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Quantifying the lifetime health and societal benefits of timely diagnosis and access to approved treatments in anti-acetylcholine receptor antibody-positive generalized myasthenia gravis in the United States

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INTRODUCTION

- Anti-acetylcholine receptor antibody-positive (AChR-Ab+) generalized myasthenia gravis (gMG) is a rare, chronic, autoimmune neuromuscular disease characterized by muscle weakness, variable disease course, and fluctuating symptoms.[1]
- Compared with the general population, patients with AChR-Ab+ gMG experience increased mortality and worse health-related quality of life outcomes[2,3]; gMG also imposes substantial socioeconomic burden on both patients and their care partners.[4-6]
- Patients with gMG frequently encounter diagnostic delays and barriers to accessing approved treatments,[7,8] which can lead to avoidable disease progression and societal productivity losses.[9]
- Understanding the impact of earlier diagnosis and earlier access to approved treatment may inform optimal gMG treatment strategies to improve patient outcomes and reduce economic burden.[9]

OBJECTIVE

- To quantify the lifetime health and societal benefits of earlier diagnosis and earlier access to approved treatments for AChR-Ab+ gMG in the United States.

CONCLUSIONS

- The results of this cohort modelling study indicate that while earlier diagnosis alone produces meaningful health benefits, when combined with earlier access to approved treatments, greater reductions in crises and exacerbations and improvements in overall health quality and functional status are achieved among patients, with corresponding societal productivity gains for both patients and care partners.
- Earlier diagnosis and earlier access to targeted treatment have the potential to substantially improve long-term health outcomes for patients with AChR-Ab+ gMG, while also generating meaningful socioeconomic benefits among patients and care partners in the United States when compared with the status quo.
- Collectively, these findings demonstrate that earlier diagnosis combined with earlier access to effective, approved targeted treatments is essential for optimizing patient health benefits and generating substantial societal productivity gains.

References

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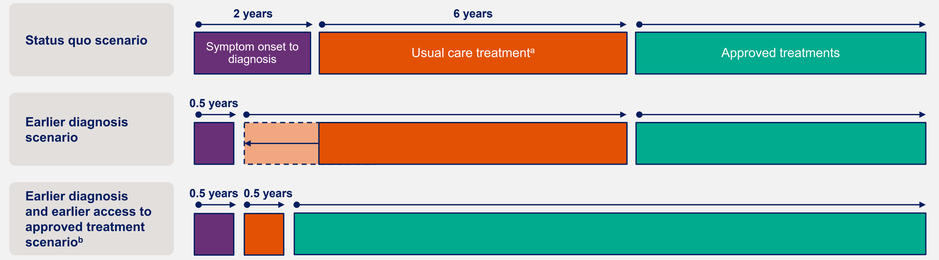
METHODS

- Disease course from symptom onset until death over a 20-year horizon in a cohort of hypothetical patients with AChR-Ab+ gMG was modeled using changes in Myasthenia Gravis Activities of Daily Living (MG-ADL) score to track the disease course.
  - Patients were assumed to receive usual care (steroidal/nonsteroidal immunosuppressive therapies) before FDA-approved treatments.
  - Use of FDA-approved therapies in 2025 was modeled using a weighted basket approach.
  - The analysis was conducted among patients with MG-ADL score > 5.0 receiving approved therapies, assuming 27.6% of all patients are eligible for approved treatments.
- Inputs for the model, such as the time from symptom onset to diagnosis, time from diagnosis to treatment, and societal costs were informed by findings from surveys and interviews among patients with AChR-Ab+ gMG and their care partners; clinical inputs and healthcare costs were informed by published literature.
  - The model assumptions were informed by published sources, real-world evidence, and expert input from a physician treating patients with AChR-Ab+ gMG.
- The model compared the following scenarios (**Figure 1**):
  - Status quo (2-year duration from disease onset to diagnosis; six years from diagnosis to treatment with approved therapies).
  - Earlier diagnosis (within 6 months of disease onset).
  - Earlier diagnosis and earlier access to approved treatments as denoted by earlier initiation of treatment by physicians, independent of reimbursement restrictions (each within 6 months of usual care).
- The following outcomes were assessed:
  - **The lifetime health impacts** for patients were measured by the number of crises, exacerbations, and quality-adjusted life-years (QALYs), as well as the number of life-years, since disease onset over a 20-year horizon, spent within categories defined by MG-ADL scores.
  - **Annual and lifetime societal productivity gains** (reduction in absenteeism, improvement in presenteeism, and preserved earnings through delay or avoidance of early retirement) among both patients and care partners.

