# PCR71

# Does disease impact go beyond the healthcare system? Lessons from generalized myasthenia gravis 360°

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### INTRODUCTION

- In the USA, an estimated 10,000 rare diseases (RDs) affect ~10% of the population
- There may be substantial direct and indirect financial and societal burdens on patients and unpaid caregivers due to RDs<sup>2</sup>
- Both groups may experience emotional and financial challenges due to inability of patients to function independently in their daily lives<sup>3</sup>
- Myasthenia gravis (MG) is a debilitating and chronic autoimmune neuromuscular RD<sup>4–6</sup>
- Patients with MG exhibit fluctuating muscle weakness, usually involving several groups such as ocular, bulbar, axial, and respiratory muscles<sup>5,7</sup>
- MG often begins with ocular muscle weakness, and ~80% of patients who progress to more severe generalized myasthenia gravis (gMG) do so within 2 years of disease onset<sup>5,7,8</sup>
- ~90% of patients with gMG are acetylcholine receptor acetylcholine receptor antibody-positive (AChR+)<sup>7</sup>
- Studies on the burden of RDs typically focus on direct medical costs borne by payers;9 this study aims to provide a holistic assessment of the impact of gMG on patients and caregivers



# **OBJECTIVE**

• To investigate the impact of gMG on patients and caregivers using a mixed-methods approach with a focus on qualitative outcomes



# CONCLUSIONS

- This study demonstrates the extensive impacts of gMG on both patients and caregivers, illustrating the breadth of the disease burden
- Caregivers reported similar levels of impact as patients, thereby suggesting that including patient experience alone may underestimate total impact to families and other caregivers
- Patients and caregivers reported 16 new impacts, which may represent a considerable burden, e.g., lost productivity or financial struggles
- Direct cost of care assessed via healthcare expenditure claims is likely underreported
- Many non-quantifiable impacts are paid for by patients, families or caregivers, and are not covered by health insurance
- The broad impact of gMG that extends to both patients and caregivers should be considered in determining the value of treatments
- To comprehensively assess the burden of disease, we suggest that payers and funding agencies consider patient and caregiver perspectives during the development of benefit coverage and utilization review guidelines, and in individual reimbursement decisions
- Limitations of this study include small sample size (which may not capture the diversity of patients with gMG), possible bias arising from self-selection of participants, and the fact that severity of gMG was not captured, only time from diagnosis

- Eligible participants were either patients with AChR+ gMG or unpaid caregivers
- Patient and caregiver interviews were conducted to identify impact elements; findings analyzed qualitatively to summarize key themes (Table 1)
- Positive and negative impacts were captured; novel impacts not previously described in the literature were highlighted
- Impacts were quantifiable if they could be expressed or measured as a quantity (e.g., cost of services or supplies, hours, direct or indirect medical costs, or foregone income)

caregiver and therefore ineligible for the study

**Table 2** for participant baseline demographics

Table 2. Baseline demographics of study participants

AChR+ status

Characteristic, n (%)

Time from diagnosis\*

Hispanic/Latino

N/A, not applicable.

Not Hispanic/Latino

American Indian/Alaskan Native

Has a caregiver

<5 years

>5 years

Race

• 30 interviews (17 patients and 13 caregivers) completed using the respective interview guide;

- 17 patient interviews conducted; 1 interview excluded from the study due to unknown

- 13 caregiver interviews conducted; 1 interview excluded after learning they were a paid

Interviews conducted July 19, 2022–September 1, 2022; all participants based in the USA. See

\*Time from diagnosis was recorded in September 2022. Patients diagnosed before or on August 31, 2017 were recorded as >5 years, and patients

diagnosed from September 1, 2017 were recorded as <5 years; †For caregivers, time from diagnosis was reported for the patient they cared for;

<sup>‡</sup>Patients and caregivers were recruited independently which may explain the disparity in patient and caregiver numbers.

