RETHINKING MYASTHENIA GRAVIS

ADVOCACY PAPER

Rethinking Myasthenia Gravis Care through a Brain Health Lens: Adaptive Pathways and Patient-Centred Insights

EBC European Brain Council

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RE THINKING MYASTHENIA GRAVIS

List of Abbreviations

AChR	Acetylcholine Receptor
CBAs	Cell-Based Assays
EHDS	European Health Data Space
EHR	Electronic Health Records
EMA	European Medicine Agency
ERN	European Reference Network
ERN EURO-NMD	European Reference Network for Rare Neuromuscular Diseases
FcRn	Neonatal Fc Receptor
ICU	Inpatient Care Unit
IgG	Immunoglobulin G
HTA	Health Technology Assessment
LRP4	Low-Density Lipoprotein Receptor–Related Protein 4
MG	Myasthenia Gravis
MG Centres	Centres of Expertise/Specialist Centres for Myasthenia Gravis
MG-ADL	Myasthenia Gravis Activities of Daily Living (profile/scale)
MGII	Myasthenia Gravis Impairment Index
MG-QoL15r	Myasthenia Gravis Quality of Life 15-item revised (questionnaire)
MuSK	Muscle-Specific Kinase
PRO(s)	Patient-Reported Outcome(s)
PROM	Patient-Reported Outcome Measurement
PREM	Patient-Reported Experience Measurement
QMG	Quantitative Myasthenia Gravis (score)
SnMG	Seronegative Myasthenia Gravis
WHA	World Health Assembly
WHO	World Health Organization
WHO IGAP	World Health Organization Intersectoral Global Action Plan on Epilepsy and Other Neurological Disorders

Rethinking Myasthenia Gravis - Background

Rethinking Myasthenia Gravis is a two-year, multistakeholder, research-driven initiative (2025–2026) designed to develop evidence-based recommendations to improve care pathways and patient outcomes for people living with myasthenia gravis (MG) across Europe. The project forms part of the European Brain Council's (EBC) Rare Brain Disease Ecosystem flagship initiative, and is led by EBC in collaboration with the Institute of Management at the Sant'Anna School of Advanced Studies (SSSA) in Pisa, Italy. Bringing together researchers, clinicians, patient representatives, European Reference Networks (ERN), notably ERN EURO-NMD, and policymakers, the project aims for a coordinated, and future-oriented rethinking of MG care.

The initiative is organised into three phases:

- Phase 1 involves a systematic literature review on the socioeconomic burden of MG, patient care pathways and the adoption of digital tools in MG management. The results will be submitted as a scientific publication and presented at the European Academy of Neurology (EAN) Congress un June of 2026.
- Phase 2 represented in this advocacy paper – "Rethinking Myasthenia Gravis Care through a Brain Health Lens: Adaptive Pathways and Patient-Centred Insights" – was launched at the EBC Rare Disease Day event on 24 February, 2026.
- Phase 3 will focus on targeted policy outreach and stakeholder engagement, particularly during myasthenia gravis Awareness Month in June 2026.

Taken together, these phases seek to bridge scientific evidence, lived experience and policy dialogue, using MG as a concrete case study to rethink how European health systems can deliver more timely, equitable and brain health–oriented care for people living with rare neuromuscular diseases.

For more information about "Rethinking Myasthenia Gravis", please visit the project page.

Executive summary and key recommendations

> *"What people often have difficulty understanding is that the disease is always changing. One day you feel all right, the next day, you could go to the hospital… it's mentally exhausting."*
> — Person living with MG / Patient representative (France).

Myasthenia gravis (MG) is a rare autoimmune neuromuscular disorder that places a substantial clinical, socioeconomic, organisational, and mental health burden across Europe. Beyond muscle weakness and fatigability, MG is associated with broader brain health impacts, including persistent fatigue, cognitive strain, anxiety, depression, sleep disruption, and reduced social participation. These aspects are not consistently addressed in care models, which remain largely focused on neuromuscular symptom control.

Through the EBC Rare Brain Disease Ecosystem, we aim to bridge the brain health and rare disease community by aligning neuroscience, clinical innovation, and health policy. `Rethinking MG´ exemplified this mission: it brings together scientific evidence, patient perspectives, and policy insights to inform care pathways, access mechanisms, and regulatory decision-making across Europe. European and international agendas increasingly converge on common priorities: earlier and more accurate diagnosis, strengthened care pathways and centres of expertise, equitable access to effective treatments, high-quality data and registries, and coordinated action through European Reference Networks, the EU Health Technology Assessment (HTA) Regulation, and the European Health Data Space (EHDS). Against this backdrop, MG illustrates a twofold challenge. Scientific progress has accelerated, including improved antibody detection methods (notably cell-based assays) and a widening array of targeted immunotherapies, which have transformed the treatment landscape. Importantly, these innovations create new opportunities for steroid-sparing strategies—reducing long-term corticosteroid exposure and its cumulative risks, including metabolic, psychiatric and cognitive side effects that directly affect brain health and quality of life. Yet, health system adaptation has not kept pace. Persistent diagnostic delays, fragmented care pathways, uneven access to specialist centres and innovative therapies, and inconsistent integration of validated outcome measures and digital tools into routine care undermine the translation of innovation into day-to-day patient benefit. As a result, many policy commitments on rare diseases and brain health remain only partially realised in routine MG care.

