Collaborating for the myasthenia gravis community

Identifying & addressing unmet needs for
people living with myasthenia gravis
in the United States

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Inspired by **patients.**
Driven by **science.**

A report commissioned by UCB in collaboration with experts from the following organizations:

Myasthenia Gravis Foundation of America

Myasthenia Gravis Association

Myasthenia Gravis Holistic Society

Conquer Myasthenia Gravis

Caregiver Action Network

Myasthenia Gravis Foundation of Michigan

Table of contents

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Preface

Pervasive long-term challenges exist for people living with myasthenia gravis (MG): to improve awareness and recognition, and to address health and care needs. The lack of awareness of MG has multiple impacts, including a struggle to achieve diagnosis (noted the "diagnostic journey"), challenges in being referred to appropriate clinicians, access to care, and a lack of support for those caring for people who have been diagnosed.

As a chronic autoimmune condition, where the body's immune system mistakenly attacks the connections between nerves and muscles, MG leaves those who suffer from it with unpredictable symptoms. This can include pain, disabling fatigue, muscle weakness, and difficulty breathing and swallowing.[1,2]

As these symptoms are nebulous and vary from one person to another, and from one day to the next, MG is often misdiagnosed, or no diagnosis is made at all.[1,2]

MG has a US prevalence of around 36,000 – 60,000 people,[3] and despite advances in treatment of some rare diseases, progress in the treatment of MG is only just starting to pick up pace.

Separately, published literature on the physiological manifestations of MG is expanding.[4] In October 2021, "The Lived Experience of Myasthenia Gravis: A Patient-Led Analysis", which explores the real-life experience of MG through the eyes of the patient, was published in Neurology and Therapy.[4]

The analysis is the result of detailed patient input and an exhaustive literature search, and offers an unprecedented level of first-hand information to help healthcare providers better understand the challenges people with MG face.

Five key themes emerged from “The Lived Experience of Myasthenia Gravis: A Patient-Led Analysis”, including:[4]

The unpredictability and fluctuating nature of symptoms

The disconnect between patients and physicians

The emotional toll that comes from the burden of disease and lack of support

A need for greater understanding of MG

A call for more open dialogue between patients and doctors

The development of “The Lived Experience of Myasthenia Gravis: A Patient-led Analysis” manuscript was led by two patient advocates in MG, Nancy Law from Colorado, US, and Kelly Davio from London, UK, in collaboration with UCB. The analysis was published posthumously following Nancy’s death in September 2021. We want to recognize Nancy’s invaluable contribution to this work, and her leadership in the MG patient advocacy community.[4]

What is needed now is a cohesive analysis of the priority challenges facing people affected by MG and how these can be addressed through scientific advances and other mechanisms.

A group of MG advocates, caregiver organizations, and patients have united to build on the findings from "The Lived Experience of Myasthenia Gravis: A Patient-led Analysis", explore current experiences and areas of greatest unmet need, and put forth actions that they believe will significantly improve lives and measurable health outcomes in MG.

About this Report

This report provides insights and analysis from several UCB-organized community listening exercises. Specifically, a robust social listening exercise covering six countries, a global roundtable held in late 2021, and a US dedicated MG Community Unmet Needs Advisory Board, held in December 2022.[5,8,9]

Three key challenges facing the MG community were uncovered from these discussions[5,8,9]

Delayed diagnosis

Lack of recognition

Barriers to accessing support networks & care

The following is a "call-to-action" to all relevant stakeholders to partner with MG advocacy communities – harnessing the renewed attention and momentum behind improving health and care for people living with a rare disease. Collaboration must be the cornerstone of any effort to improve life with MG – based on shared vision and purpose. The time is now to tackle the issues that burden those living with MG. We want the often-overlooked voices of people living with MG and other rare diseases to be heard by policymakers and in health system boardrooms, elevated among practicing clinicians, and recognized by the public. This report is intended for anyone who shares the ambition of its authors to bring about lasting change for people living with MG, and their families and care partners.

