Hospitalization Outcomes After Efgartigimod Initiation in Patients With Myasthenia Gravis

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INTRODUCTION

Myasthenia gravis (MG) and efgartigimod

- MG is a rare, antibody-mediated neuromuscular disorder leading to a failure of neuromuscular junction (NMJ) transmission, characterized by fluctuating weakness in ocular, facial, bulbar, axial, and limb muscles.[1–3] The majority of patients (~85%) have autoantibodies against the acetylcholine receptor (AChR).[3]
- Efgartigimod is a human immunoglobulin G1 (IgG1) Fc fragment engineered to bind to FcRn on endothelial cells, leading to increased degradation of IgG (including pathological IgG) in the lysosome.[2] It was approved for the treatment of anti-AChR antibody–positive MG in December 2021[2,4] and is typically dosed with 4 once-weekly infusions, with subsequent cycles administered according to individualized response.[5]

Efgartigimod impact on MG-related clinical events

- Based on phase 3 ADAPT trial data, efgartigimod treatment significantly reduced the risk of exacerbations and numerically lowered all-cause and MG-related hospitalizations.[6]
- Reduction in these MG-related clinical events can be interpreted as one of the consequences of improvement in MG symptom control by efgartigimod.
- While similar results are expected to be observed in clinical practice, no such evidence is currently available.

Objective

- The objective of this study was to utilize a large dataset based on US claims to assess changes in MG-related exacerbations and crises before and after efgartigimod initiation.

METHODS

Study type and dataset

- A retrospective cohort study was conducted using US medical and pharmacy claims (based on information licensed from IQVIA: Longitudinal Access and Adjudication Data for the period April 2016–January 2024, reflecting estimates of real-world activity [all rights reserved]).
- MG-activities of daily living (MG-ADL) scores obtained in the My VYVGART Path, a patient support program, were integrated with the primary dataset. No identifiable patient data were obtained by the investigators.

Inclusion/exclusion criteria

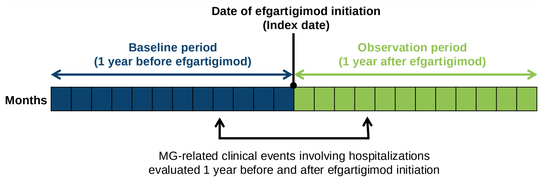
- Adults with MG with the first efgartigimod claim (index) January 1–December 31, 2022, with at least 1 year of ongoing efgartigimod usage based on claims captured[a]; continuous quarterly claims activity[b] with no claim for eculizumab, rituximab, ravulizumab, rozanolixizumab, or zilucoplan during the observation period.

[a]Patients with a gap of >120 days between consecutive efgartigimod claims were excluded. [b]Continuous quarterly activity was defined as ≥1 record in the database every quarter from 1 year pre-efgartigimod to 1 year post-efgartigimod initiation.

Outcomes

- MG-related exacerbations were defined as inpatient encounters with ICD-10 codes for MG with exacerbation. MG-related crises were defined as intensive care unit (ICU) admissions with ICD-10 codes for acute respiratory failure and MG. All acute care utilization was evaluated during the 1 year before and 1 year after efgartigimod initiation (Figure 1).
- For the study cohort with both baseline and at least 1 follow-up MG-ADL captured, baseline score (≤90 days before efgartigimod initiation) was compared with follow-up scores captured at consecutive 3-month intervals after efgartigimod initiation.

Figure 1. Study design



SUMMARY

Based on recent data from 439 patients with MG treated with efgartigimod in clinical practice, substantial reductions in exacerbations (68%), crises (71%), and MG-specific hospitalizations (63%) were observed compared to the year before efgartigimod initiation.

- These results provide direct evidence that efgartigimod reduces disease burden and could potentially offset medical costs among patients with MG.

Some limitations should be noted, including that claims-based data analyses are subject to assumptions, potential coding errors, and risk of missing data. Detailed insights into individual treatment combinations were not assessed due to limited cohort sizes; this requires further accumulation of data of patients continuing efgartigimod treatment for ≥1 year. Some potential selection bias for responders should be considered as this analysis was restricted to patients receiving continuous efgartigimod for 1 year.