This advocacy paper synthesises evidence from peer-reviewed and grey literature qualitative insights from stakeholder interviews, offering a community-informed analysis of the current landscape of MG care in Europe. Drawing on European data and examples from France, Germany, Italy and Poland, the paper underscores the urgent need for more coordinated and adaptive care pathways, wider use of real-world evidence, and systematic incorporation of patient-experienced data – including digital patient-reported outcomes – to support regulatory, HTA, and clinical decision-making. The paper highlights that rethinking MG care requires more than advancing therapeutic options. It calls for redesigning health system structures and processes, aligning incentives, and strengthening data infrastructures so that patients and caregivers fully benefit from emerging scientific and technological innovations.

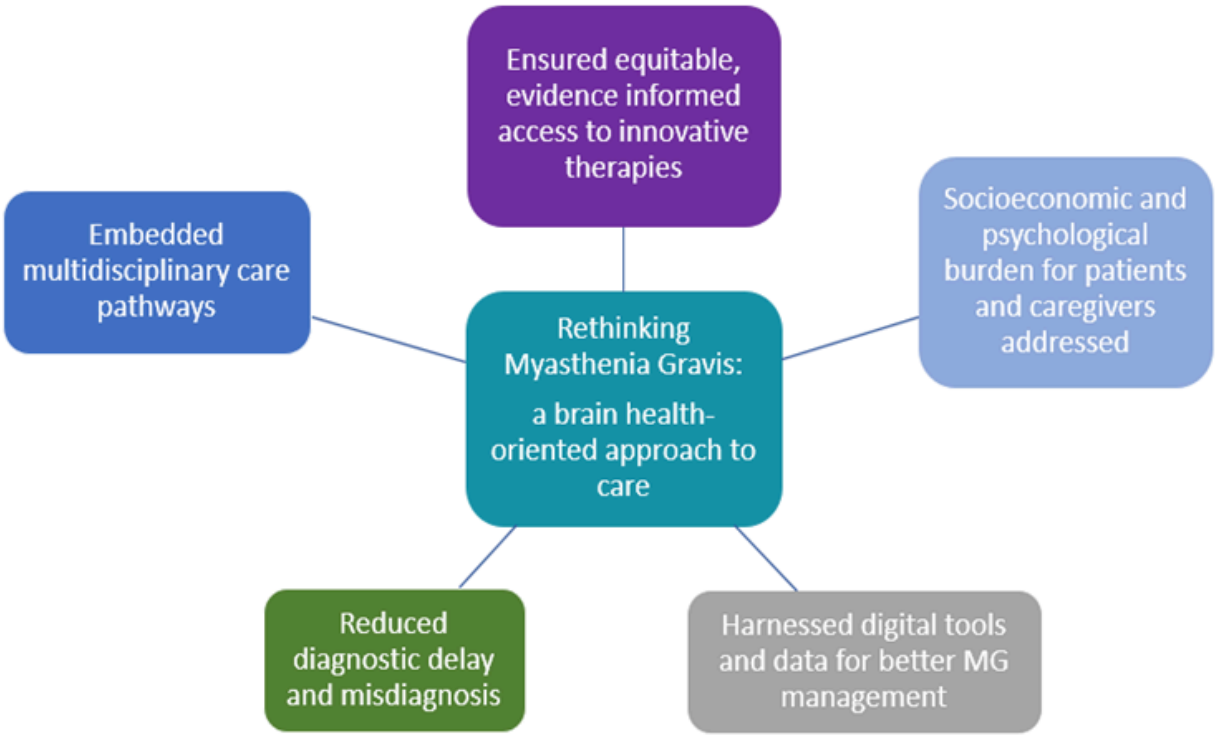


Figure 1 presents a brain health–oriented conceptual framework for MG care. It places brain health at the centre of the care pathway and connects it to five interconnected domains identified through published literature and stakeholder interviews: (1) access and diagnosis, (2) multidisciplinary care pathways, (3) innovative treatments, (4) socioeconomic and psychosocial burden, and (5) digital tools and data.

In this framework, brain health in MG extends beyond muscle strength to include mental health, cognition, fatigue, daily functioning and social participation. A brain health–oriented approach therefore seeks to preserve and improve these dimensions across the entire MG care pathway.

Recommendations aligned with these domains are advocacy-driven and summarised in **Box 1** (next page.) They aim to help policymakers, healthcare providers, payers, and patient organisations reduce diagnostic delay; embed multidisciplinary, person-centred care; enable equitable, evidence-informed access to innovative therapies; address socioeconomic and psychosocial burdens; and accelerate meaningful, co-designed digital transformation.

Collectively, these actions can support the operationalisation of global and European commitments on rare diseases and neurological disorders in the specific context of MG, while positioning MG as a test case for brain health–oriented care in rare diseases across Europe. With the European Health Data Space becoming operational, joint EU Health Technology Assessment implementation underway, and rare disease priorities reaffirmed at the World Health Assembly in 2025, Myasthenia Gravis represents a timely and feasible condition to pilot brain health-oriented care pathways in Europe.

Box 1. Key Recommendations

1. Reduce diagnostic delay and misdiagnosis

- Increase awareness and training in MG among general practitioners, emergency physicians and nurses, non-neurology specialists, and general neurologists, while strengthening referral pathways to specialist neuromuscular/MG centres.
- Ensure timely access to core diagnostics (neurophysiology, antibody testing, including broader access to validated cell-based assays (CBAs) when appropriate) and tele-expertise with MG centres.
- Promote research on disease mechanisms and long-term neuromuscular protection to inform prevention of instability and exacerbations.

