ASSOCIATION BETWEEN PATIENT SUPPORT PROGRAM PARTICIPATION AND ACCESS TO EFGARTIGIMOD TREATMENT FOR GENERALIZED MYASTHENIA GRAVIS

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Introduction and Objective

- Efgartigimod (EFG) is a recently FDA-approved therapy for 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
- **The objective of this study was to compare access to prescribed EFG treatment for gMG among patients with and without PSP participation within a specialty pharmacy (SP) population**

Methods

- This was a retrospective cohort study using SP data from February 2021 through January 2023
- Patients with an initial EFG script (index date) with ≥3 months of follow-up observation were included and stratified based on concurrent PSP participation
- PSP participation was defined as a having a PSP interaction record within 30 days of the index date
- **Access outcomes** included:
  - **Time to coverage decision from referral** (days from referral to approval or denial)
  - **Approval of the initial script** (patients with coverage approval / patients with a script)
  - **Successful EFG dispensing** (patients with successful dispensing / patients with a script)
  - **Time to dispense** (days from referral to successful dispensing)
  - **Abandonment of approved treatment** (patients without a successful dispensing / patients with coverage approval)
- Multivariate logistic regressions adjusting for baseline characteristics estimated risk-adjusted outcomes, odds ratios (ORs) and 95% confidence intervals (CIs) of dichotomous outcomes. Negative binomial models evaluated count outcomes
  - Baseline characteristics included age, gender, geographic region, treatment history, patient copay amount, payer type, and physician type

Figure 1: Sample selection

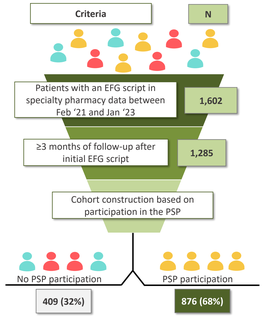


Table 1: Baseline characteristics

Characteristic[1]	PSP N = 876		Non-PSP N = 409		P-value[4]
Patient demographics					
Age in years, mean (SD)[2]	60.6	(16.8)	60.6	(17.2)	0.810
< 29, n (%)	26	(3.0%)	14	(3.4%)	0.662
30 - 49, n (%)	105	(12.0%)	52	(12.7%)	0.711
50 - 69, n (%)	198	(22.6%)	90	(22.0%)	0.811
> 70, n (%)	173	(19.8%)	89	(21.8%)	0.405
N/A	374	(42.7%)	164	(40.1%)	0.380
Gender, n (%)					
Female	375	(42.8%)	167	(40.8%)	0.504
Male	348	(39.7%)	160	(39.1%)	0.836
N/A	153	(17.5%)	82	(20.1%)	0.265
Geographic region, n (%)[3]					
Midwest	80	(9.1%)	72	(17.6%)	0.000
Northeast	72	(8.2%)	44	(10.8%)	0.139
South	249	(28.4%)	98	(24.0%)	0.093
West	180	(20.6%)	66	(16.1%)	0.061
N/A	295	(33.7%)	129	(31.5%)	0.448

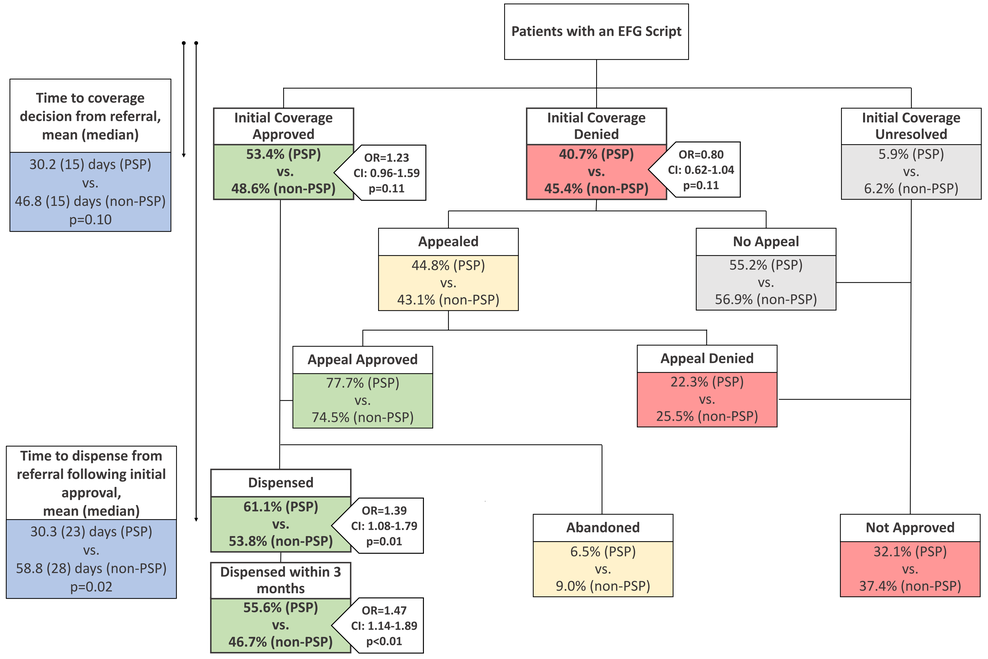
Characteristic[1]	PSP N = 876		Non-PSP N = 409		P-value[4]
Payment details					
Patient copay amount, mean (SD)	$126	($168)	$145	($176)	0.281
Payer, n (%)					
Commercial	159	(18.2%)	79	(19.3%)	0.617
Medicaid	36	(4.1%)	18	(4.4%)	0.808
Medicare	151	(17.2%)	77	(18.8%)	0.487
Other	315	(36.0%)	137	(33.5%)	0.389
N/A	215	(24.5%)	98	(24.0%)	0.821
Physician details					
Specialty, n (%)					
Neurology	496	(56.6%)	259	(63.3%)	0.023
Primary Care / Other	79	(9.0%)	30	(7.3%)	0.313
N/A	301	(34.4%)	120	(29.3%)	0.074

