Reduction in oral glucocorticoid use at 18 months following efgartigimod initiation based on a United States claims database

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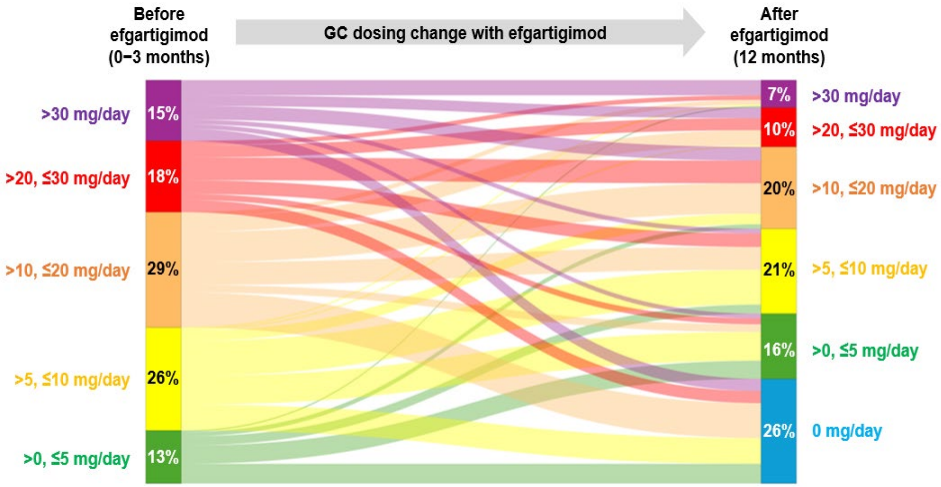
Background

Concerns for long-term reliance on glucocorticoids (GCs) in gMG

- While GC are mainstay therapy in gMG,[1,2] they can be associated with toxicity, especially when used long term at doses of ≥10mg/day.[3,4] Evidence of GC reduction is critical to support treatment decision-making with targeted therapies.

Steroid-sparing potential of efgartigimod (EFG)

- EFG was approved in the US in 2021 for the treatment of anti-AChR antibody-positive gMG, based on safety and efficacy demonstrated in the ADAPT trial.[5,6]
- Several case studies globally have demonstrated substantial GC reduction with efgartigimod.[7-12]
- We recently published that GC dosing significantly decreased after 12 months of continuous efgartigimod therapy based on 266 patients from US claims.[13]



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Objective: To evaluate longitudinal changes in GC use among patients with gMG using chronic GC, who initiated and continued efgartigimod for 18 months.

1. Engel-Nitz NM, et al. *Muscle Nerve*. Feb 27 2018. 2. Sanders DB, et al. *Neurology*. Jul 26 2016;87(4):419-25. 3. Misra UK, et al. *Acta Neurol Belg*. Feb 2020;120(1):59-64. 4. Johnson S, et al. *Med Sci Monit.* Oct 28 2021;27:e933296. 5. Howard JF Jr, et al. *Lancet Neurol.* 2021;20(7):526-536. 6. argenx BV. VYVGART (efgartigimod alfa-fcab) [package insert]. Accessed March 11, 2024. 7. Suzuki S, et al. *Neurol Clin Pract.* Jun 2024;14(3):e200276. 8. Singer M, et al. *Muscle Nerve.* Nov 21 2023. 9. Frangiamore R et al. *Eur J Neurol.* Apr 2024;31(4):e16189. 10. Jin L, et al. *Ther Adv Neurol Disord.* 2025;18:17562864251319656. 11. Silvestri NJ. *Muscle Nerve*. Mar 2025;71(3):422-428. 12. Fuchs L, et al. *J Neurol.* Mar 25 2024. 13. Goyal N, et al. *J Neurol Sci.* 2025; 123652.

EFG, efgartigimod; gMG, generalized myasthenia gravis; GC, glucocorticoid.

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Dataset and study type

Dataset

- Insurance open claims-based dataset (IQVIA)* April 2016 – January 2025
- MG-ADL scores from the My VYVGART Path patient support program dataset

Retrospective cohort study

- Inclusion/exclusion criteria:
  - At least 18 months of ongoing EFG usage based on claims captured†
  - First EFG claim between Jan 1 – Jun 30, 2023
  - GC claims present during the 1 year prior to EFG initiation‡
  - Continuous quarterly claims activity to minimize missing data
  - No concomitant usage of C5, rituximab, or non-EFG FcRn inhibitors with EFG