2. Embed multidisciplinary, brain health-oriented care pathways

- Institutionalise multidisciplinary MG clinics and networks within rare disease frameworks (ERNs and national centres of expertise).
- Systematically integrate mental health screening, management of fatigue and cognitive impairment, access to appropriate rehabilitation, and psychosocial support into MG care pathways, alongside motor symptom control.

3. Ensure equitable, evidence-informed access to innovative therapies

- Harmonise the standard of care (including diagnosis, referral pathways, and treatment sequencing), as well as national access and reimbursement criteria across EU countries, aligned with the most recent European and international MG guidelines.
- Use real-world evidence and patient-reported outcomes (PROs), including digital PROs, in Health Technology Assessment (HTA) and pricing decisions for MG treatments.

4. Address socioeconomic and psychosocial burden for patients and caregivers

- Promote flexible work arrangements, workplace adaptations, and social protection mechanisms for people living with MG.
- Develop structured caregiver support, including respite, counselling, and information on rights and entitlements.

5. Harness digital tools and data for better MG management

- Scale up MG-relevant telemedicine, remote monitoring, and wearable-based tracking in routine care.
- Improve interoperability between digital platforms, MG registries, ERN tools and the European Health Data Space to support real-world evidence and patient-experienced data.
- Co-design digital tools with patients and caregivers to ensure accessibility and usability.

Key take aways grounded in the stakeholder insights: What patients and caregivers say matters most

01 Shorten time to diagnosis and reduce misdiagnosis by strengthening awareness among general practitioners and first-contact clinicians, enabling rapid referral or direct consultation with MG expert centres, and providing concise and practical care pathway guidance.

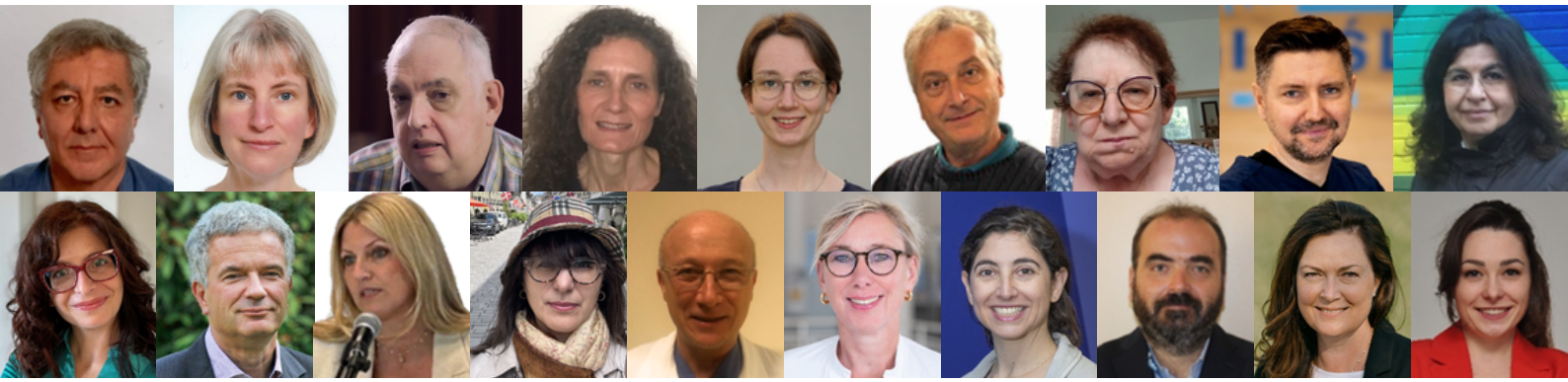
02 Implement integrated multidisciplinary care models, including a clearly defined nurse care coordination function, linking specialist centres with local healthcare providers, rehabilitation services, women's health services where relevant, and social support—particularly during life transitions.

03 Embed mental health screening and ensure timely access to psychological and psychiatric support as routine components of care, reflecting the substantial burden of anxiety, depression, and trauma-related symptoms reported by patients and clinicians.

04 Measure and act on outcomes that matter to patients—including fatigue, functional capacity, participation, and quality of life—by integrating patient-reported outcome measures and structured monitoring, supported by digital tools that complement clinical care.

05 Enable equitable access to therapeutic innovation by addressing reimbursement and administrative barriers, strengthening delivery capacity (including infusion and subcutaneous treatment pathways and adequate nursing resources), and ensuring fair access for underrepresented subgroups such as seronegative patients through targeted evidence generation and clinical research where needed.

Voices from patients, caregivers, and clinicians illustrating these points are compiled in the annex.



Foreword

Myasthenia gravis (MG) is a rare autoimmune neuromuscular disease marked by fluctuating muscle weakness. Although MG does not primarily affect the brain, its chronic, unpredictable, and disabling course has profound consequences for mental health and psychosocial wellbeing—comparable to those observed in other chronic neurological disorders, including those that directly involve the brain. MG influences far more than muscle function: it shapes how people view their future, participate in work, sustain relationships, and define their role in society. Daily life for families often requires continual adjustment to fluctuating symptoms, while employers and services frequently struggle to recognise or accommodate the condition's invisible limitations. The past decade has been described as a period of `transformative developments´ in MG care, with treatment evolving from broad immunosuppression to more precise, immune-targeted therapies that are reshaping long-term management. This therapeutic progress has been accompanied by advances in biomarker research, earlier diagnosis, and increasing attention to structured long-term monitoring.

