1 year: 7.6 to 2.6). These improvements continued to deepen through Q4 and were significantly greater than those observed in other sub-cohorts within each cohort (p<0.0125) (**Figure 3**).
- Meaningful rates of MSE were observed across the overall population, with higher achievement among earlier treatment cohorts (LoT ≤2: 44%; <1 year: 50%). MSE rates were significantly higher among patients initiating efgartigimod within <1 year and 1-3 years of diagnosis compared to >3 years after diagnosis (p<0.001 and p=0.001, respectively) (**Figure 3**).

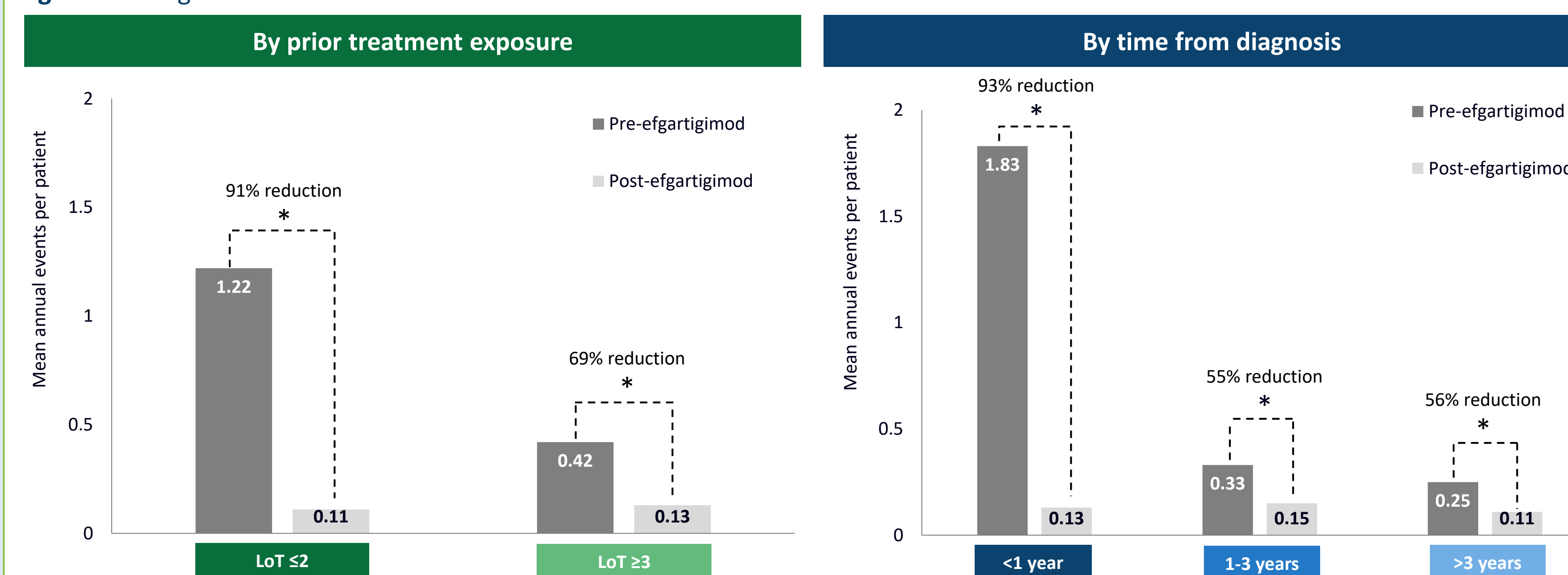
Figure 3 MG-ADL improvement by time and MSE rate



MG-related exacerbations

- Significant reductions in MG exacerbations were observed across all sub-cohorts following efgartigimod initiation, with the greatest relative reductions observed among patients treated earlier in their disease course (LoT ≤2: 91%; <1 year: 93%) (**Figure 4**).

Figure 4 Change in MG exacerbation^a event rates^b



LIMITATIONS

- MG-ADL scores were available only for the PSP-linked subset, were patient self-reported, and were inconsistently captured over time, limiting longitudinal assessment and potentially introducing bias due to missing follow-up data.
- Selection criteria for an analyzable cohort may have introduced bias toward patients with longer treatment experience, and incomplete capture of prior care may have resulted in misclassification of lines of therapy and diagnosis dates, potentially reducing observed differences between sub-cohorts.
- Outcomes may have been influenced by baseline differences between sub-cohorts, including disease duration, disease severity, and prior treatment history. Analyses were not adjusted for potential confounders, and patients initiating efgartigimod later in their disease course may have had a lower likelihood of response due to prior treatment experience (indication bias); therefore, findings should be interpreted as correlational rather than causal.

KEY TAKEAWAYS

Approximately one-third of efgartigimod initiators started treatment relatively early in their gMG disease course, reflecting real-world use of efgartigimod earlier in the treatment journey among a subset of patients.

Improvements in MG-ADL scores, steroid burden, and exacerbation rates were observed across all sub-cohorts and were greatest among patients initiating earlier, whether defined by fewer prior treatment lines or shorter time from diagnosis.

These findings are consistent with an evolving gMG treatment paradigm that emphasizes earlier intervention and defined treatment goals, such as minimal symptom expression and steroid reduction, rather than reactive maintenance.

ABBREVIATIONS

ADD, average daily dose; CCI, Charlson Comorbidity Index; Dx, diagnosis; ED, emergency department; EFG, efgartigimod; FcRn, neonatal Fc receptor; gMG, generalized myasthenia gravis; IgG, immunoglobulin G; IP, inpatient; LoT, line of therapy; MG, myasthenia gravis; MG-ADL, Myasthenia Gravis Activities of Daily Living; MSE, minimal symptom expression; OP, outpatient; PPPY, per patient per year; PSP, patient support program; Q, quarter; SD, standard deviation.

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