70

Patient characteristics, dosing patterns, and outcomes associated with intravenous and subcutaneous efgartigimod among patients with generalized myasthenia gravis in clinical practice

Cynthia Qi,[1] Ratna Bhavaraju-Sanka,[2] A. Gordon Smith,[3] Rohit R. Menon,[4] Helen Zhang,[5] Mai Sato,[5] Gil I. Wolfe[6]

[1]argenx US Inc., Boston, MA, USA; [2]Department of Neurology, University of Texas Health Science Center at San Antonio, San Antonio, TX, USA; [3]Department of Neurology, Virginia Commonwealth University, Richmond, VA, USA; [4]ZS Associates, Bengaluru, Karnataka, India; [5]ZS Associates, New York, NY, USA; [6]Department of Neurology, Jacobs School of Medicine and Biomedical Sciences, University at Buffalo, Buffalo, NY, USA

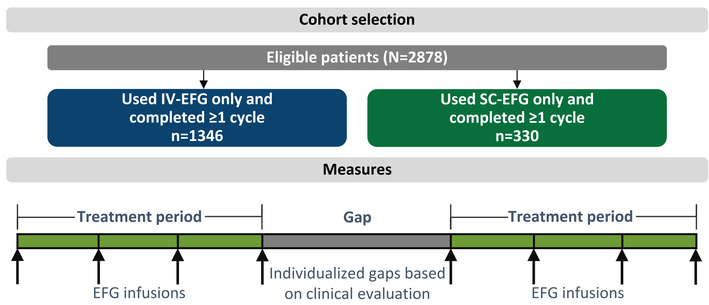
BACKGROUND | METHODS

- On December 17, 2021, the US Food and Drug Administration (FDA) approved intravenous (IV) efgartigimod (EFG) for the treatment of anti-acetylcholine receptor antibody-positive generalized myasthenia gravis (gMG).[1] Subsequently, the subcutaneous (SC) formulation received FDA approval on June 20, 2023.[2] A pre-filled syringe (PFS) for SC-EFG was recently approved on April 10, 2025.
- The SC formulation complements IV-EFG by offering more flexibility and another option for patients. It allows for faster administration compared to IV, including the possibility of self-administration with the PFS.[3]
- As IV-EFG has been available for longer, most published evidence for EFG has primarily focused on the IV formulation.[4,5] This highlights a gap in evidence related to the SC formulation in US clinical practice.
- This study assessed patient characteristics, dosing patterns, and outcomes among individuals with gMG using IV- or SC-EFG in the US, utilizing the largest dataset of EFG users to date from the My VYVGART Path patient support program (PSP).

Dataset
- Data were collected from the My VYVGART Path PSP, which captures patients initiating EFG since January 2022.

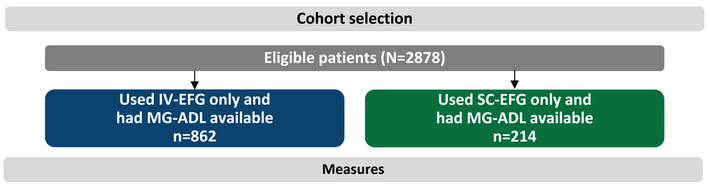
Eligibility
- Enrolled adults (aged ≥18 years) with gMG who had initiated EFG on or after August 1, 2023 (SC-EFG approval: June 20, 2023).
- Selection period: August 1, 2023, to December 31, 2024.

Dosing pattern analysis



- Mean gaps between cycles (in weeks) and mean annualized number of cycles (in weeks, calculated as 3 weeks plus mean gap between cycles, divided by 52 weeks) were measured among patients who completed at least 1 cycle.

MG-ADL analysis



- **Best achieved disease state** at any time after EFG initiation was assessed in 2 ways. (1) Largest observed response was defined as the difference between each patient's baseline and lowest Myasthenia Gravis–Activities of Daily Living (MG-ADL) score captured at any time point after EFG initiation. (2) The distribution of lowest achieved MG-ADL score was presented according to clinical severity thresholds (e.g., MG-ADL 0–4 [mild disease]).
- **MG-ADL improvement over time** was evaluated at the population level, as MG-ADL scores were not captured at discrete timepoints. All available MG-ADL scores captured in each month after EFG initiation (up to 6 months) were converted into difference from patient-matched baseline scores and averaged.