This advocacy paper synthesises cutting-edge evidence with the lived experience of patients, caregivers, and healthcare professionals across several European countries. Its message is clear: rethinking MG care requires faster and more accurate diagnosis, robust multidisciplinary care aligned with a brain health approach, equitable access to innovative therapies, and meaningful integration of digital tools and patient-experienced data. MG can serve as a test case for how Europe organises care for rare diseases—demonstrating how coordinated, patient-centred systems can be built. Strengthening MG care pathways and policies will not only benefit those living with MG but also help advance more inclusive approaches for other rare neurological and neuromuscular disorders. Together, we can turn high-level commitments into tangible improvements for people living with MG and those who care for them every day, by acting on the recommendations set out in this paper.

Prof. Suzanne Dickson
President, European Brain Council

Endorsement

01 Introduction

"Myasthenia gravis is an invisible disease. From the outside, people see you walking and talking, but they don't see how quickly your strength fades or how hard it can be to breathe, speak, or even hold a glass of water."
— Shared during stakeholder interviews informing this paper, this reflection captures fluctuating symptoms, social misunderstanding, and constant adaptation that shapes the daily lives with those with Myasthenia gravis.

Myasthenia gravis is a rare, chronic autoimmune neuromuscular disorder characterised by severe and fatigable muscle weakness caused by antibodies that target neuromuscular junction proteins such as the acetylcholine receptor (AChR), muscle-specific kinase (MuSK), or low-density lipoprotein receptor–related protein 4 (LRP4) (Gilhus et al., 2019; Dresser et al., 2021). The condition frequently presents initially with ocular symptoms (ptosis and diplopia) and, in many patients, progresses to generalised MG with bulbar, limb or respiratory involvement (Hehir & Silvestri, 2018; Gilhus et al., 2019; Dresser et al., 2021; Evoli & Iorio, 2020). MG is, however, highly heterogeneous. Approximately 10-15% of patients have seronegative MG (SnMG), in which no AChR, MuSK, or LRP4 antibodies are detectable using standard tests. These cases are more diagnostically challenging and at higher risk of misdiagnosis. SnMG can carry a substantial disease burden and because these patients are often under-represented in clinical trials, evidence supporting access to targeted innovative therapies is more limited, often contributing to longer diagnostic delays (Vinciguerra et al., 2023; Evoli et al., 2024; Lehnerer et al., 2025). Across Europe, published estimates indicate that approximately 56,000–123,000 people live with MG (depending on country and data source) with incidence peaking in younger women and in older adults, and later onset more common in men (Bubuioc et al., 2021; Sciancalepore et al.,2024; Gilhus & Breiner, 2025). Importantly, approximately 10% of people living with MG are children or adolescents (Juvenile MG or JMG), in whom disease presentation, immunological profile, and therapeutic considerations present with nuances that require age-adapted diagnostic and management strategies (Munot et al., 2020; O'Connell, Ramdas, & Palace, 2020). Juvenile MG often poses additional challenges related to growth, long-term immunosuppression, and transition to adult care, underscoring the need for dedicated care pathways across the lifespan (Munot et al., 2020; O'Connell, Ramdas, & Palace, 2020).

Although MG is biologically well characterised, it remains clinically and health-system challenging due to its heterogeneous presentation, fluctuating disease course, and persistent variation in access to specialised centres and innovative therapies across European countries. (Narayanaswami et al., 2021; Cortés-Vicente et al., 2024). Beyond neuromuscular symptoms, MG can substantially affect brain health and functional participation, including fatigue, cognitive strain, anxiety, depression, social withdrawal, and loss of autonomy – factors not consistently addressed in care models that focus primarily on symptom control (McCallion et al., 2024; Saccà et al., 2024). The economic burden is also substantial and often shifted to patients, caregivers, and the wider society through productivity losses, informal care, and out-of-pocket costs, many of which remain under-captured in traditional health technology assessment (HTA) frameworks (Avalere, 2023; Dewilde et al., 2025; Gwathmey et al., 2025). While efforts to strengthen MG care pathways are progressing, emerging digital tools— such as app- and wearable-based monitoring combined with electronic patient-reported outcomes— offer opportunities to capture symptom variability between visits and support more timely, data-informed management (Stein et al., 2025).

This advocacy paper provides an evidence-informed overview of the current MG landscape in Europe and identifies system-level gaps that hinder optimal, patient-centred care. Using a brain health lens and grounded in lived experience, it proposes actionable recommendations to strengthen adaptive care pathways, enhance the use of real-world and patient-reported data, and integrate MG more coherently into European rare disease and brain health policy frameworks. The analysis draws on a structured review of relevant literature (peer-reviewed and grey literature) and qualitative insights from 19 semi-structured interviews with patient representatives (including leaders of patient organisations), caregivers, and healthcare professionals from four countries: Italy (7 interviews: 2 patients, 1 caregiver, 4 healthcare professionals), France (4 interviews: 2 patients, 2 healthcare professionals), Germany (5 interviews: 2 patients, 1 caregiver, 2 healthcare professionals), and Poland (3 interviews: 1 patient, 2 healthcare professionals). The interviews provide contextual detail and illustrative perspectives that inform the interpretation of the literature and ensure that the recommendations reflect real-world experience across diverse healthcare settings. This approach aligns with the paper's advocacy focus on care pathway organisations, access, and value assessment.

