PARTICIPATION IN A PATIENT SUPPORT PROGRAM IS ASSOCIATED WITH IMPROVED ACCESS TO EFGARTIGIMOD TREATMENT FOR GENERALIZED MYASTHENIA GRAVIS IN PATIENTS FACING SOCIAL DETERMINANTS OF HEALTH (SDOH) CHALLENGES

HPR95

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Introduction and Objective

- Efgartigimod (EFG) was approved by the FDA in 2021 for the treatment of generalized myasthenia gravis (gMG), a rare, autoimmune neuromuscular junction disorder with high morbidity
- Patients prescribed EFG may encounter payer coverage restrictions that deny, delay, or deter them from accessing treatment
- A manufacturer-provided patient support program (PSP) aims to help patients navigate access to prescribed EFG treatment, including benefit verification, insurance requirements, and finding eligible infusion centers. Evidence on the program's effectiveness is limited
- **The objective of this study was to quantify the PSP's impact on treatment access, especially within patients facing Social Determinants of Health (SDOH) challenges**

Methods

- This was a retrospective cohort study using linked specialty pharmacy (SP) and PSP data from December 17, 2021 – May 3, 2024
- Patients with an EFG script were identified in the SP data (earliest was the index date)
  - ≥3 months of follow-up required to be included in sample
- PSP and non-PSP cohorts were constructed based on patients' involvement with the PSP
  - PSP participation was defined as a having a PSP interaction record within 30 days of the index date
- **Treatment access outcomes** included:
  - Rates of **initial coverage decision** (approval, denial, or unresolved)
  - **Coverage appeal** rate
  - **Appeal approval** and **denial** rates
  - **Coverage denial** rate
  - **Treatment dispense** rate
    - **Dispense within 3 months** of script
  - **Time to coverage decision** from date of script
  - **Time to dispense**, following initial approval, from date of script

Figure 1: Sample selection

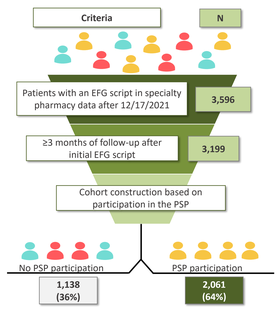


Table 1: Baseline characteristics

Characteristic[a]	PSP N = 2,061	Non-PSP N = 1,138	p-value[b]
Patient demographics			
Age in years, mean (SD)[c]	60.6 (16.4)	60.3 (17.7)	0.831
< 29, n (%)	60 (2.9%)	45 (4.0%)	0.113
30 - 49, n (%)	225 (10.9%)	116 (10.2%)	0.525
50 - 69, n (%)	475 (23.1%)	210 (18.5%)	**0.002**
> 70, n (%)	390 (18.9%)	221 (19.4%)	0.732
N/A	911 (44.2%)	546 (48.0%)	**0.040**
Gender, n (%)			
Female	855 (41.5%)	447 (39.3%)	0.224
Male	788 (38.2%)	406 (35.7%)	0.152
N/A	418 (20.3%)	285 (25.0%)	**0.002**
Geographic region, n (%)[d]			
Midwest	312 (15.1%)	186 (16.3%)	0.368
Northeast	281 (13.6%)	207 (18.2%)	**<.001**
South	989 (48.0%)	534 (46.9%)	0.565
West	473 (23.0%)	208 (18.3%)	**0.002**
N/A	6 (0.3%)	3 (0.3%)	0.888
Payment details			
Payer, n (%)			
Commercial	412 (20.0%)	210 (18.5%)	0.293
Medicaid	91 (4.4%)	45 (4.0%)	0.536
Medicare	366 (17.8%)	192 (16.9%)	0.527
Other	616 (29.9%)	345 (30.3%)	0.800
N/A	576 (28.0%)	346 (30.4%)	0.142
Physician details			
Specialty, n (%)			
Neurology	1,036 (50.3%)	573 (50.4%)	0.963
PCP/Other	261 (12.7%)	107 (9.4%)	**0.006**
N/A	764 (37.1%)	458 (40.3%)	0.077

- Baseline characteristics included age, gender, geographic region, payer type, and physician type
- Multivariate logistic regressions adjusting for baseline characteristics estimated risk-adjusted outcomes, odds ratios (ORs) and 95% confidence intervals (CIs) of binary outcomes
- Incidence rate ratios (IRRs) were calculated for continuous outcomes with negative binomial models

Table 1 Notes:
[a] All characteristics based on the first SP record for each patient.
[b] Categorical and continuous variables compared across cohorts with $\chi 2$ and Wilcoxon rank sum tests, respectively.
[c] Only year of birth is provided in the data. All patients were assumed to have a birth date of July 1st.
[d] Geographic region based on ZIP code and 2021 US Census. If patient ZIP was missing, physician ZIP was used.