Figure 1. Study scenarios



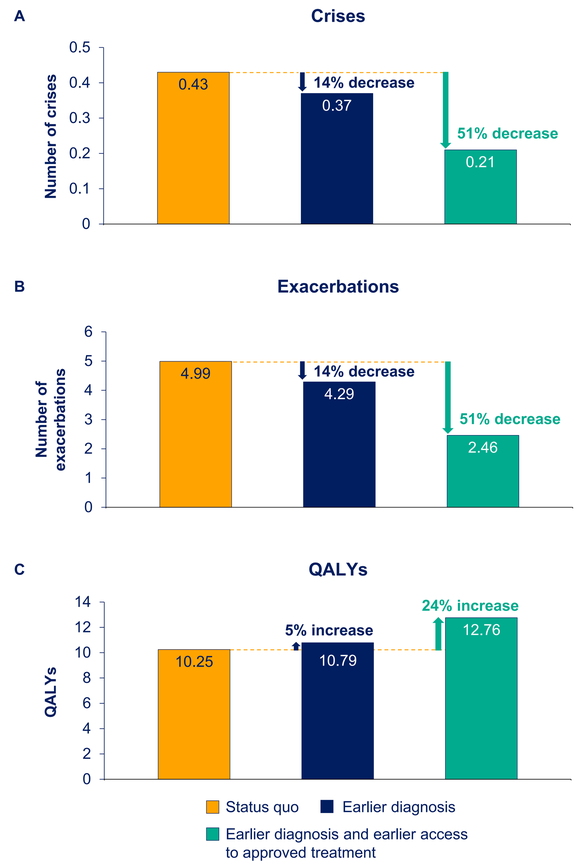
[a]Usual care includes nonapproved steroidal/nonsteroidal immunosuppressive therapies. [b]In this scenario, "earlier access" refers to patients receiving the approved treatment earlier in their disease progression at the discretion of the treating physician, rather than due to the removal of reimbursement restrictions.

RESULTS AND INTERPRETATION

Lifetime health and societal benefits

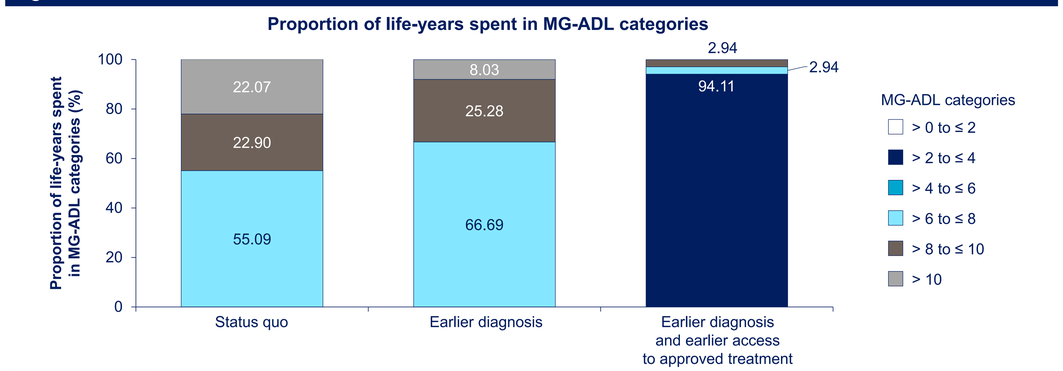
- Compared with the status quo, earlier diagnosis resulted in a 14% reduction in the number of crises, a 14% reduction in the number of exacerbations, and a 5% improvement in QALYs (**Figure 2**).
- Compared with the status quo, earlier diagnosis and earlier initiation of approved treatments resulted in a 51% reduction in the number of crises, a 51% reduction in the number of exacerbations, and a 24% improvement in QALYs (**Figure 2**).
- Compared with the status quo, diagnosis within 6 months of disease onset with earlier access to approved treatment resulted in a higher proportion of life-years spent with an MG-ADL score ≤ 6.0 (0% vs 94.11%) (**Figure 3**).
- Among patients and care partners, with earlier diagnosis alone, societal productivity gains amounted to $28.0 million/year nationally or $560.9 million/lifetime nationally (**Figure 4A**), with substantial increases when combined with earlier access to approved treatment, further amounting to $133.8 million/year nationally or $2.7 billion/lifetime nationally (**Figure 4B**).