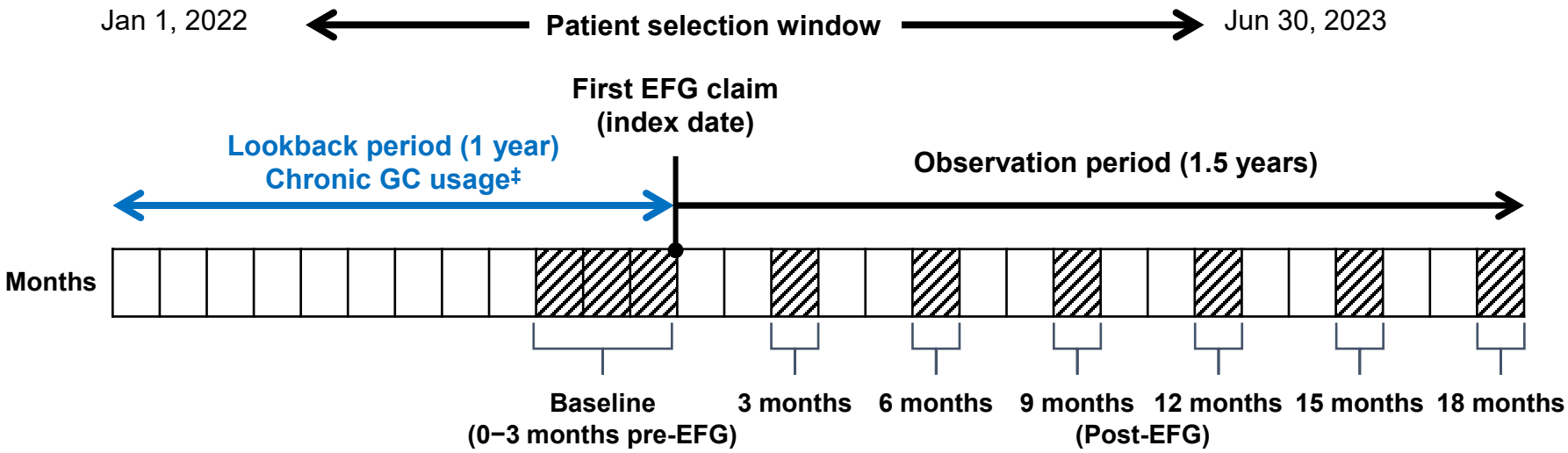
Outcomes

- **Average daily dose (ADD)** of GC at 3 months (60-90 days), 6 months (150-180 days), 9 months (240-270 days), 12 months (330-365 days), 15 months (425-455 days), and 18 months (515-545 days) from baseline (pre-EFG)
- **Percentage of patients** whose GC ADD tapered by at least 1mg/day, 25%, 50%, or 75% from baseline (pre-EFG)
- **Change in MG-ADL** from baseline (pre-EFG) over time

*Based on information licensed from IQVIA: Longitudinal Access and Adjudication Data (LAAD) for the period April 2016−January 2025, reflecting estimates of real-world activity (all rights reserved). †Patients with a gap of >120 days between consecutive EFG claims were excluded. Both IV or SC formulations were considered. ‡Baseline GC usage was defined as any GC usage present in the 0-30 days immediately prior to EFG initiation, and at least 90 days of cumulative GC usage during the 1 year prior to EFG initiation.
EFG, efgartigimod; GC, glucocorticoid; MG-ADL, Myasthenia Gravis Activities of Daily Living.

Study design and inclusion criteria

Study design



Inclusion criteria

	N (%)
Adults (≥18 years of age) with first EFG claim Jan 1 – Jun 30, 2023	2195 (100)
Continuous quarterly activity*	1748 (80)
No concomitant usage of C5, rituximab, or non-EFG FcRn inhibitors with EFG	1387 (63)
Evidence of chronic GC usage prior to EFG initiation†	518 (24)
Remained on EFG treatment for at least 18 months‡	**167 (8)**
	Final study cohort

*Continuous quarterly activity was defined as ≥1 record in database every quarter from 1-year pre-EFG to 545 days post-EFG initiation. †Baseline GC usage was defined as any GC usage present in the 0-30 days immediately prior to EFG initiation, and at least 90 days of cumulative GC usage during the 1 year prior to EFG initiation. ‡Patients with a gap of >120 days between consecutive EFG claims were excluded. In addition, 1 patient with incomplete GC dosing information was excluded.
EFG, efgartigimod; GC, glucocorticoid.