Results

Baseline characteristics

- Nearly 2/3 of the sample participated in the PSP (**Figure 1**).
- The cohorts were overall well balanced. The PSP cohort had more patients who were 50-69 years old (23.1%), living in the West (23.0% vs. 18.3%), and receiving their EFG script from a PCP/other physician (12.7% vs. 9.4%). The non-PSP cohort had more patients who were living in the Northeast (18.2% vs. 13.6%; all p<0.01) **(Table 1)**.

Initial coverage approval

- Patients in the PSP had **21% higher adjusted odds of initial script approval** than patients not in the PSP (53.5% vs. 48.9%; p=0.01).

Dispense

- Patients in the PSP had **47% higher adjusted odds of receiving a dispense** (61.6% vs. 52.5%; p<0.01) and were **56% more likely to receive a dispense within 3 months** (53.6% vs. 42.8%; p<0.01) than patients not in the PSP.

No approval

- Patients in the PSP had **30% lower adjusted odds of never receiving approval** (29.5% vs. 37.3%; p<0.01) compared to non-PSP patients.

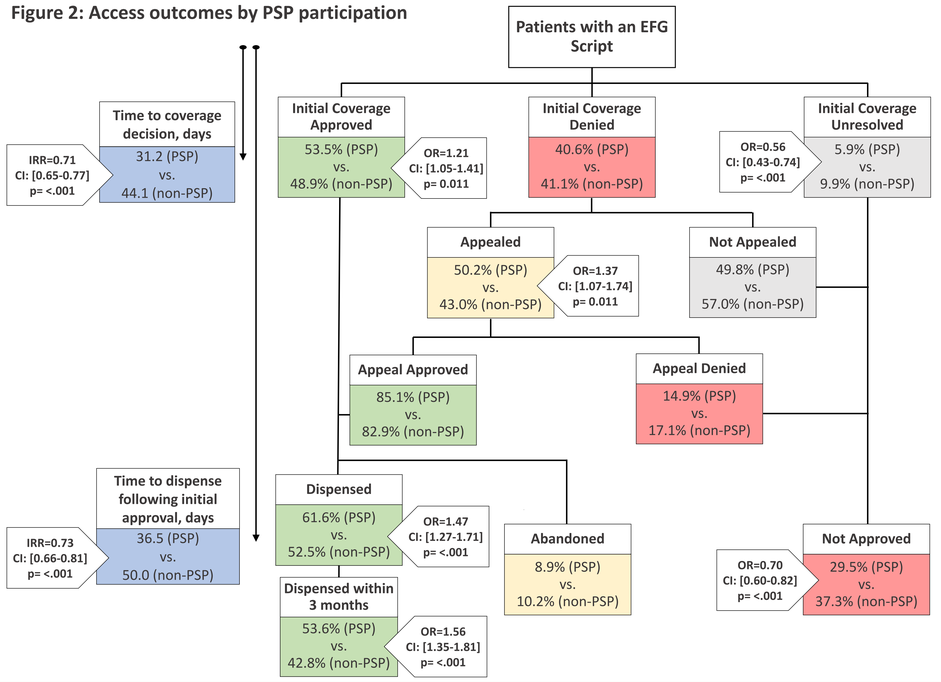
Time to coverage decision/dispense

- PSP patients had a **29% faster time to coverage decision** (31.2 vs. 44.1 days; p<0.01), and a **27% faster time to dispense** (36.5 vs. 50.0 days; p<0.01).