Notes:
[1] All characteristics assessed using the first specialty pharmacy record for each patient
[2] Only year of birth provided in the data. All patients were assigned a birth date of July 1st
[3] Geographic region based on US Census state categorizations
[4] Percentage distributions compared between cohorts with χ2 tests. Means compared between cohorts with t-tests.

Results

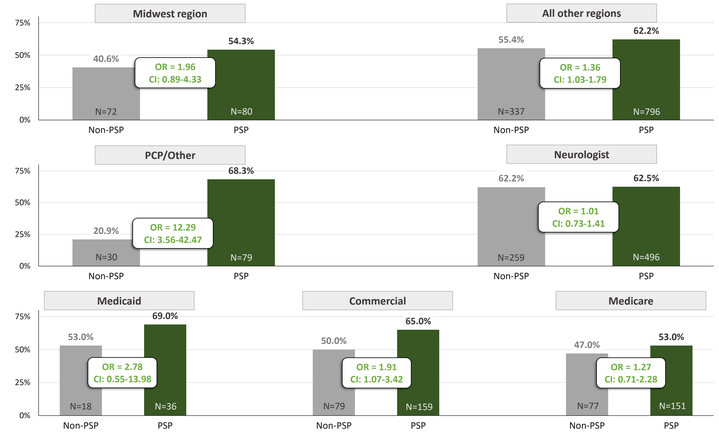
- Approximately **two-thirds of patients participated in the PSP** at the time of their initial EFG script (N=876 PSP; N=409 non-PSP) (**Figure 1**)
- **Cohorts were well-balanced** on patient demographics, except a **higher share of non-PSP patients were in the Midwest** (17.6% vs. 9.1%) and a **lower share of PSP patients were prescribed EFG by a neurologist** (56.6% vs. 63.3%) (both p < 0.05) (**Table 1**)
- Though not statistically significant, PSP patients had a **10% higher rate of initial script approval** (p=0.11) and reached an insurance coverage decision **16 days quicker** on average than non-PSP patients (p = 0.10) (**Figure 2**)
- Among patients with initial script approval, PSP patients **received EFG on average 28 days faster** than non-PSP patients (p < 0.05) (**Figure 2**)
- PSP participants had a **19% higher rate of receiving a dispense of EFG** within the first 3 months than non-PSP patients and **14% higher rate overall** (both p < 0.05) (**Figure 2**)
- PSP participants also had a non-statistically-significant **33% lower abandonment rate** than non-PSP patients (p = 0.11) (**Figure 2**)
- Descriptive differences in dispense rates with PSP participation were greatest for the **Midwest** (+34%; p < 0.10), **patients prescribed EFG by a primary care provider** (+227%; p < 0.05), and **Medicaid-insured patients** (+30%; p = 0.21) (**Figure 3**)

Figure 2: Access outcomes by PSP participation



Results

Figure 3: Variation in dispense rates among subpopulations



Conclusions and Limitations

Conclusions

- 1,285 EFG patients qualified for this analysis, of which 876 (68%) participated in the PSP
- PSP enrollment varied significantly by geographic region
- Adjusting for patient and provider characteristics, multiple measures of access to prescribed EFG treatment were improved for patients using the PSP compared to patients not using the PSP, including dispense rate, dispense rate within 3 months, initial script approval, and abandonment
- PSP participation was associated with greater improvements for patients in regions with low access rates, prescribed EFG by a PCP vs. specialist, and with Medicaid insurance
- **These findings suggest PSP participation may be associated with improved access to prescribed EFG treatment, including more successful and faster initiation, which should be continually examined as data accrue**

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 January 2023 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
- Insufficient sample size are currently available to examine the effect of PSP participation in certain subpopulations and to identify statistical significance on some outcomes

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