02 Current landscape and need to rethink myasthenia gravis through a brain health lens

Over the past decade, MG care has entered a phase of significant therapeutic and diagnostic progress (**Box 2)**. Advances in antibody testing, particularly the increasing use of cell-based assays (laboratory techniques that use living or fixed cells to measure functional responses), have improved diagnostic accuracy and enabled better disease subtyping, including among patients previously labelled seronegative (Cruz et al., 2015; Hoffmann et al., 2023). In parallel, targeted therapies, most notably neonatal Fc receptor (FcRn) antagonists and complement inhibitors, have demonstrated clinically meaningful improvements in functional outcomes and quality of life in generalised MG populations, with potential to reduce reliance on long-term corticosteroids in antibody-positive generalised MG (Howard et al., 2017; Howard et al., 2021; Mantegazza et al., 2023). These developments align with the European Medicines Agency's priorities on precision medicine and adaptive regulatory pathways for rare diseases (European Medicines Agency, 2024).

However, the real-world impact of therapeutic innovation remains constrained by fragmented care pathways, diagnostic delays and misdiagnosis, limited specialist capacity, and uneven access driven by reimbursement and HTA decisions. Clinical guidance also lags behind innovation (EU guidance from 2010; international consensus updates from 2020/2021, predating FcRn inhibitors and newer complement agents), and national guideline updates remain uneven, with continued variation in practice across countries (Skeie et al., 2010; Narayanaswami et al., 2021; Wiendl et al., 2023; De Bleecker et al., 2024. Multidisciplinary care, psychological support, and structured transition pathways remain inconsistently available. Moreover, digital tools and validated outcome measures—including patient-reported outcome measures (PROMs) such as Myasthenia Gravis Activities of Daily Living (MG-ADL) and Myasthenia Gravis Quality of Life 15-item revised MG-QoL15r, as well as clinician-rated scales such as Quantitative Myasthenia Gravis (score) (QMG)—are not routinely embedded in care pathways, despite their recognised value, as reflected in recent expert consensus calling for more standardised assessment in day-to-day practice (Meisel et al., 2024). These gaps contribute to a persistent disconnect between scientific progress and health system organisation.

Insights from a multitude of stakeholders collected for this paper reinforce that MG management is not only a pharmacological challenge, but also a health system (care pathway and system integration) challenge. Administrative barriers to accessing innovative therapies, limited recognition of caregiver burden, and time constraints in specialist care can impede outcomes. Underserved groups, including people with seronegative disease, paediatric patients, and those transitioning across life stages, may experience disproportionate barriers, reinforcing inequities despite high unmet needs (De Bleecker et al., 2024; Narayanaswami et al., 2021).

Viewed through a brain health lens, these system gaps matter because they affect not only neuromuscular symptom control but also fatigue, mental health, cognition and participation in daily life. Beyond fluctuating muscle weakness, fatigue represents a major and often under-recognised burden in conditions such as myasthenia gravis. A 2025 Rare Barometer survey found that 80% of people living with a rare disease report experiencing fatigue and/or pain, significantly affecting daily functioning and social participation (Faye et al., 2025).

In a rapidly evolving EU policy context—including the EHDS, ERNs and the EU HTA Regulation—MG represents a model condition for demonstrating how adaptive pathways, registries, real-world evidence and PROMs can support earlier access, more equitable value assessment and sustainable uptake of innovation, while capturing outcomes that matter to patients (EURORDIS-Rare Diseases Europe, 2024; ERN Monitoring Report, 2025; HTA Regulation (EU) 2021/2282; EHDS Regulation (EU) 2025/327; World Health Assembly, 2025).

Box 2. Clinical and system-level advances in myasthenia gravis care

- Improved diagnostics: Cell-based assays and related approaches can improve detection of clinically relevant antibodies (including in some "seronegative" cases), supporting more accurate diagnosis and stratification (Cruz et al., 2015; Hoffmann et al., 2023).
- Standardised outcome measures: Patient- and clinician-reported tools such as MG-ADL and QMG are increasingly used in trials and practice, enabling functional assessment and supporting regulatory and HTA evaluation (Meisel et al., 2024). In addition, newer instruments—such as the Myasthenia Gravis Impairment Index (MGII) and MG Symptoms PRO—are expanding the toolbox for patient-centred assessment, helping capture aspects that are often under-represented in routine metrics, including quality of life impacts and invisible burdens (e.g., fatigue, psychosocial effects, and daily-life costs) that this paper seeks to foreground (Meisel et al., 2024).
- Targeted therapies
- FcRn antagonists reduce circulating immunoglobulin G (IgG) levels and have demonstrated rapid clinical improvement in generalised MG in clinical trials; emerging longer-term and real-world evidence also suggests steroid-sparing potential for some agents (Howard et al., 2021; Bril et al., 2023; Antozzi et al., 2024; Fuchs et al., 2024; Antozzi et al., 2025).
- Complement inhibitors reduce complement-mediated damage and have shown rapid and sustained benefit in AChR-positive generalised MG (Howard et al., 2017; Muppidi et al., 2019; Vu et al., 2022; Howard et al., 2023).
- Stratified treatment approaches: Clinical practice is increasingly stratified by antibody status, disease activity/severity, and patient characteristics; however, important evidence gaps persist—particularly for seronegative MG and paediatric/transition populations, where practice often relies on extrapolation and expert consensus. Real-world evidence is expanding but remains limited, underscoring the need for more inclusive data across subgroups (Narayanaswami et al., 2021; Wiendl et al., 2023; O'Connell et al., 2020; Vinciguerra et al., 2023; Fuchs et al., 2024).
- Evolving treatment goals: Treatment goals are shifting towards minimal symptom expression, reduced long-term steroid burden, improved quality of life and participation – outcomes closely linked to brain health, preservation of brain health (Narayanaswami et al., 2021; Saccà et al., 2024).