RESULTS

Overall patient characteristics

- Of the total 2878 patients who initiated EFG, 1992 (69%) patients received IV-EFG only and 622 (22%) received SC-EFG only throughout their follow-up period.
- Baseline characteristics were similar between IV-EFG and SC-EFG cohorts (**Table 1**).
- Over half (55%) of SC-EFG users initiated EFG in the second half of 2024, reflecting a gradual and more recent increase in uptake of SC-EFG among new initiators, owing to its more recent approval (**Table 1**).

TABLE 1 Baseline characteristics

	IV-EFG (n=1992)	SC-EFG (n=622)
Age		
Mean age, years (SD)	65.8 (15.4)	67.0 (15.3)
≥65 years	1278 (64.2)	411 (66.1)
Sex, n (%)		
Male	1120 (56.2)	343 (55.1)
Female	868 (43.6)	279 (44.9)
Insurance,* n (%)		
Commercial	643 (32.3)	194 (31.2)
Government	1389 (69.7)	435 (69.9)
Other	47 (2.4)	10 (1.6)
Prior treatment, n (%)*†		
AChE inhibitors	709 (53.5)	277 (66.4)
Any NSIST	209 (15.8)	54 (12.9)
Any steroid	725 (54.8)	253 (60.7)
Any advanced therapy	786 (59.4)	235 (56.4)
Timing of EFG initiation, n (%)		
H2 2023	746 (37.4)	84 (13.5)
H1 2024	667 (33.5)	195 (31.4)
H2 2024	579 (29.1)	343 (55.1)
No. of cycles completed, n (%)		
1	416 (21)	131 (21)
2	308 (16)	70 (11)
3	208 (10)	62 (10)
4+	414 (21)	67 (11)
Baseline MG-ADL score‡		
Mean (SD)	8.1 (3.7)	7.6 (3.4)
Median (IQR)	8.0 (5.0, 11.0)	8.0 (5.0, 9.8)
Distribution, n (%)		
0–4 points	146 (17.0)	40 (18.7)
5–9 points	411 (47.7)	120 (56.1)
10–24 points	305 (35.4)	54 (25.2)

*Percentages might not add up to 100 since patients can be tagged to multiple payers or report multiple class of treatments.
†Only a subset of patients had prior treatment information captured (1324 [67%] in IV-EFG cohort; 417 [67%] in SC-EFG cohort). Since treatment information is no longer captured in the PSP, recent patients might not have any treatment information available.
‡Most recent score in the baseline period was considered as baseline score. Only a subset of patients were considered who had baseline (before EFG initiation) scores and at least 4 MG-ADL scores captured after EFG initiation (862 [43%] in IV-EFG cohort; 214 [34%] in SC-EFG cohort).

Dosing patterns

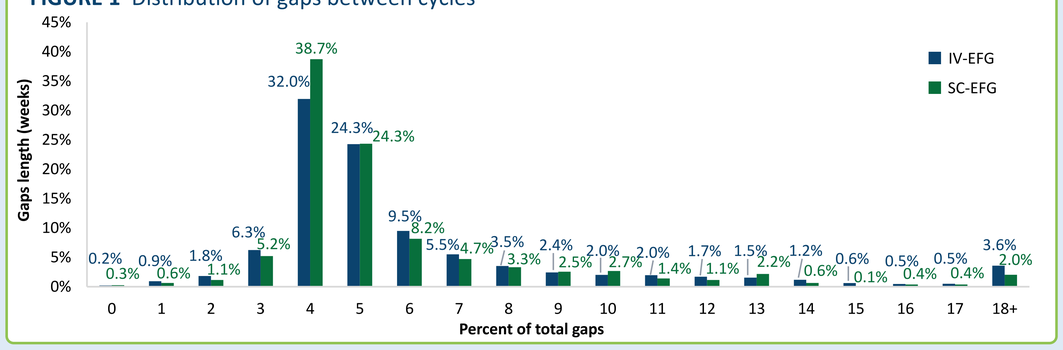
- The mean gaps between cycles (7 weeks) were similar between both formulations, with an average annualized utilization of 5.2 cycles across both groups (**Table 2**).
- The most common gap between cycles was 4 weeks for both IV-EFG and SC-EFG users (**Figure 1**).