Baseline patient characteristics

Age, years	**N = 167**
Mean (95% CI)	60 (58, 62)
Median (IQR)	62 (50-72)
Gender, n (%)	
Male	95 (57)
Female	72 (43)
Insurance type for first EFG claim, n (%)*	
Commercial	94 (56)
Medicare	67 (40)
Medicaid / Other / Unknown	>0, <20†
Common gMG comorbidities, n (%)	
Hypertension	78 (47)
Sleep disorder	47 (28)
Hyperlipidemia	43 (26)
Diabetes	42 (25)
Sleep apnea	39 (23)
Obesity	33 (20)
GERD	27 (16)
Thyroid-related disorders	20 (12)

NSIST/advanced therapy‡ usage during 1-year period prior to EFG initiation, n (%)	
NSIST only	54 (32)
Advanced therapy‡ only	27 (16)
NSIST + advanced therapy	48 (29)
No NSIST or advanced therapy‡	38 (23)

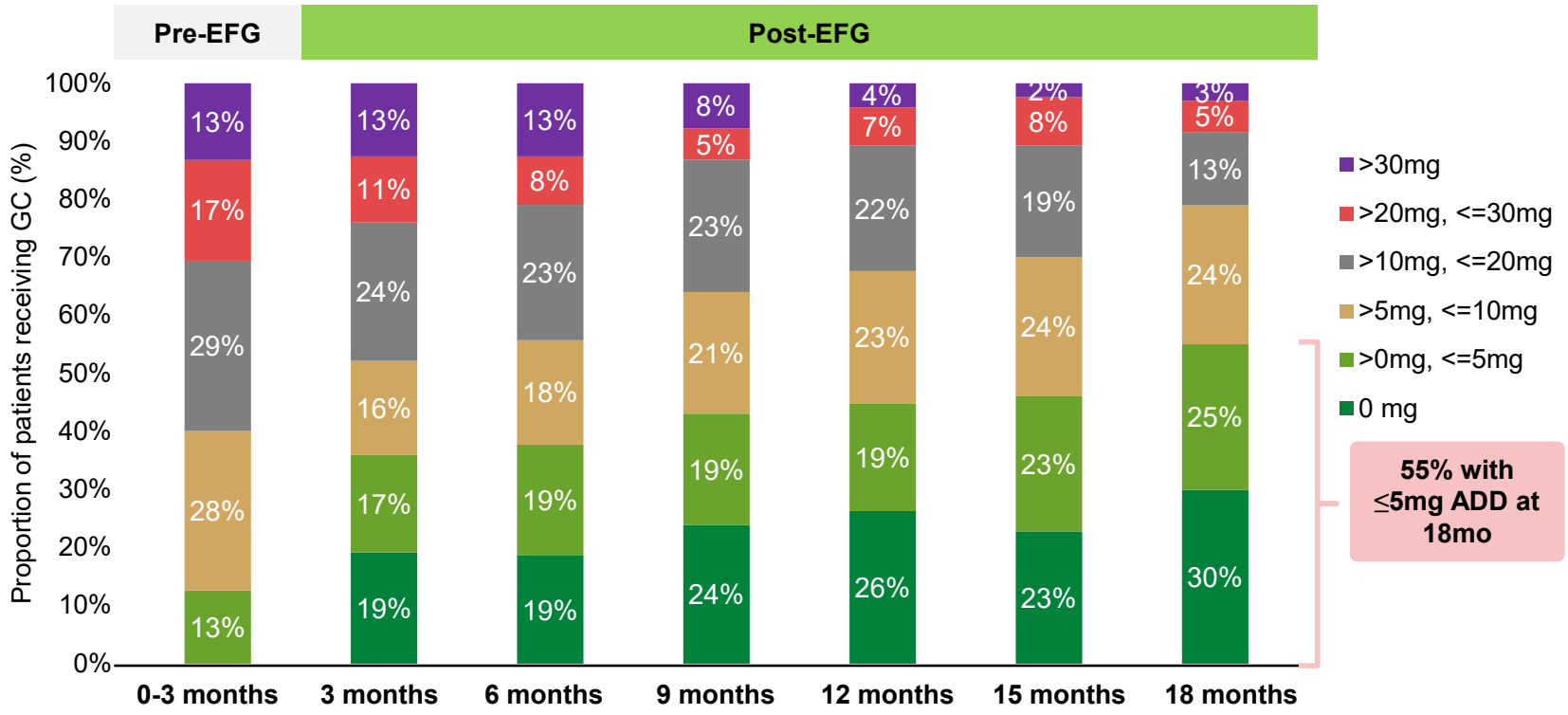
- Consistent with findings from 12-months cohort in previous publication
- High proportion of comorbidities including hypertension
- >75% of patients used NSIST and/or other advanced gMG therapies‡ concomitantly with GC prior to EFG initiation

*Percentages may not add up to 100% as patients may be tagged to multiple payer channels. †Patient counts greater than 0 but less than 20 have been masked. ‡Advanced therapy included IVIg/SCIg, PLEX, eculizumab, and rituximab.

CI, confidence interval; EFG, efgartigimod; GERD, gastroesophageal reflux disease; GC, glucocorticoid; gMG, generalized myasthenia gravis; IQR, interquartile range; IVIg/SCIg, intravenous or subcutaneous immunoglobulin; NSIST, nonsteroidal immunosuppressive treatment; PLEX, plasma exchange.