Despite the limitations, this study enabled inclusion of a large sample size, with results supporting reduction of acute MG-related events with efgartigimod. Future studies should further evaluate longer-term outcomes following efgartigimod initiation in clinical practice using additional datasets.

RESULTS

Patient cohort selection, baseline demographics and characteristics, and descriptive analysis of MG treatment utilization

- A total of 439 patients fulfilled the criteria and were included in the analysis (Figure 2).
- Mean (SD) age was 60.6 (14.6) years and 46% (n=200/439) of patients were female, consistent with typical claims-based MG cohorts. Comorbidities, such as hypertension, were common in this population of efgartigimod users (Table 1).
- Descriptively, the proportion of patients using non-efgartigimod MG treatments trended downward after efgartigimod, with a pronounced reduction in Ig and PLEX use (Table 2).

MG-related clinical events and hospitalizations before and after efgartigimod initiation

- Mean annual MG-related clinical events (exacerbations and crises) per patient were significantly reduced by 68% and 71%, respectively, after efgartigimod initiation (Figure 3).
- Similarly, MG-related acute care utilization (hospitalizations and ICU visits) was also reduced (Figure 4).
- Mean annual all-cause hospitalizations per patient were similarly reduced (0.64 before and 0.31 after efgartigimod [P<0.05]).
- Mean length of stay and annual emergency visits were numerically reduced after efgartigimod, though not statistically significant.

MG-ADL scores before and after efgartigimod initiation

- Among 190 patients (43%) with MG-ADL scores available, mean (SD) best follow-up scores were significantly reduced after index, with a 4-point reduction observed by Q4 (8.0 [3.7] to 3.7 [2.8], P<0.05) (Figure 5).

Figure 2. Patient selection

Adults (≥18 years of age) with first efgartigimod claim between January 1 and December 31, 2022
n=1385 (100%)

↓

Prior MG diagnosis and continuous quarterly activity across baseline and observation period
n=1113 (80%)

↓

Continuous efgartigimod treatment over observation period
n=462 (42%)

↓

No concurrent usage of targeted gMG therapies other than efgartigimod in observation period (prior use allowed)[a]
n=439 (95%)

[a]Targeted gMG therapies include eculizumab, rituximab, ravulizumab, rozanolixizumab, and zilucoplan. Usage of these therapies was allowed before efgartigimod, but patients with usage of these therapies concurrently with efgartigimod during the observation period were excluded.

Table 1. Baseline demographics and characteristics

	N=439
Age, years	
Mean (SD)	60.6 (14.6)
Median (IQR)	64.0 (51.0–72.0)
Gender, n (%)	
Female	200 (45.6)
Charlson Comorbidity Index (CCI)	
Mean (SD)	1.3 (1.8)
Common MG comorbidities, n (%)[a]	
Hypertension	207 (47.2)
Diabetes	124 (28.2)
Obesity	106 (24.1)
Hyperlipidemia	131 (29.8)
Thyroid-related disorders	65 (14.8)
GERD	69 (15.7)
Sleep disorders	117 (26.7)
Depression	50 (11.4)
Insurance type for first efgartigimod claim, n (%)[b]	
Commercial	242 (55.1)
Medicare	183 (41.7)
Medicaid other/unknown	22 (5.0)
MG-ADL score, n (%)[c]	
Unavailable	239 (54.4)
Available	200 (45.6)
0–1	>0, <20
2–4	30 (15.0)
5–9	94 (47.0)
10–24	71 (35.5)

Note: Patient counts greater than 0 but less than 20 have been masked. [a]Percentages may not add up to 100% since patients can have multiple comorbidities. [b]Percentages may not add up to 100% as patients may be tagged to multiple payer channels. [c]Most recent score captured pre-efgartigimod initiation was considered. Mean (SD) of baseline MG-ADL capture was 36 (46.0) days pre-efgartigimod initiation.

