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EE286

Costs and Healthcare Resource Utilisation Associated with Generalised Myasthenia Gravis: A Systematic Literature Review

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KEY FINDINGS & CONCLUSIONS

- The review underscores the significant economic burden and HCRU associated with gMG, particularly in refractory cases and during disease exacerbations and crisis events.
- Patients with gMG often face substantial declines in work productivity, especially those with advanced disease (MGFA class III–IV). The condition also significantly affects caregivers, many of whom modify their employment by reducing hours or leaving the workforce altogether to meet their caregiving responsibilities.
- Further research is needed to generate data on direct and indirect cost burden in regions such as Europe and Asia, and also to understand the economic impact on gMG subpopulations based on serotype status.

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INTRODUCTION

- Generalised myasthenia gravis (gMG) is a chronic autoimmune disorder affecting the neuromuscular junction, characterised by fluctuating muscle weakness and fatigue.[1]
- gMG imposes a significant economic burden, driven by long-term pharmacotherapy, regular specialist monitoring, and, in severe cases, hospitalisation or intensive-care support.[2,3]
- This systematic literature review (SLR) was performed to identify and collate the published evidence on the costs and healthcare resource use (HCRU) associated with gMG.

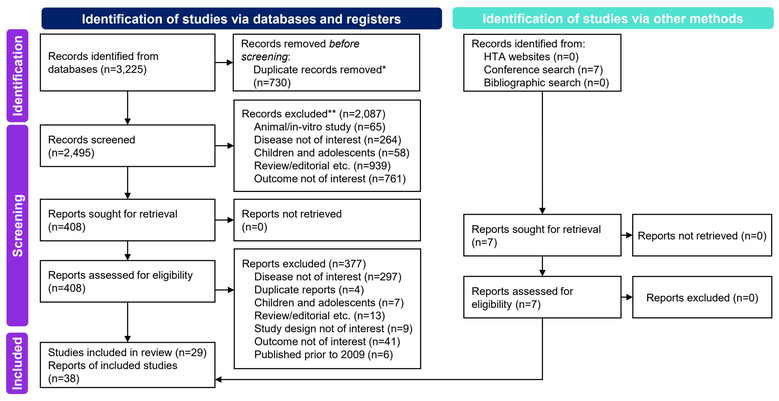
METHODS

- A comprehensive literature search was performed on the Ovid platform across Embase®, Medline® and National Health Service (NHS) Economic Evaluation Databases to retrieve evidence published in the last 15 years (2009–April 2024).
- To ensure a comprehensive evidence base, supplementary searches included conference abstracts (2021 onward), health technology assessment (HTA) websites, and reference lists of relevant SLRs.
- Inclusion criteria encompassed English-language studies involving adults with gMG that reported outcomes related to direct costs, indirect costs (e.g., productivity loss), and HCRU.
- Quality assessment of the included studies was performed using National Institutes of Health (NIH) Quality Assessment Tool for Observational Cohort and Cross-Sectional Studies.[4]

RESULTS

- A total of 29 studies from 38 publications were included (**Figure 1**). Additionally, eight relevant HTA reports were identified; most of these reported only model input costs/HCRU.

Figure 1. PRISMA flow chart: Flow of studies through SLR stages



Abbreviations: HTA, health technology assessment; PRISMA, Preferred Reporting Items for Systematic Reviews and Meta-Analyses; SLR, systematic literature review.
Notes: *De-duplication was done using the Ovid search platform. **No automation tools were used. All the titles/abstracts were screened manually.

Description of included studies

- Most studies were conducted in single countries, led by the United States (US; n = 11), followed by Iran and Taiwan (n = 2 each), and then the United Kingdom (UK), Spain, Italy, China, Japan, Australia and India (n = 1 each). Seven studies were conducted in multiple countries.
- Most studies (n = 20) targeted the overall gMG population; others addressed acetylcholine receptor antibody–positive (AChR-Ab+; n = 3) gMG, refractory vs. non-refractory gMG (n = 3), gMG with exacerbations (n = 2), and suboptimally controlled gMG (n = 1).
- The majority of the studies (n = 24) reported HCRU, while a few (n = 6) reported healthcare costs associated with gMG.