The next sub-sections examine three areas in depth: (1) socioeconomic and psychosocial impact of MG on patients, caregivers, and society; (2) organisation and performance of care pathways; and (3) underused role of digital tools in monitoring, care coordination, and real-world evidence generation. Table 1 provides an illustrative snapshot of key MG care features in Germany, France, Italy, and Poland, synthesising findings from the literature and stakeholder interviews; it is not intended as a fully harmonised cross-country comparison, as indicators and reporting practices vary between settings.

Table 1. Overview of MG care pathway features (Germany, France, Italy, Poland) (*)

Domain	Germany	France	Italy	Poland
Typical diagnostic pathway bottlenecks	Variability in referral speed; access depends on region and centre density	Variation across regions; specialist centres often centralised	Regional variation; pathways differ by region/centre	Limited specialist capacity and longer referral chains frequently reported
Advanced antibody testing /Cell-based assays (CBAs)	Often available through specialised labs/centres	Often available through specialised labs/centres	Availability varies regionally	Less consistently available; capacity and access constraints often reported
Multidisciplinary MG care	Available mainly in tertiary centres; uneven regional coverage	Specialist reference-centre model; regional access varies	Regionalised system creates heterogeneity	Concentrated expertise; rural access challenges
Access to innovation (HTA / reimbursement realities)	Broad access possible but processes vary by indication and rules	Access frameworks exist; regional implementation varies	Variation by region/centre and criteria	More restrictive access and capacity bottlenecks often reported
Digital health infrastructure relevant to MG	Strong national digital health policy environment (e.g., DiGA ecosystem) (Goeldner et al., 2024)	Telemedicine scaled post-COVID; national guidance and reimbursement evolution (Garibaldi et al., 2021)	Project-based adoption; variable integration	Uneven adoption; workflow and resource constraints frequently reported

2.1 Socioeconomic and psychological impact of myasthenia gravis

MG imposes substantial direct and indirect costs on patients, caregivers, and society. Economic analyses show that the burden varies substantially by disease severity and care setting, with higher costs linked to instability, crises, intensive treatment pathways, and productivity loss (Landfeldt et al., 2020; Lehnerer et al., 2025; McCallion et al., 2024; Zhou et al., 2025). Myasthenic crisis and exacerbations are major drivers of cost and patient outcomes; associated with emergency care, hospitalisation (inpatient care), and downstream morbidity and mortality risk (Claytor et al., 2023; Mück et al., 2024; Zhou et al., 2025).

Indirect and intangible costs add considerably to the overall burden. Many people with MG report reduced work capacity and unstable employment trajectories; caregiver time and career impacts compound these losses, particularly where care remains fragmented and crisis-driven (Dewilde et al., 2025; McCallion et al., 2024). The socioeconomic impact of JMG on patients and families has not been studied, but is likely to be similar to that in adults. The impact of JMG on education may affect their career achievements and have long-term secondary socioeconomic impact. Across studies, fatigue and mental health symptoms are consistently reported and closely linked to reduced participation and quality of life—reinforcing the relevance of a brain health lens in MG and a disability-rights framing that prioritises inclusion and participation (McCallion et al., 2024; Saccà et al., 2024; European Commission, 2021; United Nations General Assembly, 2021).

Insights from stakeholder interviews

Stakeholder testimonies across Italy, France, Germany, and Poland emphasise three recurring socioeconomic and psychosocial priorities that should be reflected in pathway design and policy:

- Employment flexibility and `fit-for-work´ guidance: tailored work arrangements, support to negotiate reasonable adjustments, and proactive discussion of realistic roles across the disease course.
- Lifestyle adaptations and financial support: costs linked to home modifications, mobility and transport, and clearer guidance on entitlements and practical support mechanisms.
- Loneliness and psychological support: social withdrawal, difficulty finding peers with shared experience, and inconsistent access to psychological care despite its importance for daily functioning and well-being.

Voices from patients, caregivers. and clinicians illustrating these points are compiled in the Annex.

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2.2. Optimising patient care pathways

Current MG care pathways across Europe remain marked by fragmentation, variability in practice, and uneven access to specialised services. Diagnostic delay remains a major central challenge across settings: symptom overlap with other neurological, ophthalmological, and psychiatric conditions frequently leads to misdiagnosis and prolonged diagnostic journeys, particularly in young women (Cortés-Vicente et al., 2024; Sobierajski et al., 2023). These delays are not only a matter of time to diagnosis; they can translate into avoidable crises and exacerbations, unnecessary corticosteroid exposure (and its long-term sequelae), increased disability, and poorer patient-reported outcomes and quality of life (including lower MG-QoL15r scores). This reinforces the need for earlier and more accurate diagnosis supported by clear, structured referral routes (Claytor et al., 2023; Narayanaswami et al., 2021).

Access to advanced diagnostics is a key determinant of timely and accurate diagnosis. Cell-based assays and related approaches can improve antibody detection, particularly for individuals previously labelled `seronegative´, and support more precise subtyping of AChR-, MuSK- and LRP4-associated disease (Cruz et al., 2015; Hoffmann et al., 2023; Vincent et al., 2012). Yet access remains inconsistent across Europe, due to variations in laboratory capacity, reimbursement policies, and centre-level expertise. This creates an equity gap: the likelihood of receiving timely diagnosis and appropriate stratification often depends more on geography and successful referral rather than on clinical need.