Results: GC tapering

Change in GC ADD distribution after EFG initiation over time (N=167)



Mean ADD (95%CI), mg/d	16.6 (14.5,18.6)	13.8 (11.5, 16.1)	12.8 (10.6,14.9)	10.7 (8.8,12.6)	8.5 (7.1,10.0)	9.1 (7.4,10.8)	7.5 (5.9,9.1)
P-value vs. Pre-EFG	-	< 0.05	< 0.001	< 0.001	< 0.001	< 0.001	< 0.001

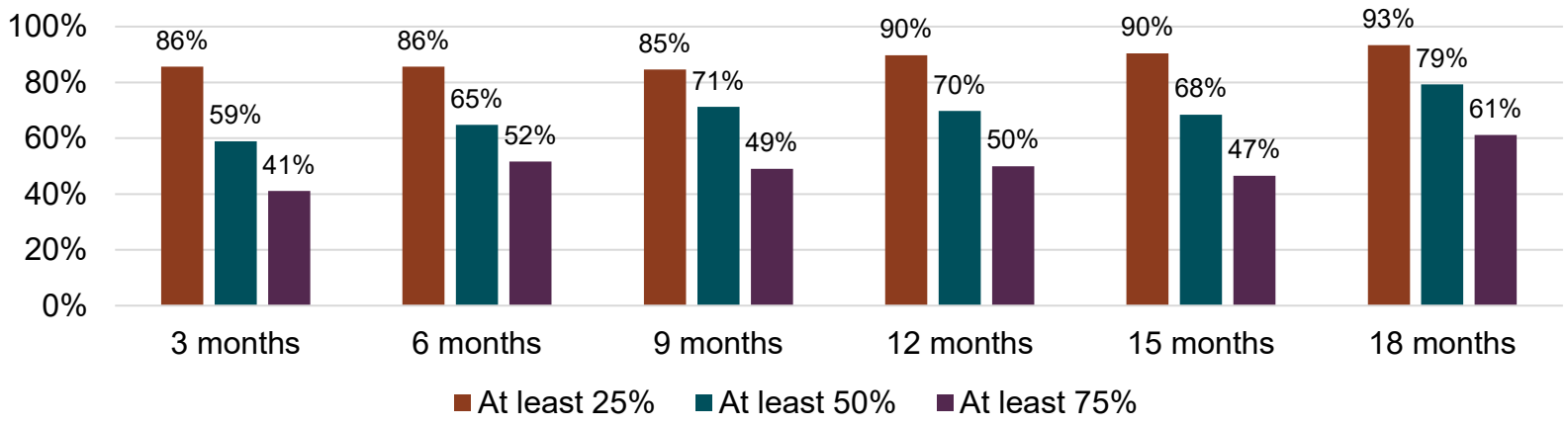
*P-values for ADD were calculated against the ADD at baseline (pre-EFG) using Wilcoxon signed rank tests. $P < 0.05$ was considered statistically significant.
ADD, average daily dose; EFG, efgartigimod; GC, glucocorticoid.

Results: GC tapering

Proportion of patients with GC ADD tapered, increased, or unchanged vs. pre-EFG, n (%)

	Pre-EFG	Post-EFG					
	0-3 months	3 months	6 months	9 months	12 months	15 months	18 months
Tapered (≥1mg)	-	90 (53.9)	91 (54.5)	104 (62.3)	116 (69.5)	114 (68.3)	121 (72.5)
Unchanged (<±1mg)	-	23 (13.8)	>0, <20†	>0, <20†	21 (12.6)	>0, <20†	20 (12.0)
Increased (≥1mg)	-	54 (32.3)	60 (35.9)	45 (26.9)	30 (18.0)	34 (20.4)	26 (15.6)

Magnitude of GC ADD reduction compared to baseline, among those who tapered GC*



†Patient counts greater than 0 but less than 20 have been masked. *Percentages have been calculated by considering the patients who decreased their GC ADD as the denominator.
ADD, average daily dose; CI, confidence internal; EFG, efgartigimod; GC, glucocorticoid.