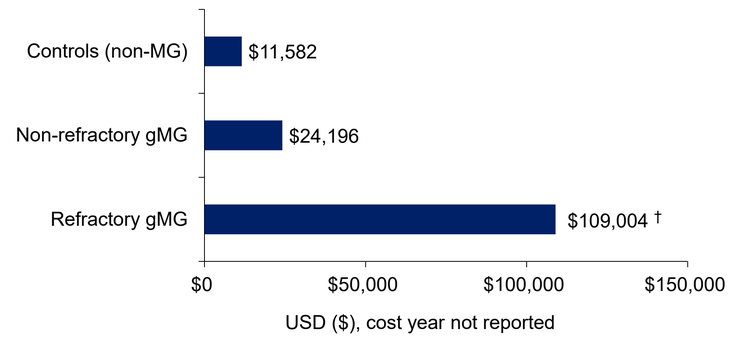
Healthcare costs

In the US, gMG is associated with high economic burden—largely attributable to initial diagnosis, refractory cases, and exacerbation or crisis events:

- A retrospective study by Engel-Nitz et al. (2018) reported that refractory patients incurred > 4 times higher costs than non-refractory patients and almost 10 times higher costs than individuals without MG (**Figure 2**).[5]
- Another retrospective study by Phillips et al. (2022) reported that newly diagnosed patients incurred higher annual all-cause costs, followed by previously diagnosed patients, with costs peaking at $43,734 among those experiencing exacerbation events (**Figure 3**). Among the crisis subgroup, total costs increased during the year preceding the crisis event compared with the prior 2 years and rose further in the year following the crisis (**Figure 4**).[6]
  - The elevated post-crisis costs were primarily attributed to disease progression requiring intensive therapies such as intravenous immunoglobulin/subcutaneous immunoglobulin (IVIg/SCIg), with mean 1-year post-crisis costs reaching $12,488. For comparison, mean 1-year post-crisis drug costs were as follows: eculizumab, $6,949; plasma exchange (PLEX), $2,412; rituximab, $1,689; acetylcholinesterase inhibitors $884; non-steroidal immunosuppressive therapies, $594; and corticosteroids, $59.[6]
- This finding was further supported by a retrospective study by Qi et al. (2022), which reported medical costs (USD, 2018) of up to $161,478 per patient per year (PPPY), with $133,155 (IVIg cost) for chronic use versus $64,888 (annual medical costs) and $35,202 (IVIg cost) for intermittent use ($p < 0.001$ for both).[7]

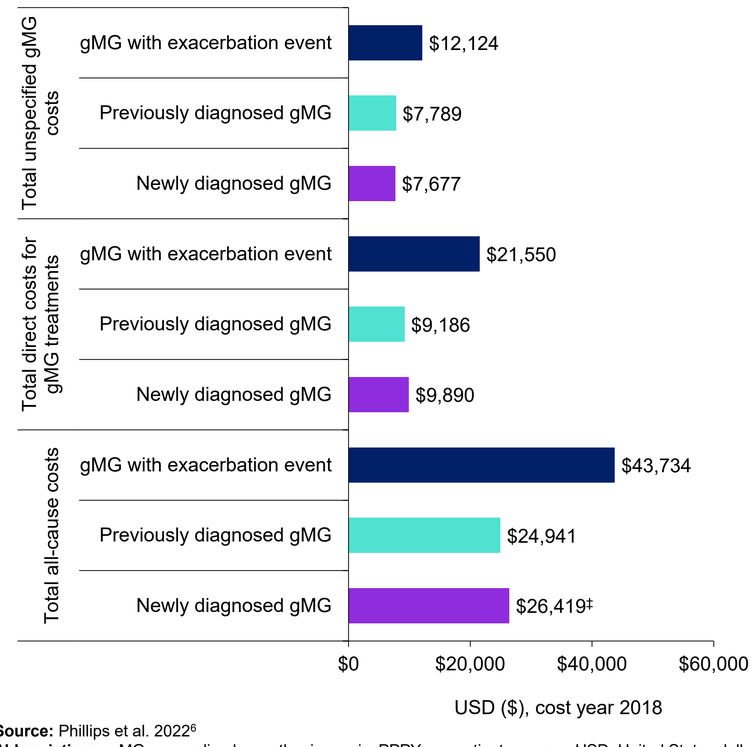
Limited data were available from other key markets.

