P40

Differences Between Patient and Provider Perceptions in Assessing Myasthenia Gravis Disease Burden and Minimal Symptom Expression: A Real-World Survey

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KEY TAKEAWAYS

- Compared with providers' responses, patients reported almost twice the negative impact of gMG across various areas of their lives, particularly overall health, daily activities, and ability to plan for the future
- While both patients and providers recognized MG-ADL as an important measure of disease severity, significant disconnects exist regarding acceptable total MG-ADL scores, disease burden, the negative impact of gMG on QoL, and the likelihood of attaining MSE
- Survey results highlight a need for continued education on MG-ADL and gMG disease burden and treatment expectations to bridge the gaps between patients and providers

BACKGROUND

- Myasthenia gravis (MG) is a rare, chronic autoimmune disorder that causes muscle weakness and fatigue when immunoglobulin G autoantibodies attack the neuromuscular junction
  - Burdensome symptoms include ptosis, diplopia, dysarthria, dysphagia, dyspnea, and, if severe MG, respiratory failure
- Recent clinical trials of targeted therapies (aimed at reducing autoantibody-producing B cells) have included the Myasthenia Gravis Activities of Daily Living (MG-ADL) scale as a primary or secondary endpoint. Trial outcomes have demonstrated improvement in patients' daily activities and achievement of minimal symptom expression (MSE)[1-5]
- We assessed patient and provider awareness of disease burden, MSE, perceptions regarding MG-ADL as a measure of symptom severity, and MG quality-of-life (QoL) scores

METHODS

- A cross-sectional, quantitative ~20 min online survey of **patients** with generalized MG (gMG) and **providers** in the US was conducted from October to November 2024 (recruited via email invitations and consented to the research)
- Provider inclusion criteria: practicing neurologist, neuromuscular specialist, nurse practitioner, or physician assistant with 3-30 years of experience and treating ≥5 patients with MG and ≥1 patients with gMG
- Patient inclusion criteria: age ≥18 years, diagnosis of gMG, and aware of gMG treatment
- Descriptive statistical analyses (means, frequencies) were conducted using Q Research software. Tests of differences (chi square, *t* tests, *z* tests) within respondent types were performed using Q Research Software and SPSS v29. Statistical significance was set at $p < 0.05$
- LIMITATION: Patients included in the survey were not necessarily the same patients whom providers treated. No direct comparison between physicians' perceptions and their own patients' perceptions can be drawn

RESULTS

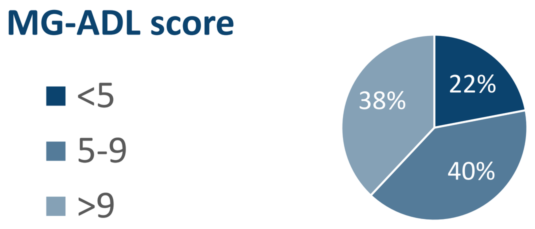
Patient Demographics (n=202)

Current MG characteristics

Current MG characteristics	
Generalized muscle weakness	100%
Speech and swallowing	63%
Ocular	71%

mean **9.5** years since gMG diagnosis

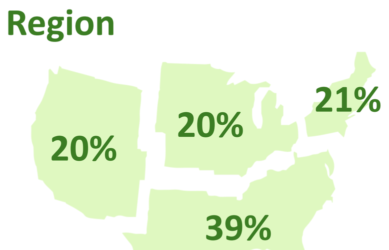
MG-ADL score



Provider demographics (n=140)

Role, n (%)	
Neurologist	53 (38%)
Neuromuscular specialist	46 (33%)
Nurse practitioner or physician assistant	40 (29%)