Table 2. MG treatment utilization before and after efgartigimod

Patients with at least 1 claim for non-efgartigimod MG-related treatments, n (%)	N=439	
	1 year before efgartigimod	1 year after efgartigimod
Overall	420 (95.7)	380 (86.6)
AChE inhibitors	312 (71.1)	293 (66.7)
Oral glucocorticoids	308 (70.2)	289 (65.8)
NSISTs[a]	201 (45.8)	193 (44.0)
Mycophenolate mofetil	122 (27.8)	121 (27.6)
Azathioprine	72 (16.4)	64 (14.6)
Methotrexate	>0, <20	>0, <20
Tacrolimus	>0, <20	>0, <20
Cyclosporine	>0, <20	>0, <20
Cyclophosphamide	>0, <20	0
Targeted therapies	68 (15.5)	-
Eculizumab	56 (12.8)	-
Rituximab	>0, <20	-
Ravulizumab	>0, <20	-
IVIg/SCIg	166 (37.8)	25 (5.7)
PLEX	35 (8.0)	>0, <20

Note: Patient counts greater than 0 but less than 20 have been masked.
[a]Percentages may not add up since patients might be using multiple NSISTs/biologics.

Figure 3. MG-related clinical events before and after efgartigimod

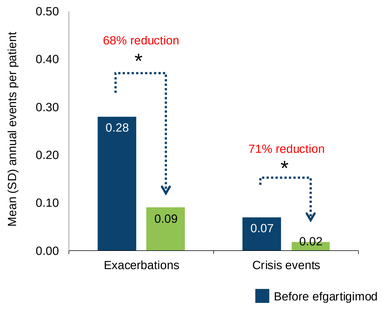
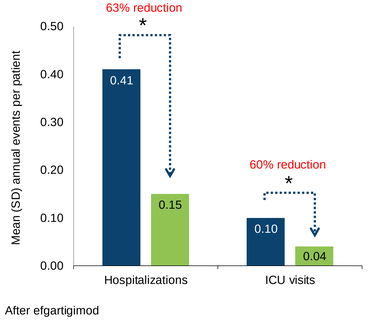
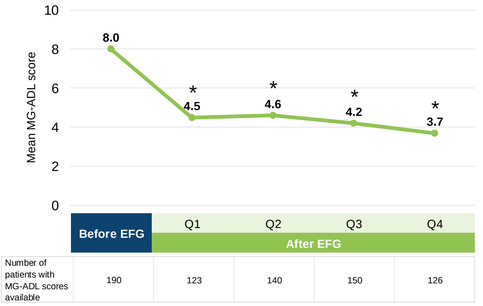


Figure 4. MG-related acute care before and after efgartigimod



P-values were calculated using t-tests. P<0.05 (denoted by) was considered statistically significant.

Figure 5. MG-ADL scores before and after efgartigimod



Number of patients with MG-ADL scores available	190	123	140	150	126

Note: A subset of patients with MG-ADL scores available in the integrated dataset were included in the analysis. Any (or best) available MG-ADL score captured during the following quarters post efgartigimod initiation was used. *P-values were calculated using t-tests. P<0.05 (denoted by *) was considered statistically significant.

ABBREVIATIONS: AChE, acetylcholinesterase; AChR, acetylcholine receptor; ADL, activities of daily living; EFG, efgartigimod; Fc, fragment crystallizable region; FcRn, neonatal Fc receptor; GERD, gastroesophageal reflux disease; gMG, generalized MG; ICU, intensive care unit; Ig, immunoglobulin; IgG, immunoglobulin G; IQR, interquartile range; IVIg/SCIg, intravenous or subcutaneous immunoglobulin; MG, myasthenia gravis; MG-ADL, Myasthenia Gravis Activities of Daily Living; NMJ, neuromuscular junction; NSIST, nonsteroidal immunosuppressive treatment; PLEX, plasma exchange; SD, standard deviation; US, United States.

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REFERENCES: **1.** Gilhus NE, et al. *Nat Rev Dis Primers*. 2019;5(1):30. **2.** Howard JF Jr, et al. *Lancet Neurol*. 2021;20(7):526–536. **3.** Gilhus NE, Verschuuren JJ. *Lancet Neurol*. 2015;14(10):1023–1036. **4.** US Food and Drug Administration. News Release. Accessed March 6, 2025. https://www.fda.gov/news-events/press-announcements/fda-approves-new-treatment-myasthenia-gravis **5.** argenx BV. VYVGART (efgartigimod alfa-fcab) [package insert]. **6.** Qi CZ, et al. Poster presented at: ISPOR Annual Meeting. 2022.

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