Figure 2. Annual healthcare costs in refractory gMG*, non-refractory gMG, and non-MG controls (USD, cost year not reported)



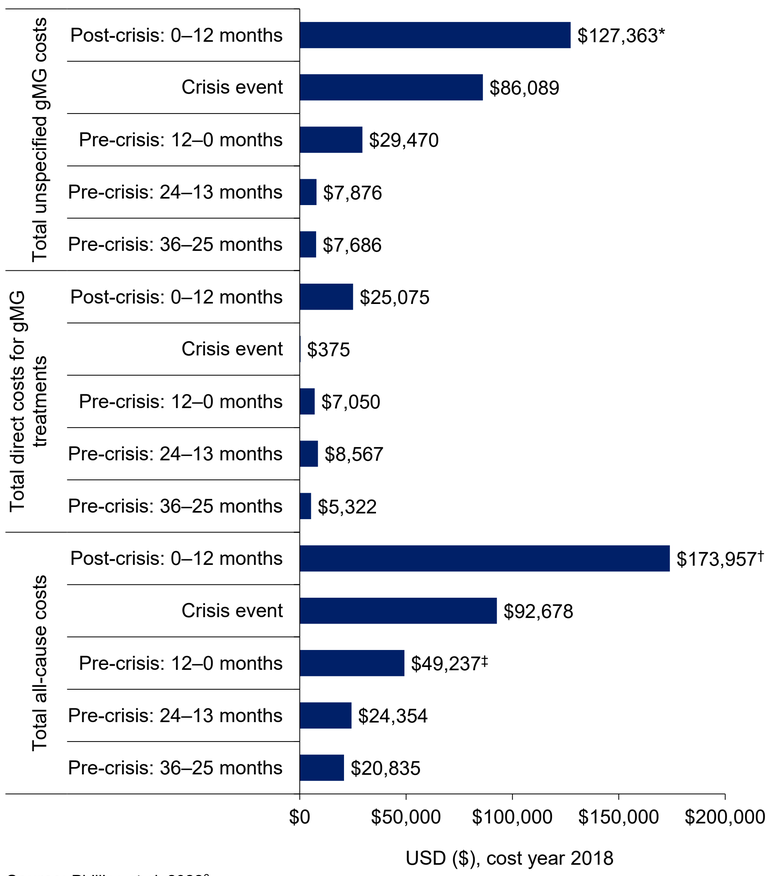
Source: Engel-Nitz et al. 2016[5]
Abbreviations: gMG, generalised myasthenia gravis; IST, immunosuppressive therapies; MG, myasthenia gravis; USD, United States dollars.
Notes: *Patients with refractory gMG were those with any of the following: 1) ≥ 3 ISTs (including oral corticosteroids) within 2-years; 2) ≥ 1 IST (including oral corticosteroids) plus ≥ 1 therapy reserved for MG resistant to conventional therapy; 3) regular treatment with PLEX (≥ 6 claims within 1-year). IVIg was excluded from the definition of refractory because maintenance use could not be distinguished from fast-acting "bridge" therapy. †$p < 0.001$ vs. non-refractory gMG and vs. controls.

Figure 3. Standardised mean PPPY cost in patients with gMG who were newly diagnosed*, previously diagnosed†, and had exacerbation events (USD, cost year 2018)



Source: Phillips et al. 2022[6]
Abbreviations: gMG, generalised myasthenia gravis; PPPY, per patient per year; USD, United States dollars.
Notes: Costs were calculated as standardised mean (total costs divided by the number of patients in the cohort). *Newly diagnosed refers to those diagnosed with gMG during the study period (January 2017 to December 2018). †Previously diagnosed refers to those diagnosed prior to the study period. ‡$p < 0.001$ vs. previously diagnosed patients.