After diagnosis, the lack of standardised multidisciplinary pathways is a recurrent gap. International guidelines highlight the importance of coordinated, specialist teams encompassing neurology, specialist nursing, respiratory care, rehabilitation, and psychosocial support (Narayanaswami et al., 2021). In practice, integrated neuromuscular clinics are concentrated in tertiary centres, leaving many regional areas underserved for follow-up. Workforce constraints and uneven neurologist density further limit consistent implementation.

Access to innovative therapies is similarly shaped by national reimbursement and HTA decisions and, critically, whether health systems have the practical capacity and monitoring infrastructure needed for safe initiation or continuation of therapy.

Insights from stakeholder interviews

Interview findings consistently emphasise three care pathway priorities:

- Reduce diagnostic delay: strengthen primary care and general neurology training, and speed up access to key investigations (including neurophysiology), especially when fatigable weakness is a presenting symptom.
- Make multidisciplinary care the default: address coordination gaps between inpatient/outpatient services and across specialties (e.g., neurology, ophthalmology, respiratory care, rehabilitation, psychosocial support) through structured collaboration in specialist MG centres.
- Make innovation usable in real life: `access´ extends beyond reimbursement, therefore ensuring practical pathways for administration, monitoring, education, and follow-up, supported by trained teams and adequate service capacity.

Voices from patients, caregivers. and clinicians illustrating these points are compiled in the Annex.

2.3 Digital tools in myasthenia gravis management

Digital innovation has significant potential to strengthen MG care, yet implementation remains uneven and is often project-based, limited to local pilots, or confined to research initiatives. During the COVID-19 pandemic, telemedicine demonstrated its feasibility for neuromuscular disorders and helped maintain continuity of care for patients facing mobility or access constraints (El-Hassar et al., 2023; Garibaldi et al., 2021; Pareyson et al., 2021). For example, in Germany, the Digital Healthcare Act and the DiGA framework enabled prescription and reimbursement of certified digital health applications, creating one of the first large-scale policy environments supporting routine digital health use in clinical care (Goeldner et al., 2024).

MG specific digital approaches are now emerging. Tools such as MyaLink in Germany illustrate an expanding landscape of digital platforms designed for remote disease monitoring and are increasingly described in the literature (Stein et al., 2025). Several groups have proposed MG-tailored telemedicine frameworks, including structured remote assessment protocols using simple functional tests (Ricciardi et al., 2021). Decentralised real-world studies using smartphones show feasibility for collecting patient-reported and functional data outside clinic settings (Steyaert et al., 2023; Pasqualin et al., 2022). Recent work points toward combining mobile application–based patient-reported outcome measures (PROMs) with wearable data streams for continuous symptom tracking and potentially earlier detection of deterioration between visits (Stein et al., 2025). For instance, PROMMY, developed within the European Reference Network for Rare Neuromuscular Diseases (ERN-NMD), is a mobile application designed to collect and track PROMs and support sharing with clinicians (Mancuso, 2025).

Digital tools also have system-level roles in coordination and evidence generation. Integrated electronic health records (EHRs) and secure teleconsultation platforms can strengthen communication across primary, secondary and specialist care. At the EU-level, initiatives such as the EHDS create opportunities for secondary use of health data, including patient-reported outcomes (PROs) and digital measures, to support research, registries, and HTA (Regulation (EU) 2025/327). In parallel, recent expert consensus initiatives emphasise the need for greater harmonisation of MG registries and core data elements to ensure comparability of outcomes across countries and studies (Slioui et al., 2025). However, several barriers limit adoption. Patients and clinicians report challenges related to limited integration into existing workflows, variable reimbursement models, accessibility constraints (including visual symptoms such as diplopia), and concerns about digital literacy and device access. Many stakeholders also caution that without deliberate equity-oriented design, digital tools risk widening rather than reducing inequalities in MG care.

Insights from stakeholder interviews

Interview data highlights four practical use-cases and one design principle:
·Use-case 1 (access): tools that help schedule appointments and reduce administrative burden.

- Use-case 2 (monitoring): simple symptom tracking and PRO capture, linked to real clinical feedback.
- Use-case 3 (information and connection): trusted information sources and peer-connection channels, including for under-discussed topics (e.g., sexuality, contraception, life transitions).
- Use-case 4 (education): co-designed educational tools that support self-management and prepare patients for consultations.
- Design principle: rethink digital tools, so they facilitate care rather than constrain it—telemedicine can reduce travel burden, but it does not replace in-person care when physical examination, infusion administration, or complex decision-making is required (Goeldner et al., 2024; Ricciardi et al., 2021).

Voices from patients, caregivers, and clinicians illustrating these points are compiled in the Annex.

03 Conclusions and policy implications

Findings from this paper point to a clear policy message: MG must be addressed not only through therapeutic innovation, but also through integrated, brain health-oriented systems that protect functioning, participation, and quality of life. In this context, MG offers a concrete case to operationalise global neurological policy recommendations and priorities, particularly the WHO Intersectoral Global Action Plan on Epilepsy and Other Neurological Disorders (WHOiGAP). Like many other neurological disorders, MG is an important cause of morbidity and disability that, as stated in the WHOiGAP, requires concerted intersectoral efforts to address the needs of people with MG by providing them with equitable access to effective health care and social, educational, and vocational interventions and services (World Health Organization, 2023).