Subgroup analyses

- The relative benefits of PSP participation on **initial coverage approval** were greatest within three subgroups facing SDOH challenges:
  - In the **Midwest**, PSP patients had **53% higher adjusted odds of initial approval** (52.4% vs. 42.8%, p=0.03) compared to non-PSP patients **(Figure 3)**.
  - Among patients with **Medicaid**, PSP patients had **90% higher adjusted odds of initial approval** (54.0% vs. 39.6%, p=0.11) compared to non-PSP patients **(Figure 3)**. Although this finding is numerically large, it is not statistically significant as the study was underpowered to detect a difference in the limited Medicaid sample available.
  - Among patients with **non-specialist prescribers**, PSP patients had **198% higher adjusted odds of initial approval** (63.4% vs. 37.8%, p<0.01) compared to non-PSP patients **(Figure 3)**.
- The relative benefits of PSP participation on **dispense rate** were greatest within two subgroups facing SDOH challenges:
  - Among patients with **Medicaid coverage**, patients in the **PSP had 157% higher adjusted odds of receiving a dispense** than patients not in the PSP (64.2% vs. 45.4%; p=0.03) **(Figure 4)**.
  - Among patients with a **non-specialist prescriber**, patients in the **PSP had 244% higher adjusted odds of receiving a dispense** than patients not in the PSP (65.6% vs. 38.0%; p<0.01) (**Figure 4**).
  - Regional differences in the relative effect of PSP participation on dispense rates were not found (**Figure 4**).

Figure 2: Access outcomes by PSP participation



Results

Figure 3: Variation in initial coverage approval rates among subpopulations

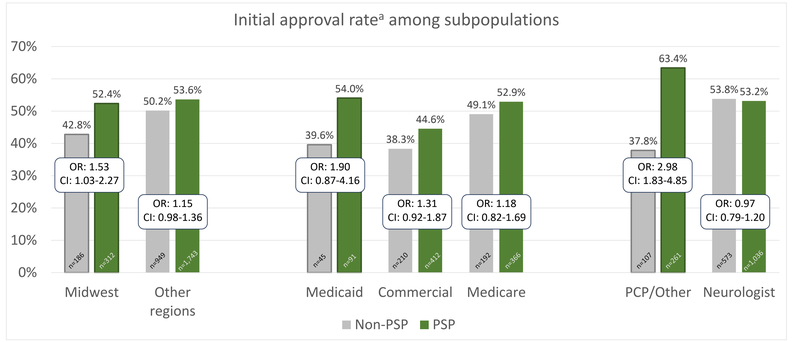


Figure 4: Variation in dispense rates among subpopulations

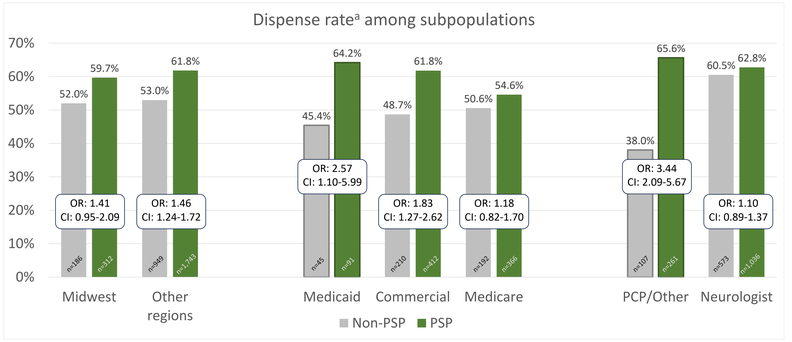


Figure 3 and Figure 4 note: [a] Adjusted outcomes based on multivariate model controlling for characteristics listed in Table 1

Conclusions and Limitations

Conclusions

- 3,199 EFG patients qualified for this analysis, of which 2,061 (64%) participated in the PSP.
- Adjusting for patient and provider characteristics, patients in the PSP had better access to treatment, compared to patients not in the PSP
  - Patients in the PSP had higher rates of initial coverage approval and dispense, and lower rates of never being approved.
  - Patients in the PSP had faster time to dispense and time to coverage decision.
- PSP patients in low access subgroups had the greatest relative improvements in initial approval and dispense rates
- **These findings suggest PSP participation may help patients initiate EFG sooner and more successfully, especially patients facing SDOH challenges.**

Limitations

- This was an observational study, and no causal effect of PSP participation was established
- Individuals enrolling in the PSP may be different from individuals initiating treatment without PSP participation in characteristics that are not observable in the data
- The SP data used in this study do not include eligibility or healthcare coverage information, and thus, it cannot be ensured that patients are continuously covered and that their complete medical and pharmacy activity are captured
- Data were only available through May 2024 and thus the results may not reflect benefits of patients and clinicians gaining more experience with accessing EFG and of improvements to the services offered by the PSP

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