• Patients and caregivers appear to be impacted by gMG to a similar extent (**Figure 1**)

- Patients often described gMG as an "invisible disease" and expressed concerns about

- Caregivers expressed guilt when describing the negative impacts of gMG on their lives

- Greater proportion of caregivers impacted in 5 of the 8 domains when compared with patients

• Impact elements categorized into 8 domains (Figure 1)

Figure 1. Impact domains reported by patients and caregivers

burdening those around them

and tried to "look on the bright side"

28 met the inclusion criteria and were included in the analysis (16 patients and 12 caregivers)

- Reported impacts classified at 3 hierarchical levels:
- Domain (captures multiple impact elements, e.g., occupation)

- Impact element (captures multiple impacts, e.g., career aspirations)

**Patients** 

(n=16)

8 (50)

8 (50)

5 (31)

11 (69)

7 (44)

6 (38)

1 (6)

16 (100)

13 (81)

- Impact (captures higher levels of nuance, e.g., passing up career opportunities)

Caregivers

N/A

6 (50)†‡

6 (50)†

5 (42)

Patients (n=16)

Caregivers (n=12)

# Table 1. Study methodology

• A literature review informed creation of interview guides (one for patients and one for guide caregivers) to elicit a deeper understanding of the impact of gMG

recruitment

**METHODS** 

- Participants recruited in partnership with the Schlesinger Group and through patient advocacy groups; screening surveys ensured standardized inclusion and exclusion criteria were met: - Patient AChR+ status self-attested or confirmed
- via a provider note
- Eligible caregivers defined as those providing unpaid support

completion and transcript processing

development

and interview

analysis

- Patient and caregiver interviews completed using the respective interview guide via a web-based platform
- Interview transcripts generated and cleaned; identifying information removed before coding

Patients and caregivers treated as distinct groups

 Codebook created based on the interview guides 4. Codebook

throughout the study

 Interview transcripts coded using NVivo® software; key themes across transcripts were identified for final analysis

and refined after interviews were completed

AChR+, acetylcholine receptor antibody-positive; gMG, generalized myasthenia gravis.