Social Media Dialogue: Living with MG

To delve deeper into the experiences of people living with MG, UCB used a robust assessment of unprompted comments made on social media platforms from across the United States, as well as France, Germany, Spain, Italy, and the UK, to gain a representative picture of the lived experience of MG. Four prevalent themes were identified in the US data:[5]

1: A failure to be recognized:

Frequent misdiagnoses and confusion related to the condition are reported. Feelings of exasperation and being misunderstood are also reported, in addition to physical symptoms being overlooked.[5]

Notable findings:

12

In the US, MG was reported to have been misdiagnosed as 12 different diseases, including stress, fibromyalgia, and diabetes.[5]

32%

32% of these misdiagnoses are mental health conditions.[5]

3%

Yet only 3% of online discussion is attributed to mental-health-related symptoms in relation to MG.[5]

2: Lack of adequate support:

Barriers to accessing care in a complex specialist landscape, with no clear support system to rely on, compounds negative experiences. A lack of clinician awareness and defined pathway through the healthcare system makes it difficult for those with MG and clinicians to interact consistently and productively.[5]

Notable findings:

11

MG patients in the United States reported contacting over 11 different types of specialists at some point in their care.[5]

25%

In the US, 25% of patients mentioned being referred to neurologists; 1 in 10 are referred to other specialists such as: Rheumatologists (1%), Pulmonologists (1%), Psychiatrists (1%), and Pediatricians (1%).[5]

3: High self-sufficiency:

Coping with MG requires self-education and adaption to everyday life, often without professional support. Those with the disease feel isolated and alone, struggling to cope with the unpredictable impacts of MG.[5]

Notable findings:

21

Patients in the United States describe using 21 different coping mechanisms to manage the physical and psychological burden of diseases.[5]

51%

In the US, informing other MG patients (51%) was the most frequently mentioned coping mechanism.[5]

Patients make practical adaptations every day to cope with symptoms such as when washing, dressing, and undertaking caring duties.[5]

4: A need to feel truly "seen":

People affected by MG are driven by hope for greater recognition from health care systems and the public, and personal acceptance of their disease. This must happen for them to feel adequately supported.[5]

Notable findings:

The number one driver of patient happiness is recognition of MG as a significant condition.[5]

> These findings were used as the basis for the initial discussion at the MG Community Unmet Needs Advisory Board, where participants aligned on three priority areas of unmet need.[9]

The Three Challenges in MG

Participants in the MG Community Unmet Needs Advisory Board identified the following three overarching challenges as the highest priority, based on the likelihood to most improve the lives of people affected by MG if addressed:[9]

Delayed diagnosis

Lack of recognition

Barriers to accessing support networks & care

In this section, we explore each of the three challenges, drawing on the latest literature and insights from the Advisory Board participants.

Delayed Diagnosis

Not all symptoms are treated equally: Susan Woolner – Myasthenia Gravis Foundation of Michigan

MG manifests differently for different people, causing a wide variety of symptoms. The myriad of symptoms can provide a challenge for their disease and its effects to be recognized and diagnosed, and to receive the correct degree of care. For example, Susan has learned from many with MG that they live with pain; their weakness and inability to move can cause muscular pain, but oftentimes doctors don't make that connection and dismiss the pain as a part of the MG.

It is well known that people living with rare diseases like MG often face a long and arduous journey to secure an initial diagnosis, as Susan, a Community Unmet Needs Advisory Board participant, explains above. This so-called "diagnostic odyssey" can generally be attributed to a lack of knowledge and awareness across multiple clinical centers, specialties, and practitioners[7] (e.g., primary care doctors need a basic level of knowledge, whereas specialists need the tools to diagnose MG quickly when individuals present with symptoms).

