MG9

Patterns Of Efgartigimod Dosing In Clinical Practice In The United States

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INTRODUCTION AND OBJECTIVES

- Efgartigimod is a human IgG1 Fc fragment that is a natural ligand of the neonatal Fc receptor (FcRn); it is engineered to have increased affinity for FcRn and outcompete endogenous IgG.[1] It was approved in the United States (US) for treatment of generalized myasthenia gravis (gMG) in December 2021.[1-3]
- gMG, a rare autoimmune disorder affecting ~60,000 patients in the United States (US),[4,5] is fundamentally heterogeneous in both its presentation and its disease course. To enable custom treatment plans based on varying patient needs, individualized dosing based on clinical response is recommended for efgartigimod.[1,3]
- Previously, we reported that for patients initiating efgartigimod in the real world, the mean time between the last infusion of Cycle 1 and the start of Cycle 2 was 50.7 days (as of September 15, 2022).[6] These early real-world estimates were consistent with results reported in the ADAPT phase 3 trial (median of 7 weeks).[1]
- Now that prescribers may have become more accustomed to individualized dosing, the objective of this study was to evaluate updated real-world dosing patterns of efgartigimod in clinical practice in the US. We report our findings up to September 26, 2023.

METHODS

Dataset description

- Data were obtained from My VYVGART Path, a patient support program (PSP), which started data collection in Q1 of 2022 after efgartigimod launch in the US. Through regular phone contact, the PSP captures patient and efgartigimod dosing information of those enrolled.

Inclusion criteria

- Patients (≥18 years of age) enrolled in the PSP who had initiated efgartigimod by September 26, 2023.

Cohort selection and analyses

- Two cohorts were selected. Dosing patterns were assessed for both cohorts (Figure 1), and treatment cadence was assessed for Cohort B only.

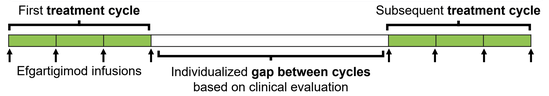
Cohort A:
Patients who had initiated at least Cycle 2* to assess overall dosing patterns

Cohort B:
Patients who had initiated at least Cycle 6* to evaluate longer-term utilization patterns

Dosing pattern analysis (cohorts A and B)

Treatment cycles were defined as time from the first to last efgartigimod infusion administered 1 week apart (Figure 1). Gaps between cycles were defined as last infusion of the previous cycle to the first infusion of the subsequent cycle. All periods were defined as weeks, from 0 weeks (0-<7 days), 1 week (7-<14 days), etc.

Figure 1. Definition of treatment cycles and gap between treatment cycles.



Cadence analysis (cohort B only)

Fixed dosing was defined as patients with more than two-thirds of their gaps ±7 days of their median gap length (at the patient level) from Cycle 3 onward. Those who did not fulfill this criterion were considered as having **flexible dosing.**

*To provide a comprehensive representation, all distinct measurable gaps between treatment cycles available were included in the analyses, including patients who discontinued efgartigimod. To avoid potential bias introduced from discontinuation, only gaps prior to the cycle that patients discontinued were included in the analyses.

RESULTS

1. Study cohorts and baseline characteristics

Table 1. Baseline patient demographics and characteristics

	Cohort A (n=2616)	Cohort B (n=490)
Duration on efgartigimod, days		
Mean (SD)	270.7 (133.7)	422.5 (79.4)
Median (IQR)	259 (156-375)	429 (364.3-484.8)
Age/gender		
Mean age (SD), years	65.4 (15.3)	64.9 (14.9)
18 to <65 years, n (%)	996 (38.1)	198 (40.4)
≥65 years, n (%)	1620 (61.9)	292 (59.6)
Female, n (%)	1121 (42.9)	196 (40.0)
Insurance, n (%)*		
Commercial	785 (30.0)	155 (31.6)
Government	1734 (66.3)	342 (69.8)
Other	175 (6.7)	10 (2.0)
Prior treatment, n (%)		
Any NSIST	803 (30.7)	151 (36.9)
Any corticosteroid	1546 (59.1)	296 (72.4)
PLEX, eculizumab, rituximab, or IVIg	1579 (60.4)	331 (67.6)
Baseline MG-ADL (pre-efgartigimod)†		
Mean (SD), points	8.5 (3.7)	8.6 (3.7)
Prescriber region, n (%)		
West	486 (18.6)	105 (21.4)
Midwest/North Central	563 (21.5)	82 (16.7)
Northeast	420 (16.1)	89 (18.2)
South	1124 (43.0)	210 (42.9)
Multiple/unknown	23 (0.9)	4 (0.8)
Patient weight‡		
Mean (SD), kg	93.9 (27.6)	95.7 (26.2)
<80, n (%)	653 (32.5)	113 (28.5)
80-120, n (%)	1042 (51.9)	218 (55.1)
≥120, n (%)	314 (15.6)	65 (16.4)

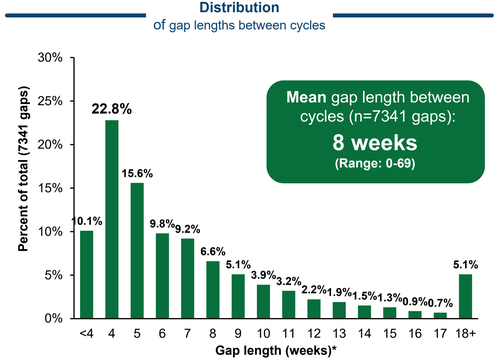
*Total of percentages for insurance exceed 100% as patients may have had multiple plan subscriptions during the analysis duration. *Other" included government, patient assistance programs, federal employee programs, and Veteran's Affairs/Department of Defense. †Baseline MG-ADL score was only available for 1616 (61.8%) and 299 (61.0%) of patients in cohorts A and B, respectively. ‡Patient weight was only available for 2009 (76.8%) and 396 (80.8%) of patients in cohorts A and B, respectively. Percentages are calculated based on patients who had scores available.