Region



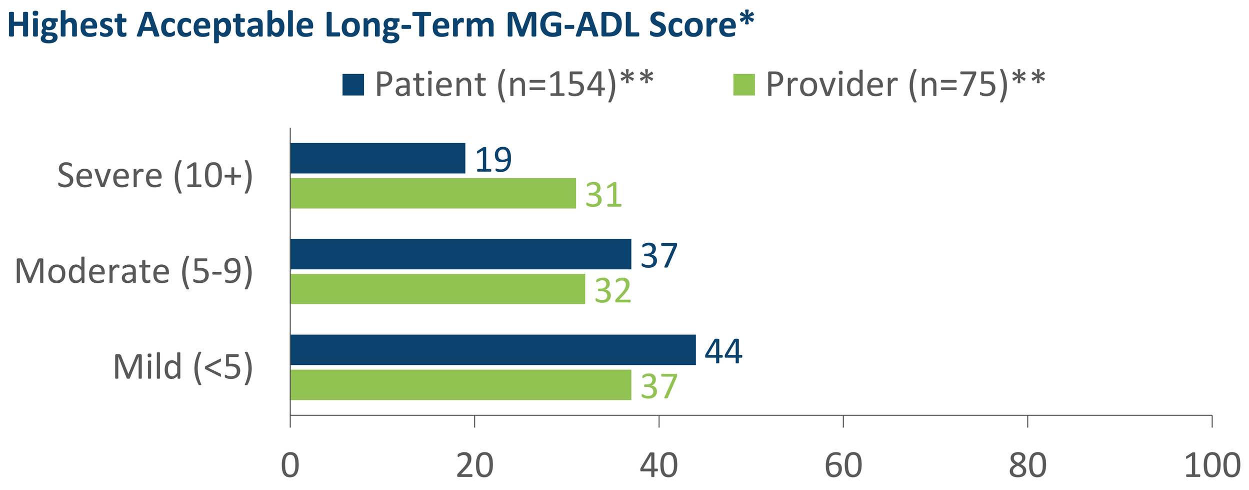
Manage average of **36** patients

Proportion of patients within each type of MG

gMG	73%
Ocular MG	27%
Other MG	8%

gMG, generalized MG; MG, myasthenia gravis; MG-ADL, Myasthenia Gravis Activities of Daily Living scale. "Other MG" was not specified.

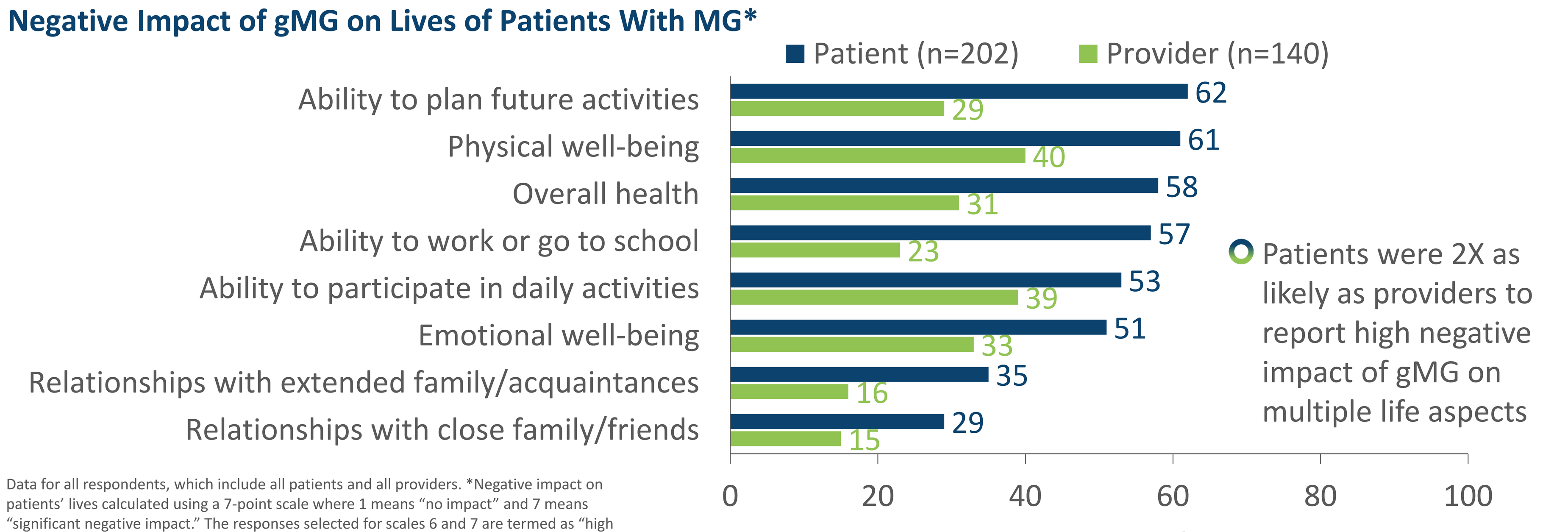
Highest Acceptable Long-Term MG-ADL Score*



- 40% of patients reported high disease burden
- 21% of providers reported their patients had high disease burden
- Patients & providers agreed that lower MG-ADL scores meant well-managed disease. However, 19% of patients and 31% of providers perceived scores of 10+ (severe) as the highest acceptable long-term score

Data for all respondents. *MG-ADL score ranges from 0-24, where "0" means "no MG symptom expression" and "24" means "severe MG symptom expression."
**Patients (24%, n=48) and providers (46%, n=65) who selected "not sure" were excluded from the analysis. MG-ADL, Myasthenia Gravis Activities of Daily Living scale.