These delays place a substantial burden on the healthcare system; but more importantly, participants highlighted the real and emotional impact of delayed diagnosis on patients and their families.[9]

According to the report "Barriers to Rare Disease Diagnosis, Care and Treatment in the US: a 30-year Comparative Analysis" developed by the National Organization for Rare Disorders (NORD), a comparative study done in rare diseases (not specific to MG), despite scientific advancements and increased information sharing, particularly through online resources in recent years, the challenges to timely and accurate diagnosis persist across the rare disease space.[6]

36% of respondents had been diagnosed with a rare disease in 2019 within the first year, compared to 51% in 1989.[6]

Likewise, in 2019, 28% said diagnosis took seven or more years, while in 1989, 15% reported it took six years or longer. [6]

For Discussion:

With all the medical advancements in the past 30 years, how is this possible?

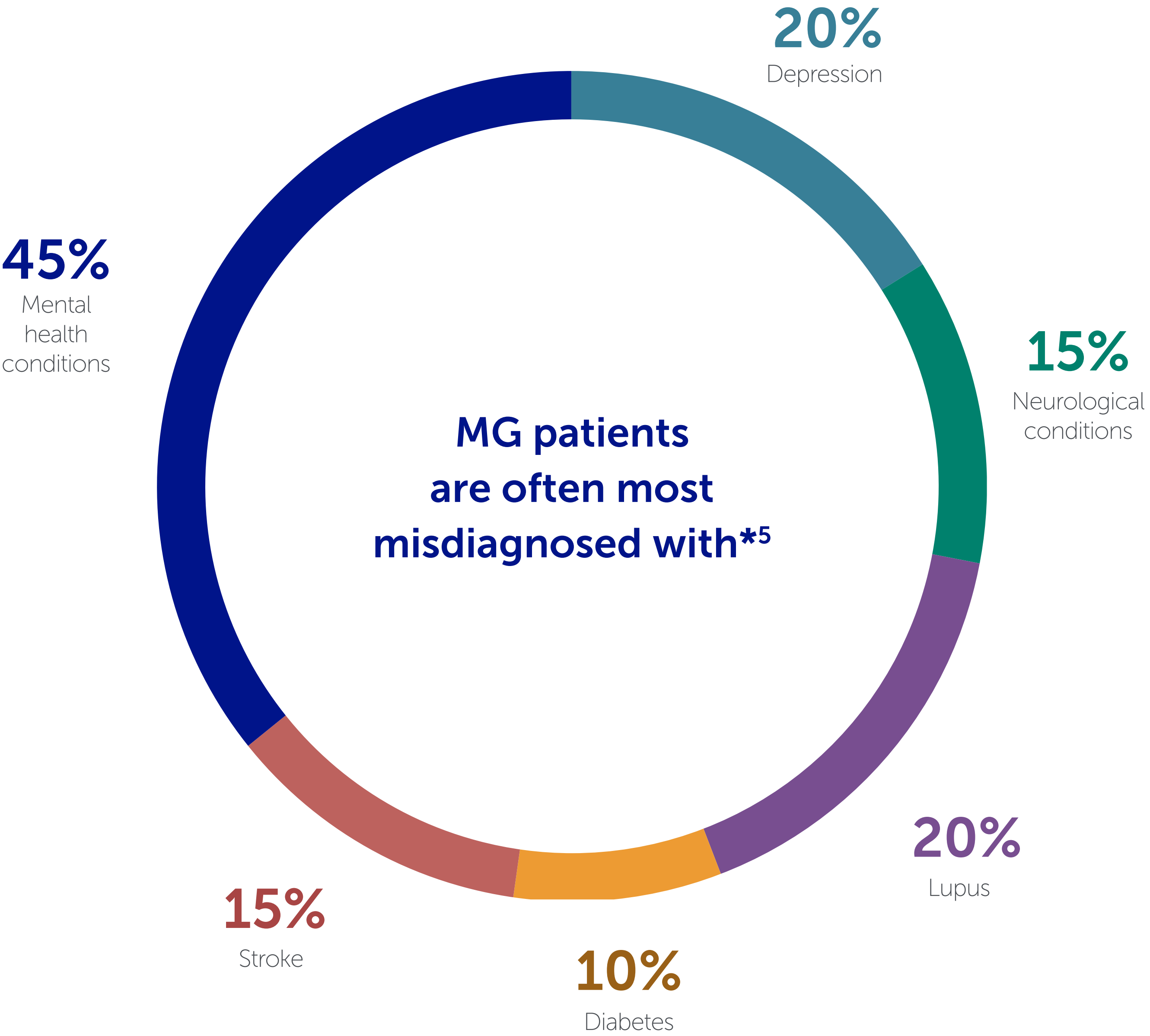
Like many other rare diseases, MG presents significant challenges for an accurate and timely diagnosis. Those with MG report experiencing nearly 35 different symptoms.[5] Symptoms are diverse, fluctuating, and can be non-specific, such as weakness and fatigue, with each individual experiencing unique combinations. Mental health challenges are commonly co-occurring symptoms, which can also lead to misdiagnoses.[5]