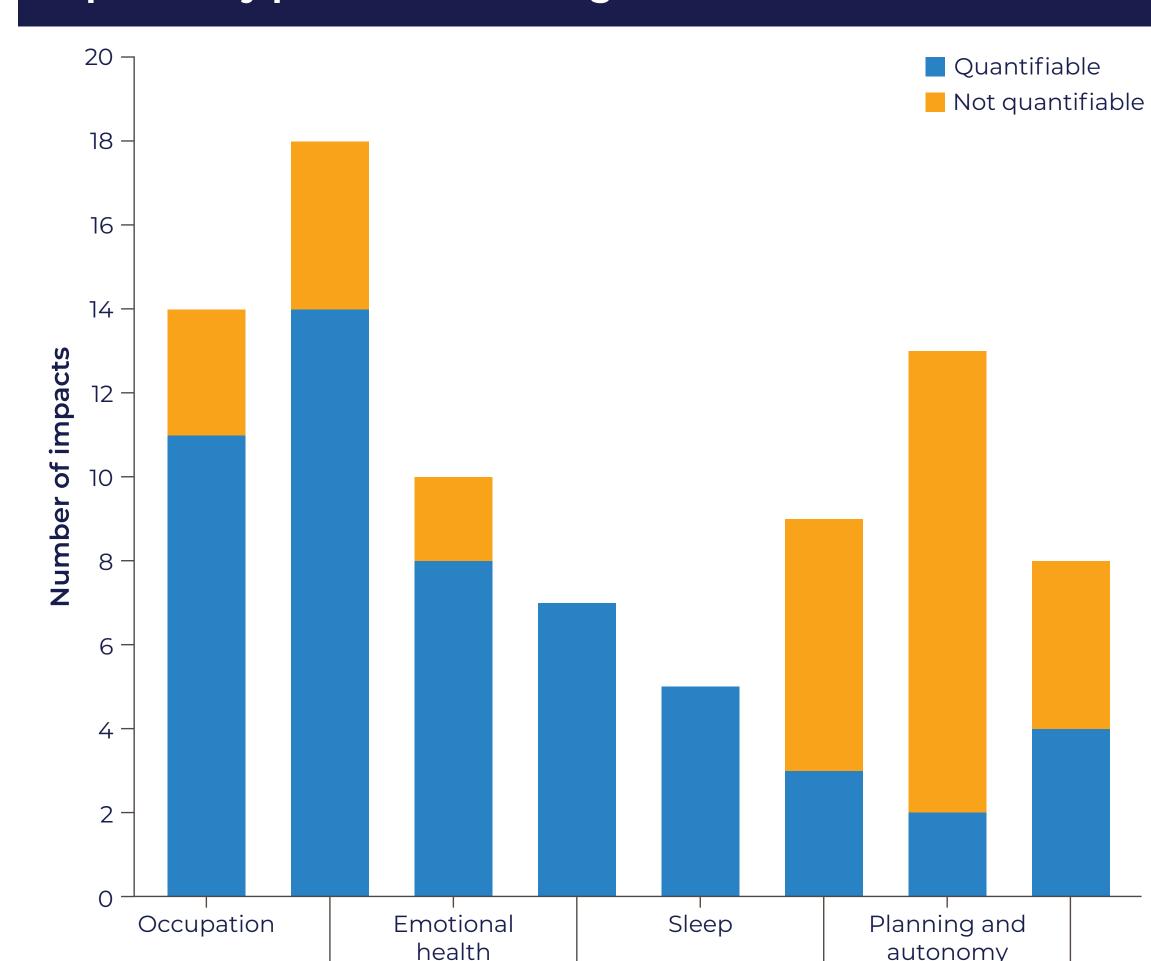
### **RESULTS**

- Interviewees described 84 unique impacts of gMG, including 16 not previously described in the literature (**Table 3**)
- Most impact elements were experienced by both patients and caregivers; however, they may be experienced differently (**Table 3**)
- Impacts classified as quantifiable or not quantifiable; overall, most impacts reported by patients and caregivers were quantifiable (Figure 2)

### Table 3. Domains, impact elements, and examples of impacts from patient and caregiver interviews **Impact elements +16 new** (total: 84) **+11 new** (total: 47) Patient examples Caregiver examples Career aspirations, freedom of occupation, unemployment or underemployment Being "stuck" Passing up job on-the-job disruption, productivity and performance, absenteeism, and in a role opportunities educational disruption Reduced income, financial trade-offs, financial toxicity, out-of-pocket costs, **Needing to hire** Taking on additional Financial\* cost of daily living, and high-cost expenses to support quality of life household support dependents Anger or resentment, fear or anxiety, frustration, sadness or depression, shame **Emotional** Shame or or embarrassment, guilt, stress, loss of identity, impaired cognitive function, Guilt embarrassment and disproportionate sense of responsibility **Physical** Lack of focus on Downstream Neglecting health needs and downstream health impacts personal health health impacts Irregular sleep Not sleeping Sleep\* Insomnia, quality of sleep, and reliance on sleep aid schedule comfortably Strain or change to intimate, immediate, or non-immediate relationships, social Difficulty forming Social Social isolation isolation, reduced ability or desire to participate in activities across varying sectors, new friendships real or perceived negative public perception, and poor public understanding Vigilance, disruptions to plans, loss of autonomy, feelings of instability about the Lack of Altering of future, personal aspirations, necessary life adaptations, and illness work (e.g., time independence personal priorities spent managing insurance, medical providers, and other healthcare needs) Real or perceived physical safety risks, real or perceived medical mistreatment, Risk of experiencing Risk and experience medical mistreatment and **powerlessness** of injuries Impact elements and impacts in bold pink format have not been previously reported.

\*Domains split from original "Physical health and sleep", and "Occupation and financial".

# Figure 2. Quantifiable and not quantifiable impacts reported by patients and caregivers



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