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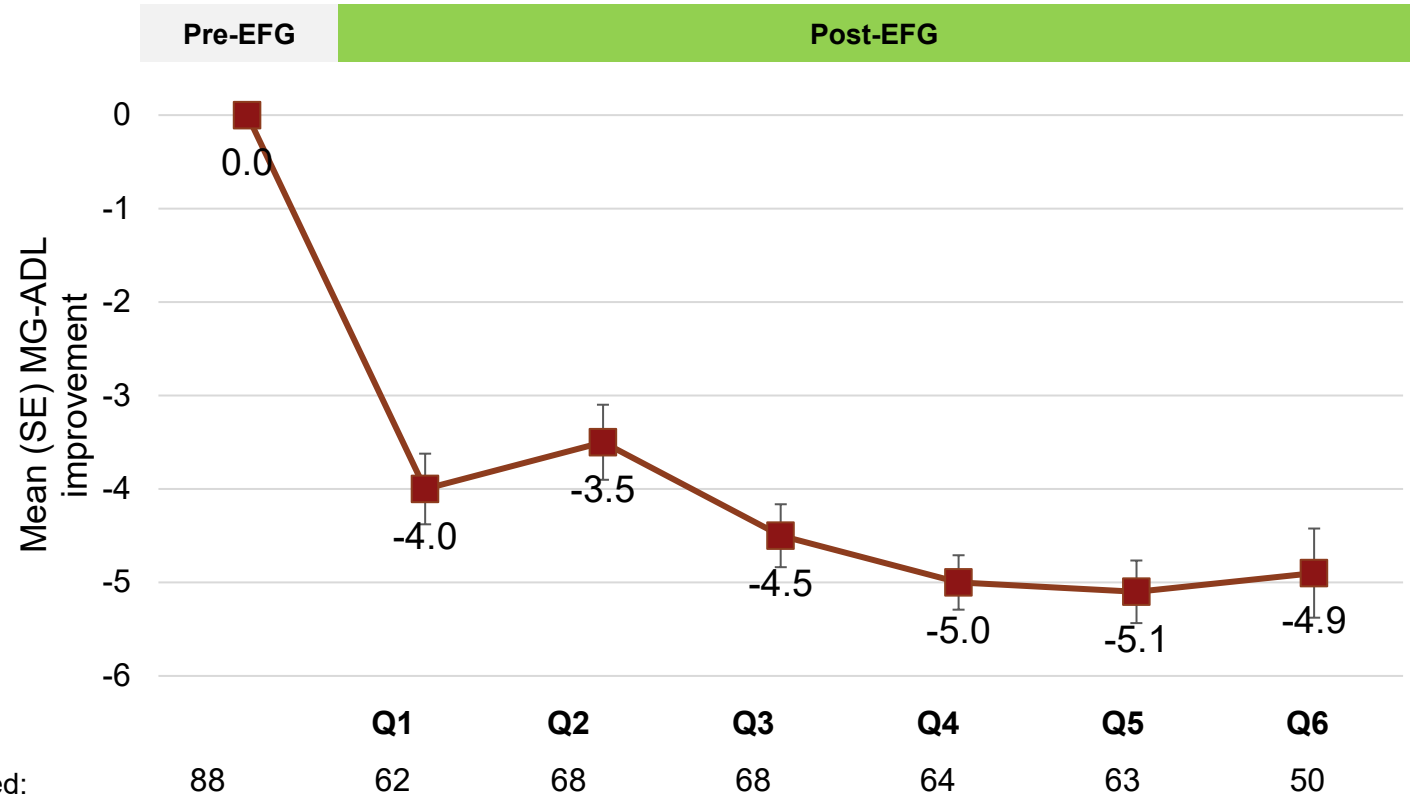
Results: Efficacy (MG-ADL)

MG-ADL outcomes among a subset with scores available (N=88/167)*



*Patients with a baseline and at least 4 follow-up MG-ADL scores available were considered for analysis. †Mean of the best MG-ADL scores recorded during each quarter after efgartigimod initiation was calculated, with the denominator in each quarter being the subset of patients with at least 1 MG-ADL captured in that quarter.‡Quarters were defined by days after EFG initiation (Q1: 1-90 days, Q2: 91-180 days, Q3: 181-270 days, Q4: 271-365 days, Q5: 366-455 days, and Q6: 456-545 days after EFG initiation). [a]Baseline scores were captured before EFG initiation.

EFG, efgartigimod; MG-ADL, Myasthenia Gravis Activities of Daily Living.

Conclusions and future steps

Key conclusions

- In this real-world cohort of chronic GC users, 18 months of continuous EFG therapy led to a significant and progressive reduction in GC use, while retaining improved MG-ADL scores.
  - At 18 months, the mean GC dose was reduced by over 50% from baseline (16.6 mg/day to 7.5 mg/day). Majority of patients (55%) achieved GC ADD of ≤5mg/day, and 30% had no GC usage.
  - Among a subset of patients with MG-ADL data captured, there was a significant and sustained improvement in MG-ADL scores (~5 points).

Strengths

- The study enabled inclusion of a large sample size with longitudinal follow-up, with results supporting reduction of GC with the use of EFG observed in previously published case series.

Limitations

- Claims-based data analyses are subject to several inherent limitations including assumptions, potential coding errors, and risk of missing data.
- Insights into how prescribers are approaching GC tapering on EFG were not assessed and require alternative datasets to explore.

ADD, average daily dose; EFG, efgartigimod; GC, glucocorticoid; MG-ADL, Myasthenia Gravis Activities of Daily Living.

Thank you!