This can then send patients on frustrating and ineffective paths around specialists (the diagnostic odyssey), or even lead to them being sent home with a prescription for antidepressant or anxiety medication, rather than an MG diagnosis.[10]

Our social listening data reported MG patients being most often misdiagnosed with mental health conditions (45%), chief among them depression (20%).[5] Oftentimes, as the below chart illustrates, patients could receive multiple misdiagnoses outside of mental illness, including stroke, diabetes, lupus and other neurological conditions.

Patient experts reinforced the finding, stating that mental health symptoms are often recognized first by doctors, despite other MG symptoms being present.[9]

People in the United States living with MG reported 11 different medical specialists being involved in their care, with individual patients mentioning up to three specialists each.[5]



*Percentages in chart equal over 100% due to some patients receiving multiple diagnoses over time

During the MG Community Advisory Board Meeting,

Wendi Huff of the Myasthenia Gravis Foundation of America highlighted the reality that many practitioners misdiagnose MG because of the emotional burden caused by the disease: “We’re not addressing the anxiety, depression, and so many of the active emotional needs among those in the MG community, and, to do so, is to negate the reality of their experience.”

The advisory board participants identified three key contributors to delayed diagnosis:[9]

The endless diagnosis cycle among primary care providers

Lack of research among all symptoms of MG

Lack of education for healthcare (OR primary care) providers and general practitioners on MG

Wendi Huff noted that, "When patients present with primary care physicians, many times that is where the barrier starts, because they just get rotated around and they're never breaking out of that cycle to get to a neuromuscular specialist." The participants advised bringing more experts to the table to address these challenges.

As one participant said: "I'd really like to see pharma bringing together physicians and patients all at the same table. Let's bring them all together. Let's put them all in a room and see what happens."

Recommendations:

Training and raising awareness among primary care professionals, in particular to tackle symptom diversity

Investing in awareness and further education on MG for specialists, such as rheumatologists, ophthalmologists, and psychologists, who often have MG patients referred to them

Providing educational resources, such as videos, Q&As, or support groups for multiple audiences, including healthcare providers, patients, and caregivers

Lack of Recognition

Caregiver support: Joan Wincentsen – Conquer Myasthenia Gravis

I was blessed to have support from those in my community during my MG journey; however, I'll never forget being dropped off for an appointment and informed by someone in the office that this support would not last forever. For so many, this is their experience – that support erodes for those with chronic illnesses. Couples get divorced because caregivers grow frustrated with their partner; those who live alone oftentimes feel forgotten by friends and family; children feel left behind by classmates.

Clinical and real-world research, such as the recently published, "The Lived Experience of Myasthenia Gravis: A Patient-Led Analysis" in Neurology & Therapy, is generating a better understanding of physiological challenges, but there remains a lack of recognition of the first-hand lived experience of MG. Experts have called for phenomenology-led approaches to address this,[1] but a crucial gap remains that impedes further progress in recognizing the true needs of the MG population.