Figure 4. Standardised mean PPPY cost before and after a crisis event (USD, cost year 2018)



Source: Phillips et al. 2022[6]
Abbreviations: Costs were calculated as standardised mean (total costs divided by the number of patients in the cohort). gMG, generalised myasthenia gravis; PPPY, per patient per year; USD, United States dollars.
Notes: *$p < 0.001$ vs. pre-crisis. †Period including the crisis event duration; $p < 0.001$ vs. pre-crisis. ‡$p < 0.001$ vs. pre-crisis: 24–13 months.

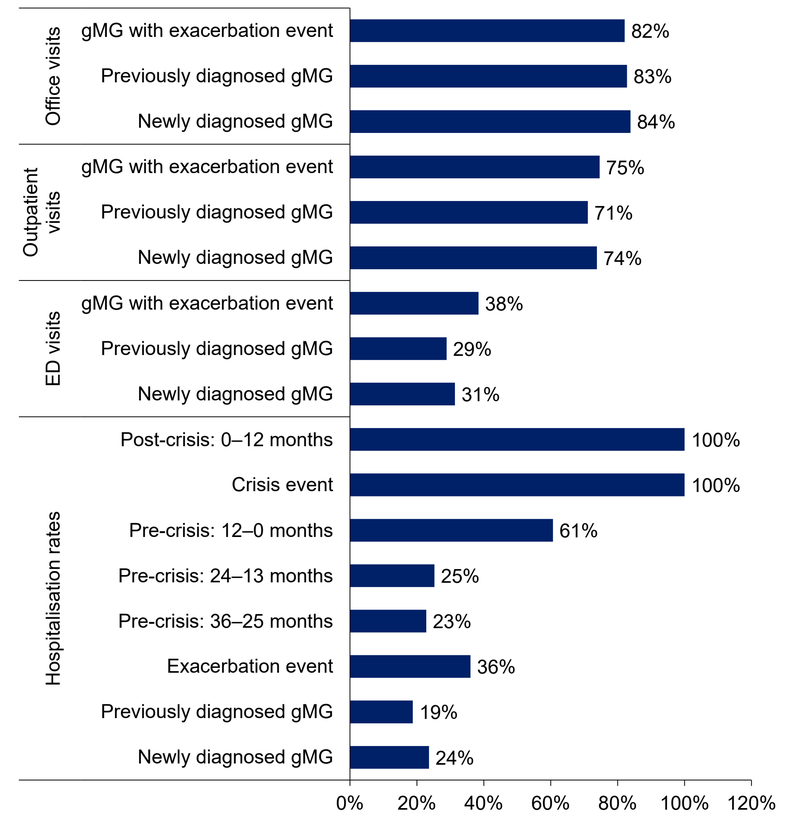
HCRU

Patterns of HCRU are consistent with economic burden findings, indicating higher utilisation among patients with new diagnoses, treatment-resistant disease, and acute events.

US

- A study by Phillips et al. (2022) revealed that new diagnosis, exacerbation, and crisis events resulted in increased hospitalisations and emergency department (ED) and outpatient visits (**Figure 5**). Length of stay (LoS) ranged from 0.99 days for patients with previously diagnosed gMG to 15.38 days for those experiencing crisis events.[6]
- A study by Engel-Nitz et al. (2018) reported that patients with refractory gMG had higher hospitalisation rates and longer LoS per year (52.1%; 10.7 days) than those with non-refractory gMG (23.6%; 3.7 days) and controls without medical claims for MG (18.6%; 1.7 days) ($p < 0.001$ for both).[8] These findings were substantiated in a study by Harris et al. (2020), wherein patients with ever-refractory gMG consistently had higher ED visits and intensive care unit (ICU) use than non-refractory patients, except at baseline.[9]
  - ICU admission rates (timepoint): 24% vs. 34% (baseline, $p = 0.07$); 12% vs 4% (< 1 year); 7% vs 3% (1–2 years); 6% vs 2% (2–3 years); and 5% vs 1% (3–4 years).[9]
- A cross-sectional survey by Mahic et al. (2022) found that there was an increased number of consultations with an increase in disease severity: mean number of consultations was 4.8 for Myasthenia Gravis Foundation of America (MGFA) class IIa, 5.4 for class IIb, 6.0 for class IIIa and IIIb, 6.4 for class IVa, and 20 for class IVb.[10]