This advocacy paper demonstrates that the gap between scientific advances and real-world care delivery remains substantial in MG. Diagnostic delays, fragmented pathways, and inequitable access to innovation persist, while socioeconomic strain and under-addressed psychosocial impacts continue to shape outcomes and daily functioning (Cortés-Vicente et al., 2024; Dewilde et al., 2025; McCallion et al., 2024; Saccà et al., 2024). Closing this gap requires coordinated action across the entire pathway: earlier and more accurate diagnosis supported by validated diagnostics and clear referral systems; institutionalised, multidisciplinary, brain health-oriented care within rare disease networks; HTA and reimbursement frameworks that incorporate meaningful outcomes and real-world evidence; and digital tools that are accessible, interoperable and usable in diverse clinical settings (Meisel et al., 2024; Regulation (EU) 2021/2282; Regulation (EU) 2025/327).

Viewed through this lens, MG is not only a neuromuscular junction disorder, but also a test case for how Europe can organise adaptive, patient-centred, brain health-oriented care for rare diseases more broadly (Table 2). As the EU advances new data, pharmaceutical and rare disease strategies, delivering on the recommendations outlined in this paper will require coordinated action from policymakers, payers, regulators, clinicians, researchers, employers—and critically —people living with MG and their respective patient organisations. Such collective action is essential to guarantee effective and timely diagnosis, treatment, and holistic care, ultimately reducing disease burden and improving the well-being and quality of life of individuals affected by MG.

Table 2. Myasthenia gravis and policy implications from a brain health perspective

MG Dimension	Brain Health Impact	Relevant EHDS Data Categories	HTA Domains Impacted	Policy Relevance / Use Case
Fluctuating muscle weakness and fatigability	Cognitive strain, reduced attention, mental exhaustion; stress related to unpredictability	• EHRs (clinical assessments, MG-ADL) • PROMs (fatigue, daily functioning)	• Clinical effectiveness • Patient relevance	Demonstrates that functional stability and fatigue reduction are core outcomes
Delayed diagnosis and referral	Anxiety, loss of trust, psycho-logical distress; prolonged uncertainty	• EHRs (time to diagnosis) • PREMs (diagnostic experience)	• Societal impact • Ethical / equity aspects	Supports earlier diagnosis pathways and GP - specialist connectivity
Chronic disease course	Long-term mental health burden; adaptation fatigue	• Longitudinal registries • PROMs (mental health, QoL)	• Clinical effectiveness • Patient relevance	Highlights need for long-term outcome data beyond short trials
Myasthenic crises / ICU admissions	Trauma, PTSD, fear of recurrence; cognitive recovery issues	• Hospital episode data • EHRs (acute events) • PROMs (post-crisis wellbeing)	• Safety • Patient relevance	Justifies inclusion of crisis prevention and psychological sequelae in value assessment
Use of targeted biologics	Reduced treatment burden; improved psychological stability when effective	• EHRs (treatment sequences) • RWE datasets • Registries	• Clinical effectiveness • Safety • Organisational aspects	Supports adaptive pathways and RWE-informed reassessment

Table 2. Myasthenia gravis and policy implications from a brain health perspective (continued)

MG Dimension	Brain Health Impact	Relevant EHDS Data Categories	HTA Domains Impacted	Policy Relevance / Use Case
Seronegative or biomarker-negative MG	Frustration, exclusion, inequity-related distress	• Registries (phenotypes) • Off-label use data	• Ethical / equity aspects • Clinical effectiveness	Highlights unmet need and limitations of biomarker-gated access
Paediatric and adolescent MG	Neurodevelop-mental impact; educational disruption	• EHRs (age-specific data) • PROMs (school participation)	• Societal impact • Ethical aspects	Supports differentiated assessment for children and adolescents
Mental health comorbidities	Anxiety, depression, PTSD; reduced resilience	• PROMs (mental health scales) • Mental health records	• Patient relevance • Societal impact	Reinforces brain health as a core outcome domain
Care fragmentation / lack of coordination	Cognitive overload, stress, caregiver burnout	• PREMs (care coordination) • Organisational data	• Organisational aspects • Societal impact	Supports integrated care models and multidisciplinary pathways
Caregiver burden	Secondary brain health impact; emotional exhaustion	• PREMs (caregiver experience) • Socioeconomic data	• Societal impact • Ethical aspects	Justifies inclusion of caregiver outcomes in HTA
Digital monitoring and telemedicine	Reduced anxiety through continuity and early intervention	• Digital health data • Remote PROMs	• Organisational aspects • Clinical effectiveness	Demonstrates value of digital tools under EHDS

RETHINKING MYASTHENIA GRAVIS

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The EBC is a network of key players in the "brain space," with a membership encompassing scientific and professional societies, patient organisations, and industry partners. A non-profit organisation based in Brussels, its main mission is to promote brain research with the main goal of improving the lives of those living with brain conditions, neurological and mental alike.

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RETHINKING MYASTHENIA GRAVIS

ADVOCACY PAPER

Rethinking Myasthenia Gravis (MG) is a 2-year multistakeholder research-driven project offering policy recommendations to make tangible changes with the aim to improve the lives of people living with Myasthenia Gravis across Europe. The project officially kicked off during EBC's Rare Disease Day 2025 event, `Towards a Rare Brain Disease Ecosystem`.

Phase II: Rethinking Myasthenia Gravis Care through a Brain Health Lens: Adaptive Pathways and Patient-Centred Insights

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