- Among 3961 patients who had initiated efgartigimod by September 26, 2023, 2616 patients (66%) had initiated at least Cycle 2, and 490 patients (12%) had initiated at least Cycle 6 (Table 1).
  - 10 patients in Cohort A and 6 patients in Cohort B had usage of subcutaneous efgartigimod (approved June 2023), while all other efgartigimod infusions were intravenous.
- Baseline patient characteristics were similar across cohorts and consistent with published gMG populations, with heavier representation of government insurance users (Table 1).
- Larger proportions of patients who had initiated at least Cycle 6 had used other common gMG treatments prior to efgartigimod initiation compared with patients who had initiated at least Cycle 2 (Table 1).

2. Overall, gaps between efgartigimod treatment cycles were most commonly 4 weeks

- Among Cohort A, 7341 total gaps between treatment cycles were identified.
- The overall average gap length between cycles was 8 (range: 0-69) weeks, translating into an average of 4.7 annual cycles.
- The most common gap length between cycles was 4 weeks, with nearly half of all gaps (48.2%) being 4-6 weeks (Figure 2).

Figure 2. Overall efgartigimod dosing patterns (Cohort A)

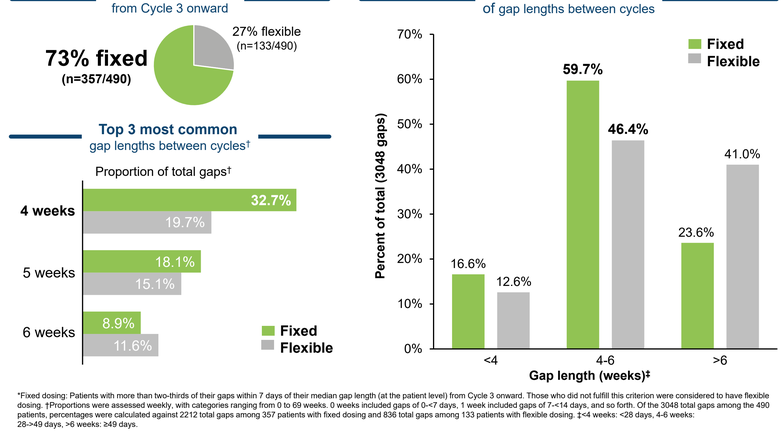


*Proportions were assessed weekly, with categories ranging from 0 to 69 weeks. 0 weeks included gaps of 0-<7 days, 1 week included gaps of 7-<14 days, 2 weeks included gaps of 14-<21 days, and so forth. Percentages were calculated against 7341 total gaps identified.

3. Most patients who had initiated at least Cycle 6 had fixed dosing from Cycle 3 onward

- Among Cohort B, 3048 total gaps between treatment cycles were identified.
- After completing the first 2 treatment cycles, the majority of patients (73%, n=357/490) established fixed dosing (Figure 3).
- 46% (n=224/490) established fixed dosing starting from Cycle 1.
- Regardless of treatment cadence, the majority of gaps between cycles were 4-6 weeks (Figure 3).

Figure 3. Efgartigimod dosing patterns and treatment cadence (Cohort B)



*Fixed dosing: Patients with more than two-thirds of their gaps within 7 days of their median gap length (at the patient level) from Cycle 3 onward. Those who did not fulfill this criterion were considered to have flexible dosing. †Proportions were assessed weekly, with categories ranging from 0 to 69 weeks. 0 weeks included gaps of 0-<7 days, 1 week included gaps of 7-<14 days, and so forth. Of the 3048 total gaps among the 490 patients, percentages were calculated against 2212 total gaps among 357 patients with fixed dosing and 836 total gaps among 133 patients with flexible dosing. ‡<4 weeks: <28 days, 4-6 weeks: 28-≥49 days, >6 weeks: ≥49 days.

SUMMARY

- **Efgartigimod dosing patterns in US clinical practice were assessed based on a dataset containing the most direct, comprehensive, and up-to-date insights into infusion dates as of September 26, 2023**
- **The most common gap length between efgartigimod treatment cycles overall was 4 weeks**
- **The majority of patients who had initiated at least Cycle 6 had fixed dosing starting from Cycle 3**
- **These results based on an increased number of patients and longer-term data suggest that efgartigimod dosing patterns in US clinical practice remain similar to results published in an earlier study[6]**

Limitations and future studies

- As data analyzed were patient reported, further analyses should be conducted to provide additional insights.
- The extent to which external factors (eg, payer policies) may influence gap lengths and treatment cadence were not available in this dataset and should be explored.
- No tolerability or adverse event data were collected as part of the dataset to inform the safety associated with different dosing cadence.
- As dosing patterns may continue to evolve, future studies should continue to collect real-world data to help optimize outcomes for patients.

ABBREVIATIONS
IQR, interquartile range; IVIg, intravenous immunoglobulin; MG-ADL, Myasthenia Gravis-Activities of Daily Living; NSIST, nonsteroidal immunosuppressing treatment; PLEX, plasma exchange; SD, standard deviation.

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