Figure 5. Annual HCRU in newly diagnosed* gMG, previously diagnosed† gMG, gMG with exacerbation, and gMG crisis groups‡



Source: Phillips et al. 2022[6]
Abbreviations: ED, emergency department; gMG, generalised myasthenia gravis; HCRU, healthcare resource utilisation.
Notes: *Newly diagnosed refers to those diagnosed with gMG during the study period (January 2017 to December 2018). †Previously diagnosed refers to those diagnosed prior to the study period. ‡$p < 0.001$ (HCRU was higher in gMG with exacerbation vs. newly diagnosed gMG and previously diagnosed gMG groups; HCRU was higher during the 12 months immediately preceding the crisis events vs. 36–25 months and 24–13 months leading up to the crisis index date).

Non-US

- In a cohort study of **AChR-Ab+ gMG** patients in **Japan** (Kataoka et al. 2014), 21 patients underwent thymectomy only and 16 underwent thymectomy with perioperative steroids; those who received steroids had a shorter mean postoperative ICU stay (2.93 [SD: 1.52] days) than those who underwent thymectomy only (5.09 ± 4.82 days) ($p = 0.329$).[11]

Work productivity and activity impairment

- Two studies utilised data from the Adelphi gMG Disease Specific Programme, a cross-sectional survey conducted across **five European countries and the US:**
  - One study revealed that patients with more severe gMG (MGFA class III–IV) experienced 14.3% greater work impairment ($p = 0.01$) and 14.8% higher overall work impairment ($p = 0.04$), as well as a 15.5% increase in impairment in daily activities ($p < 0.01$), compared with those with MGFA class II disease.[12]
  - Another study emphasised the burden on caregivers, reporting that 23% reduced their working hours and 14% ceased working altogether due to caregiving responsibilities for patients with gMG.[13]
- A **UK-based analysis** that combined ADAPT trial and MyRealWorld-MG study data found that efgartigimod + conventional therapy reduced sick leave by 21% and caregiver worktime losses by 16%, resulting in annual productivity savings of £3,165 per patient with gMG.[14]

Quality assessment

- According to the NIH Quality Assessment Tool, the quality of all the included studies was fair.

References

1. Phillips WD et al. F1000Res. 2016;5:F1000 Faculty Rev-1513.
2. Saccà F et al. Eur J Neurol. 2024;31(6):e16180.
3. Sonkar KK et al. J Clin Neurosci. 2017;38:114–117.
4. National Institutes of Health. Study Quality Assessment Tools. URL: https://www.nhlbi.nih.gov/health-topics/study-quality-assessment-tools. Accessed 1 June 2024.
5. Engel-Nitz NM et al. J Neuromuscul Dis. 2016;3(Suppl 1):S198–S199.
6. Phillips G et al. Front Neurol. 2022;12:809999.
7. Qi CZ et al. J Neurol Sci. 2022;443:120480.
8. Engel-Nitz NM et al. Muscle Nerve. 2018;58(1):99–105.
9. Harris L et al. J Clin Neuromuscul Dis. 2020;22(1):11–21.
10. Mahic M et al. Neurol Ther. 2022;11(4):1535–1551.
11. Kataoka H et al. Eur Neurol. 2014;72(3-4):228–233.
12. Pesa J et al. Neurology. 2023;100(17_supplement_2):1787.
13. Kleman M et al. Eur J Neurol. 2022;29(Suppl 1):612.
14. Saccà F et al. J Neuromuscul Dis. 2022;9(Suppl 1):S108–S109.

Disclosures

This study is sponsored by Novartis Pharma AG. **Rambabu Vatte** and **Mohit Kumar Bhutani** are employees of Novartis Healthcare Private Limited, Hyderabad, India. **Nicholas Kirvassilis** and **Nicholas Adlard** are employee of Novartis Pharma AG, Basel, Switzerland. **Rahul Khairnar** is an employee of Novartis Pharmaceuticals Corporation, Jersey City, NJ, USA.

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