TABLE 2 Dosing patterns

	IV-EFG (n=1346)	SC-EFG (n=330)
Mean gap, weeks (range)	7 (0–56)	7 (0–39)
Estimated average annualized* EFG utilization, by number of cycles	5.2	5.2

*Annualized cycles were calculated by dividing weeks in a year (52) by gap between cycles and mean treatment period length.

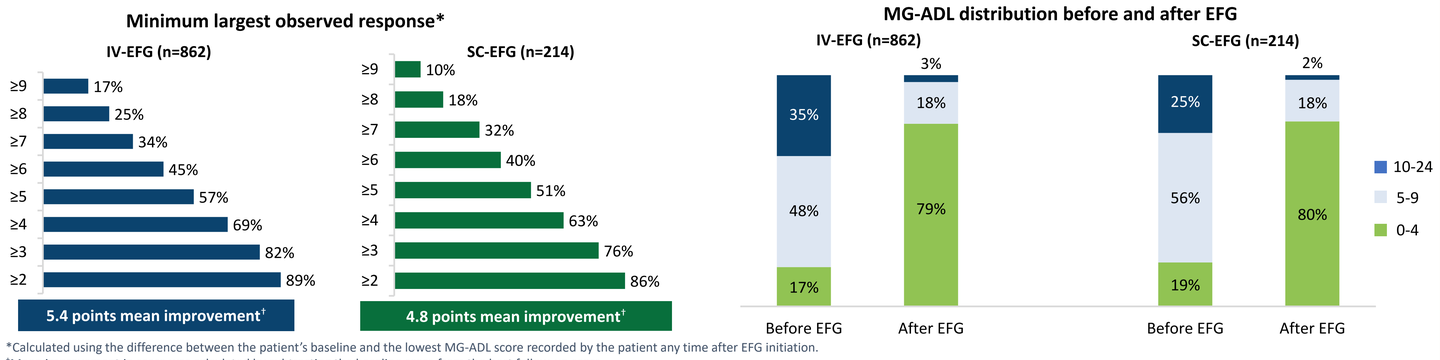
FIGURE 1 Distribution of gaps between cycles



MG-ADL analysis: Best achieved disease state

- MG-ADL improvements were similar between IV and SC users, with mean reduction of 5.4 and 4.8 points from baseline, respectively; nearly 80% of patients achieved MG-ADL scores ≤4 (indicates mild disease state) following EFG initiation (**Figure 2**).

FIGURE 2 Best achieved disease state after EFG initiation

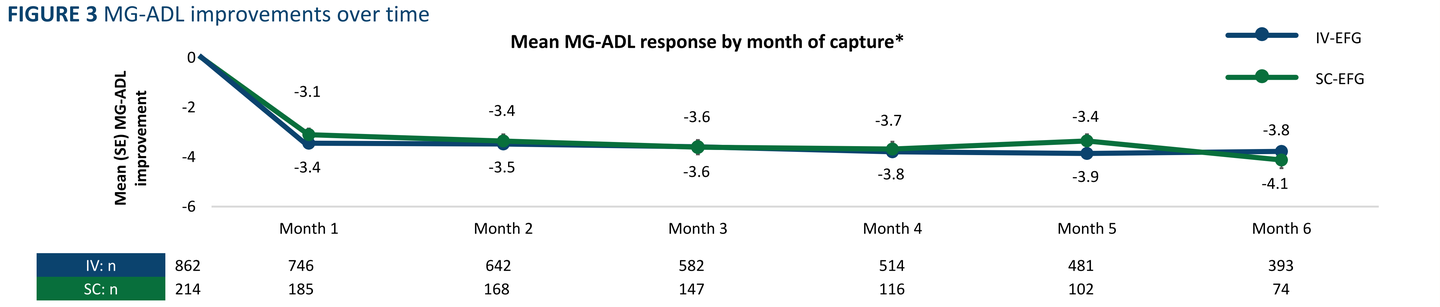


*Calculated using the difference between the patient's baseline and the lowest MG-ADL score recorded by the patient any time after EFG initiation.
†Mean improvement in score was calculated by subtracting the baseline score from the best follow-up score.