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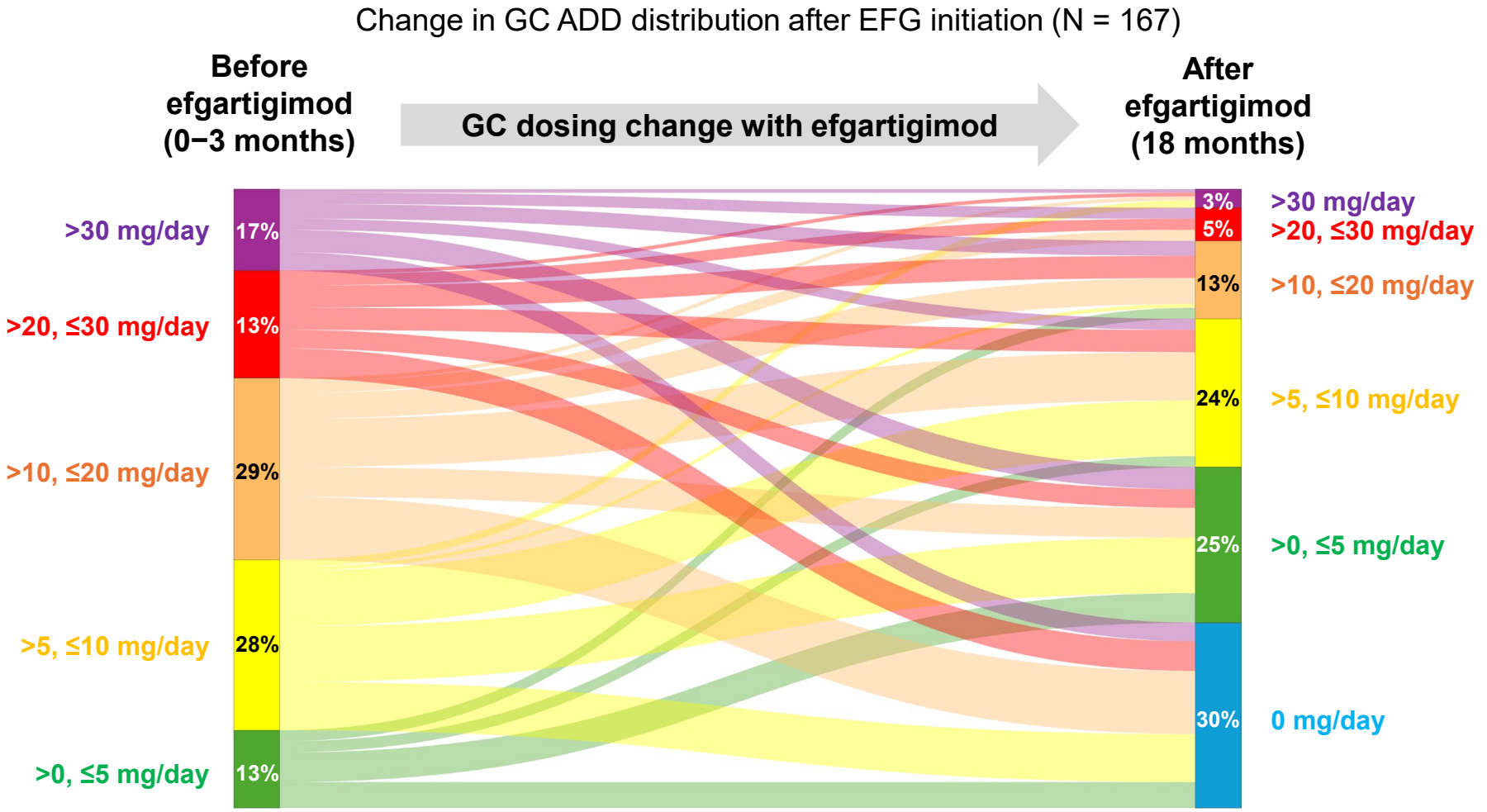
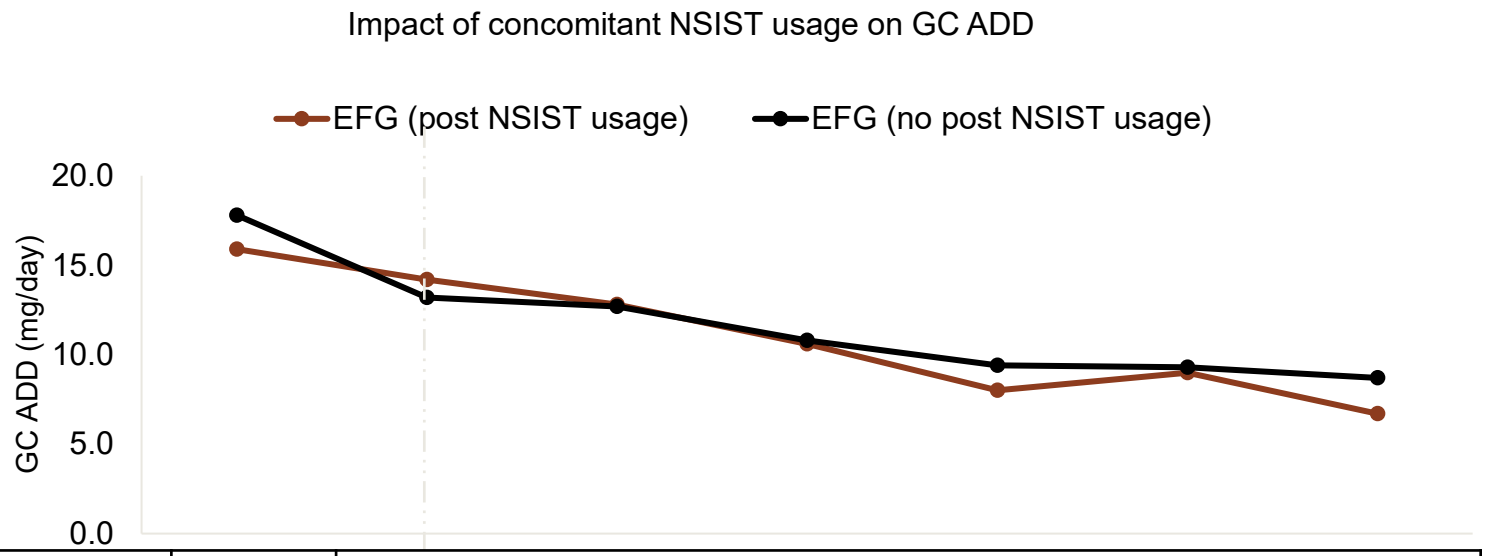
Results: Change in GC ADD distribution