> People living with MG are acutely vulnerable to the detrimental impacts of low recognition of the disease burden. In the analysis, the priority driver of happiness is recognition of MG as a significant condition.[5]

For Discussion:

Thanks to the ubiquity of social media and the Internet, we are more connected than ever. How can we utilize online resources to provide support and a sense of community for those who feel they have none?

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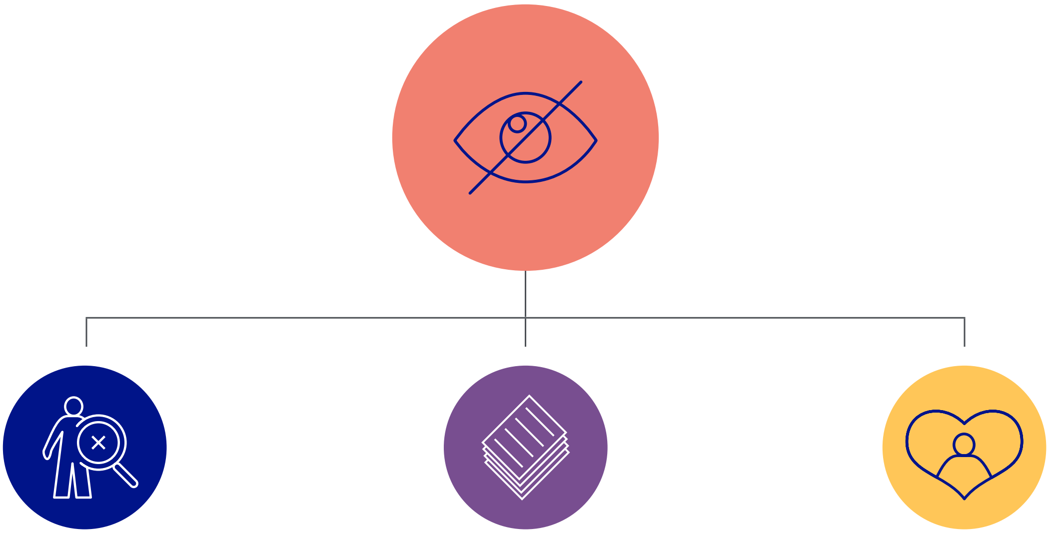
People living with MG are acutely vulnerable to the detrimental impacts of low recognition of the disease burden. In the analysis, the priority driver of happiness is recognition of MG as a significant condition.[5]

The advisory board participants identified three key contributors to lack of recognition of MG:[9]



Not all symptoms being recognized

Access to Family and Medical Leave Act (FMLA) resources

Self-care and the emotional burden of MG

As one participant put it, "I think we are currently in an interesting situation where there's a lot of research going on in myasthenia gravis. And while that's a good thing, the disease manifests differently amongst our population. And I think sometimes when a person brings up a symptom that isn't necessarily considered significant in a physician's mind or maybe not necessarily MG related, it gets dismissed."

For Susan Woolner of the Myasthenia Gravis Foundation of Michigan, the biggest challenge is research into the functional impact of MG. Someone might need to change a job or their role at work or have in intermittent FMLAs. "That patient can't always change these things (job, role, etc.). So, those are things that we need to be having conversations about."

Participants recognized that efforts to increase broad general awareness of MG would be challenging, given the sheer number of medical conditions – rare or otherwise – vying for attention at a public level. However, they did identify recommendations for the engaged MG community.

Recommendations:

Training and resources to manage employer conversations

Targeted materials to support women of childbearing age and other special populations

Materials for patients, caregivers, and healthcare providers on the full symptomatic burden of the illness

Resources for patients and caregivers to support self-care

Barriers to Accessing Support Networks & Care

Accessing care: Niki Grossheim – Myasthenia Gravis Holistic Society

Access to care can prove especially difficult to find in rural areas of the United States. For example, where I live, the closest neuromuscular specialist is at least two to three hours away. Between the driving time and our complicated weather, it can be very difficult for people to receive the care they need.