Negative Impact of gMG on Lives of Patients With MG*

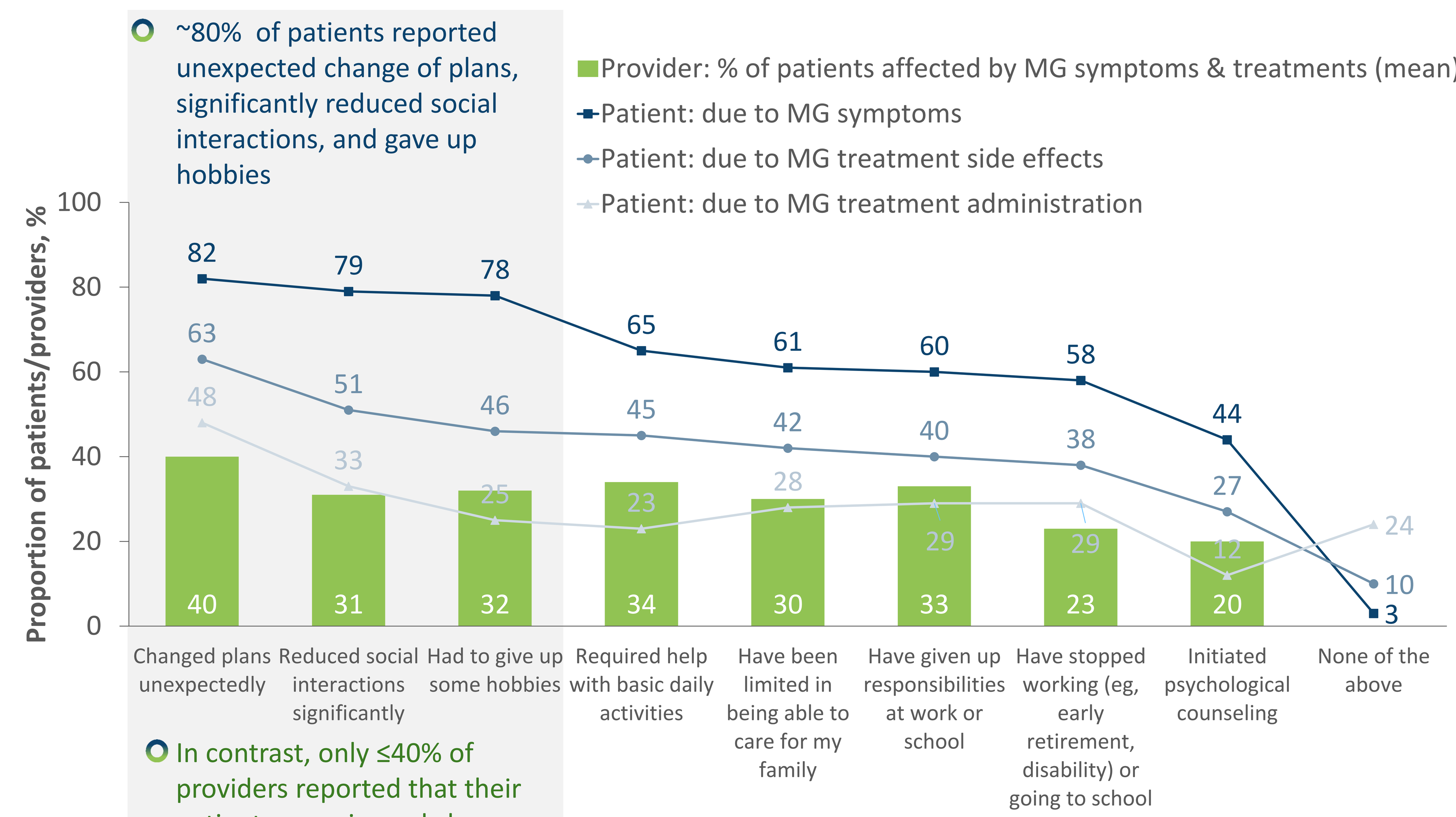


- Patients were 2X as likely as providers to report high negative impact of gMG on multiple life aspects

Data for all respondents, which include all patients and all providers. *Negative impact on patients' lives calculated using a 7-point scale where 1 means "no impact" and 7 means "significant negative impact." The responses selected for scales 6 and 7 are termed as "high negative impact." Patients and providers who selected scales 6 and 7 for the various attributes are represented here. gMG, generalized MG; MG, myasthenia gravis.