ADD, average daily dose; EFG, efgartigimod; GC, glucocorticoid.

Results: NSIST usage



Mean (95% CI) ADD, mg/d	Before EFG	After EFG					
	Baseline (0–3 months)	**3 months**	**6 months**	**9 months**	**12 months**	**15 months**	**18 months**
No NSIST with efgartigimod (n = 64)	17.8 (13.8, 21.7)	13.2 (9.9, 16.4)	12.7 (9.3, 16.0)	10.8 (7.8, 13.8)	9.4 (7.1, 11.7)	9.3 (7.0,11.6)	8.7 (6.0, 11.4)
Change from baseline	-	-4.6	-5.1	-7.0	-8.4	-8.5	-9.1
p value*	-	<0.05	<0.05	<0.001	<0.001	<0.001	<0.001
NSIST usage with efgartigimod (n = 103)	15.9 (13.6, 18.1)	14.2 (11.1, 17.3)	12.8 (10.0, 15.7)	10.6 (8.2, 13.0)	8.0 (6.2, 9.8)	9.0 (6.6,11.3)	6.7 (4.7, 8.8)
Change from baseline	-	-1.7	-3.1	-5.3	-7.9	-6.9	-9.2
p value*	-	<0.05	<0.05	<0.001	<0.001	<0.001	<0.001

*P-values were calculated using Wilcoxon signed-rank test.
ADD, average daily dose; CI, confidence interval; EFG, efgartigimod; GC, glucocorticoid; NSIST, nonsteroidal immunosuppressive treatment.

Results: Driver of GC tapering

Multivariate logistic regression to explore drivers of GC tapering

		Descriptive		Statistical association	
Variable	Overall (N=167)	Tapered (n=121)	Not tapered (n=46)	OR (95% CI)	*P*
Age, mean (SD)	59.9 (14.7)	59.8 (14.2)	60.1 (16.1)	1.04 (1.00, 1.08)	0.058
Gender, n(%)					
M	95 (56.9)	64 (52.9)	31 (67.4)	REF	-
F	72 (43.1)	57 (47.1)	>0, <20†	2.64 (1.09, 6.79)	**0.036**
Region, n(%)					
Midwest	35 (21.0)	26 (21.5)	>0, <20†	REF	-
Northeast	26 (15.6)	>0, <20†	>0, <20†	0.92 (0.25, 3.47)	0.90
South	81 (48.5)	58 (47.9)	23 (50.0)	0.68 (0.23, 1.88)	0.46
West	25 (15.0)	>0, <20†	>0, <20†	1.04 (0.25, 4.59)	0.96
Insurance at efgartigimod initiation, n(%)					
Commercial	94 (56.3)	74 (61.2)	20 (43.5)	REF	-
Medicare	67 (40.1)	44 (36.4)	23 (50.0)	0.12 (0.01, 1.39)	0.074
Medicaid	>0, <20†	>0, <20†	>0, <20†	0.37 (0.14, 0.97)	**0.047**
Charlson Comorbidity Index					
Mean (SD)	1.2 (1.7)	1.3 (1.8)	0.9 (1.3)	1.23 (0.93, 1.69)	0.16
At least 1 NSIST claim, n (%)					
1-year pre-EFG	102 (61.1)	82 (67.8)	20 (43.5)	2.30 (0.75, 7.26)	0.14
18-months post-EFG	103 (61.7)	80 (66.1)	23 (50.0)	1.26 (0.39, 4.02)	0.69
Baseline GC ADD, n(%)					
<10mg/day	62 (37.1)	34 (28.1)	28 (60.9)	REF	-
≥10mg/day; <20mg/day	53 (31.7)	40 (33.1)	>0, <20†	2.34 (0.98, 5.78)	0.059
≥20mg/day; <30mg/day	29 (17.4)	25 (20.7)	>0, <20†	4.12 (1.23, 16.9)	**0.031**
≥30mg/day	23 (13.8)	22 (18.2)	>0, <20†	33.1 (5.64, 644)	**0.001**