Figure 2. Effect on lifetime health outcomes



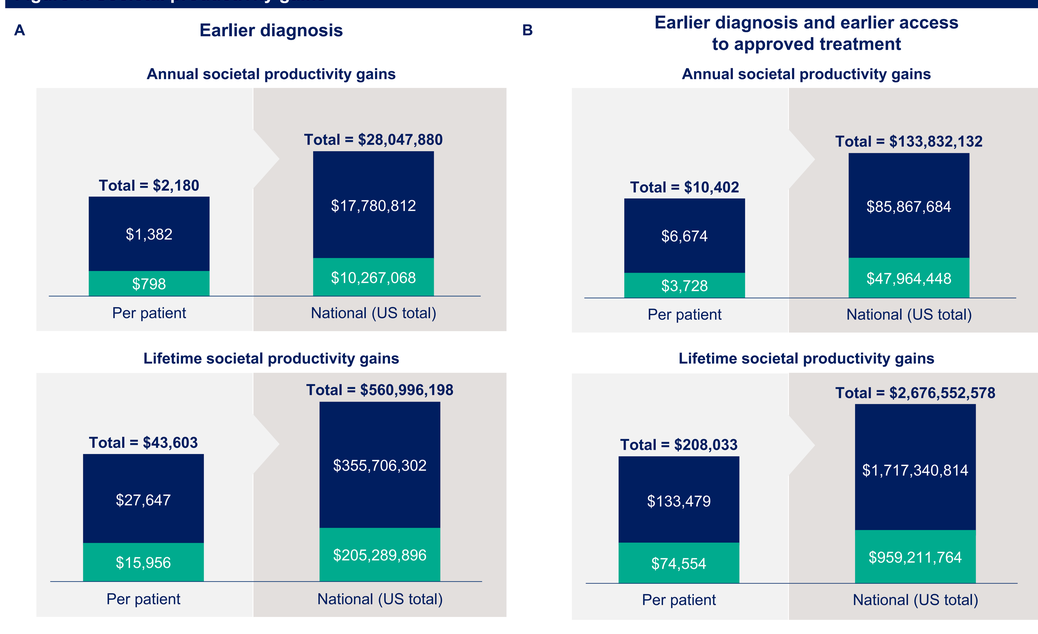
QALY, quality-adjusted life-year.

Figure 3. Effect on functional status



Percentages may not equal 100 due to rounding.
MG-ADL, Myasthenia Gravis Activities of Daily Living.

Figure 4. Societal productivity gains[a]



[a]Based on 12,866 patients with AChR-Ab+ gMG with MG-ADL score > 5.0 on approved therapies in the United States. USD have been discounted to the present term.
AChR-Ab+, anti-acetylcholine receptor antibody-positive; gMG, generalized myasthenia gravis; MG-ADL, Myasthenia Gravis Activities of Daily Living; USD, United States Dollar.

Study limitations

- The model is based on representative disease course, but real-world disease trajectories may vary between individuals; heterogeneity in patient-level data, including severity of crises and exacerbations, recovery, functional status, diagnosis timing, treatment initiation, and treatment effectiveness cannot be fully captured within a cohort-level framework.