MG-ADL analysis: Improvements over time

- MG-ADL improvements over time were similar and sustained in both groups (**Figure 3**).

FIGURE 3 MG-ADL improvements over time



*Calculated as the difference between the baseline score and the lowest (best) MG-ADL score recorded during the specific month after EFG initiation. n represents patients who had any MG-ADL score captured in the month.

KEY TAKEAWAYS

- Analysis of a large cohort of EFG users in the US demonstrated similar clinical benefit between IV-EFG and SC-EFG.
- Most patients achieved rapid, substantial, and sustained clinical improvements regardless of EFG formulation.
- The observed annual utilization patterns of IV-EFG and SC-EFG were similar, with 4 weeks as the most common gap between cycles.
- Similar dosing patterns suggest that physicians are applying comparable individualized dosing strategies irrespective of the administration route.

LIMITATIONS

- Data were obtained as part of a PSP, resulting in missing capture of detailed clinical variables that may uncover additional insights.
- Follow-up availability was limited owing to the relatively recent approval of SC-EFG; further data collection is warranted for additional analyses.

PRESENTED AT THE AMERICAN ASSOCIATION OF NEUROMUSCULAR AND ELECTRODIAGNOSTIC MEDICINE (AANEM) ANNUAL MEETING; OCTOBER 29–NOVEMBER 1, 2025; SAN FRANCISCO, CALIFORNIA, UNITED STATES

ABBREVIATIONS

AChE: acetylcholinesterase; EFG: efgartigimod; gMG: generalized myasthenia gravis; IgG: immunoglobulin G; IV: intravenous; IQR: interquartile range; FDA: US Food and Drug Administration; MG-ADL: Myasthenia Gravis–Activities of Daily Living; NSIST: nonsteroidal immunosuppressive treatment; PSP: patient support program; PFS: pre-filled syringe; Q: quarter; SC: subcutaneous; SD: standard deviation; SE: standard error; US: United States.

DISCLOSURES

This study was funded by argenx US, Inc. The authors thank Ahmed Elgebaly (SIRO ClinPharm Pvt. Ltd. UK) for medical writing support.

ACKNOWLEDGMENTS

CQ is an employee of argenx; **RB-S** has served on advisory boards for argenx and is a consultant to Amgen; **AGS** has served as a member of advisory boards or paid consultant for Alexion, Arcellx, argenx, Eidos, Lexicon, Lilly, Seismic, and UCB; **RRM**, **HZ**, and **MS** are employees of ZS Associates and served as paid consultants for argenx; **GIW** has served as member of advisory boards or provided paid consultations to Alexion, argenx, Cartesian, Janssen, and UCB, is on speaker bureaus for Alexion and UCB, and has received research support from Alexion, argenx, Immunovant, Roche, UCB, and the MG Foundation of America.

REFERENCES

1. VYVGART® (efgartigimod alfa-fcab) Prescribing Information. US Food and Drug Administration. Updated August 2024. Accessed September 14, 2025.
2. VYVGART® HYTRULO (efgartigimod alfa and hyaluronidase-qvfc) Prescribing Information. US Food and Drug Administration. Updated June 2024. Accessed September 14, 2025.
3. Howard Jr et al. *Neurotherapeutics*. 2024;21(5):e00378.
4. Silvestri NJ. *Muscle Nerve*. 2025;71(3):422–428.
5. Singer M et al. *Muscle Nerve*. 2024;69(1):87–92.

SCAN ME