Effect of MG Symptoms, Treatments, and Side Effects on Patients' Lives*
(Patient n=202; Provider n=117)**

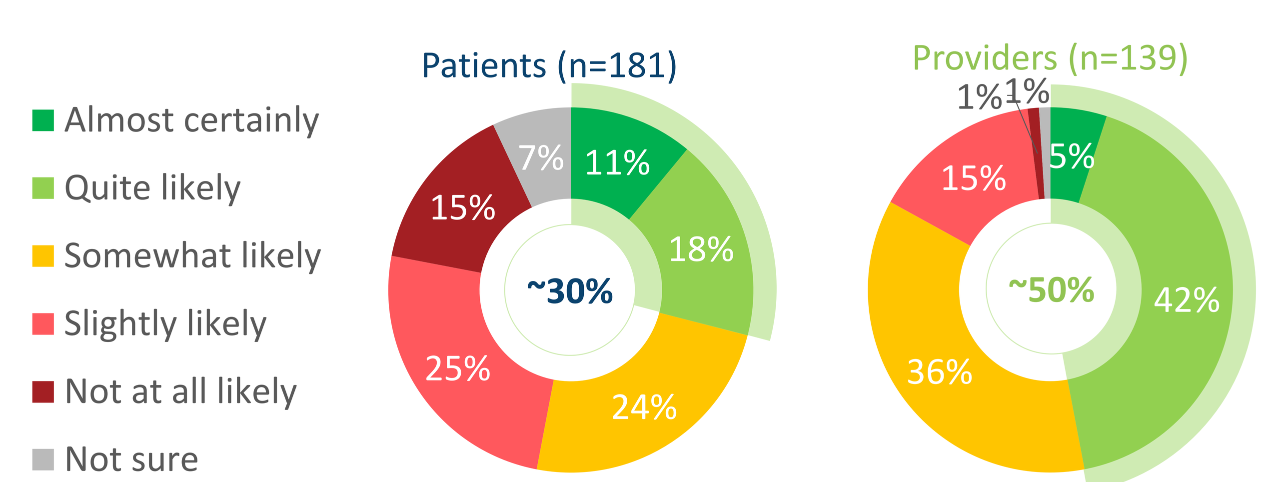
- ~80% of patients reported unexpected change of plans, significantly reduced social interactions, and gave up hobbies



- In contrast, only ≤40% of providers reported that their patients experienced changes in those activities

*The attributes on the x-axis represent patients' perspectives of how MG symptoms, treatments, and side effects affect their lives and providers' perspectives of how their patients are affected by MG symptoms and treatment.
**The "n=117" for provider represents the highest response received for an attribute after excluding "not sure" responses. The "n" size for each attribute varied due to varying number of providers selecting "not sure" responses for each attribute; therefore, the "n" range is 93-117 after excluding "not sure." MG, myasthenia gravis.

Likelihood of Achieving Remission or MSE*



- Only ~30% of patients and ~50% of providers responded that MSE was an achievable outcome
- 10% of patients reported achieving MSE, whereas 31% of providers estimated their patients achieved MSE

*Data for patients who were "not in remission" and providers whose patients were "not 100% in remission." MSE, minimal symptom expression.

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ABBREVIATIONS

gMG, generalized myasthenia gravis; MG-ADL, myasthenia gravis activities of daily living; MSE, minimal symptom expression; QoL, quality-of-life; SPSS, Statistical Package for the Social Sciences

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REFERENCES

1. Howard JF Jr, Barohn RJ, Cutter GR, et al. A randomized, double-blind, placebo-controlled phase II study of eculizumab in patients with refractory generalized myasthenia gravis. *Muscle Nerve.* 2013;48(1):76-84. doi:10.1002/mus.23839
2. Howard JF Jr, Utsugisawa K, Benatar M, et al. Safety and efficacy of eculizumab in anti-acetylcholine receptor antibody-positive refractory generalised myasthenia gravis (REGAIN): a phase 3, randomised, double-blind, placebo-controlled, multicentre study. *Lancet Neurol*. 2017;16(12):976-986. doi:10.1016/S1474-4422(17)30369-1
3. Muppidi S, Silvestri NJ, Tan R, Riggs K, Leighton T, Phillips GA. Utilization of MG-ADL in myasthenia gravis clinical research and care. *Muscle Nerve*. 2022;65(6):630-639. doi:10.1002/mus.27476
4. Vissing J, Jacob S, Fujita KP, O'Brien F, Howard JF; REGAIN study group. 'Minimal symptom expression' in patients with acetylcholine receptor antibody-positive refractory generalized myasthenia gravis treated with eculizumab. *J Neurol*. 2020;267(7):1991-2001. doi:10.1007/s00415-020-09770-y
5. Howard JF Jr, Nowak RJ, Wolfe GI, et al. Clinical effects of the self-administered subcutaneous complement inhibitor zilucoplan in patients with moderate to severe generalized myasthenia gravis: results of a phase 2 randomized, double-blind, placebo-controlled, multicenter clinical trial. *JAMA Neurol*. 2020;77(5):582-592. doi:10.1001/jamaneurol.2019.5125

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