†Patient counts greater than 0 but less than 20 have been masked
GC, glucocorticoid; NSIST, nonsteroidal immunosuppressive treatment; OR, odds ratio; SD, standard deviation.

Methods

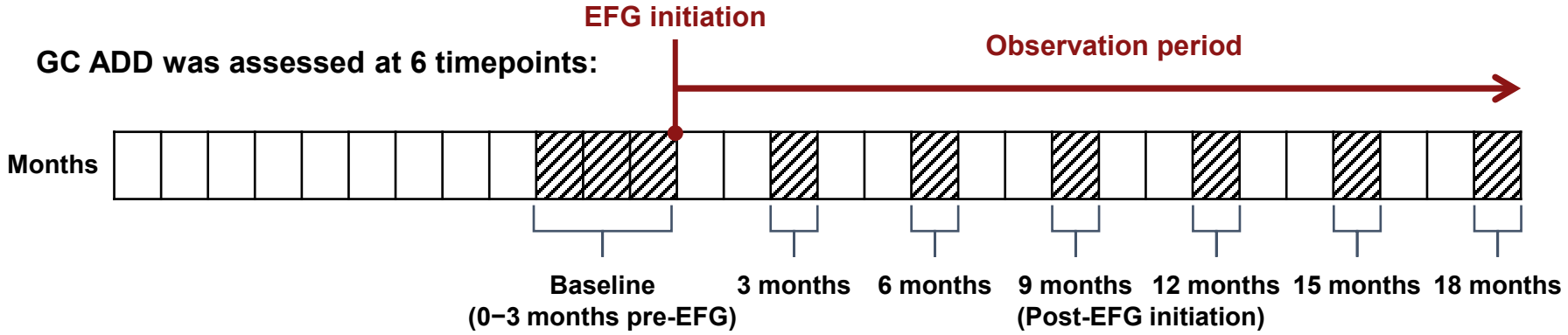
- **GC ADD** was defined as[1]:

$$\frac{\text{Total GC dose (strength x quantity)*}}{\text{Total number of days within timepoint}^{\dagger 1}}$$

- **GC tapering** was defined as ≥1 mg reduction in GC ADD from baseline (pre-EFG).

Example: Patient has 1 claim for 10mg tablets for 20 days, and 1 claim for 20mg tablets for 10 days during baseline period.

$$\frac{(10*20) + (20*10)}{90 \text{ days in baseline period}} = 4.4 \text{ mg/day}$$



*GC claims that were within 14 days of one another were considered as part of 1 GC episode. Detailed calculation methods are included in the appendix. †GC ADD was calculated at 6 timepoints: Pre-EFG (0-90 days immediately prior to EFG initiation), Post-EFG 3 months (60-90 days post-EFG initiation), Post-EFG 6 months (150-180 days post-EFG initiation), Post-EFG 9 months (240-270 days post-EFG initiation), Post-EFG 12 months (330-365 days post-EFG initiation), Post-EFG 15 months (425-455 days post-EFG initiation), and Post-EFG 18 months (515-545 days post-EFG initiation). GC doses were converted to prednisone-equivalent strengths. Sensitivity analyses were performed wherein ADD was calculated based on the number of days of supply dispensed as the denominator.

1. DerSarkissian M, et al. *ACR Open Rheumatol.* Jun 2023;5(6):318-328.

ADD, average daily dose; EFG, efgartigimod; GC, glucocorticoid.

Additional detail on IQVIA LAAD database

Fact Sheet: Longitudinal Access and Adjudication Data (LAAD). IQVIA. Accessed 18 Feb 2024. https://www.iqvia.com/-/media/iqvia/pdfs/library/fact-sheets/iqvia-longitudinal-access-and-adjudication-data-fact-sheet-2023.pdf.