> Even if those in rural areas are physically able to make the trip to their nearest doctor, appointments are far and few between with so few doctors working in these areas.

For Discussion:

With technology advancing faster than ever, how do we ensure no one gets left behind?

The advisory board participants identified several specific barriers that make accessing support networks and care such a challenge:

Poor access to diagnostic support and care

Caregiver burnout

Emotional burden of illness

Technology barriers

Connecting men with the right support

Children's needs

Transportation

For example, participants shared that while it's best to see a neuromuscular specialist, these specialists can take up to a year for patients to see.[9]

> One participant advocated that it's better to see someone than no one – "I mean that's the diagnostic odyssey, so to speak; it's taking that long because of the current system we have in place." Turning to solutions, the advisors had several recommendations focused on larger systemic change in the United States.

Recommendations:

Improvements in healthcare in rural areas through telehealth and other provider-based resources

Improved access to neuromuscular specialists and reduced time to wait to see a neuromuscular specialist

Better support for caregivers

It's important to note that, while many resources for caregivers exist, Lisa Winstel of the Caregiver Action Network reports that these resources aren't getting to the people who most need them. "Our challenge is dissemination, is getting that information out there, and constantly making sure that the information that we have is relevant for each and informed by each community."

Conclusion

The genesis of this report highlights the need to improve the overall MG community experiences, so that it is on par with the advancements in scientific knowledge and rare disease policy. It is fundamental that we know what life with MG is really like to achieve faster diagnosis, targeted treatments, and better quality of life for those living with MG. Insights and recommendations included in this report are anchored by this need to better understand the MG community experience with an emphasis on what can be done – across stakeholders – to improve it. This is just the start; new evidence, continued collaboration, and industry endorsement are all required to turn recommendations into action.

Priority Focus Areas and Other Unidentified Needs:

To bring about real change and help deliver the care deserved by people living with MG, this report endorses the following priority actions – a synthesis of all recommendations, as agreed by advisory board representatives:

Multi-stakeholder roundtable – an opportunity to bring people from across the healthcare ecosystem together to discuss the challenges and identify workable solutions

Tool kits for specialized populations that meet individualized needs – caregivers, men, women of childbearing age, and children

Educational resources on the impact of MG holistically – including under-recognized symptoms

Improved distribution channels for materials – including websites, social media, and other targeted approaches

Training resources for general practitioners and others in the healthcare ecosystem to recognize and refer patients quickly

Improving access to neuromuscular specialists through improved telehealth reach in remote areas

Just the Beginning

Development of this report is also an important step forward, as insights from the advocacy community in the United States add to data previously collected in Europe and Japan to issue a rallying cry to those who make decisions about MG management and treatment. Given the unique experiences of each person living with MG, progress relies on forward-thinking collaboration with the community, and their voices remaining at the heart of MG advocacy efforts.

References

1. UN Addressing the challenges of persons living with rare disease and their families. Brazil, Central African Republic, Côte d'Ivoire, Cyprus, Ecuador, Equatorial Guinea, France, Italy, Peru, Portugal, Qatar, South Africa, Spain, Ukraine and Vanuatu: revised draft resolution Seventy-sixth session. Third Committee; Agenda item 28; Social development. https://undocs.org/A/C.3/76/L.20/Rev.1 (Last accessed: Jan 2023)

2. Conquer Myasthenia Gravis. What is MG? *https://www.myastheniagravis.org/about-mg/what-is-mg/* (Last accessed: Jan 2023)

3. Howard, James F. Jr., M.D. Clinical Overview of MG. Myasthenia Gravis Foundation of America. *https://myasthenia.org/Professionals/Clinical-Overview-of-MG*. (Last accessed: Jan 2023)

4. Law et al. The Lived Experience of Myasthenia Gravis: A Patient-Led Analysis. Neurol Ther. 2021;10(2):1103-1125. https://doi.org/10.1007/s40120-021-00285-w

5. UCB Data on file – Real Chemistry 2021 the MG Patient Experience.

6. NORD. Barriers to Rare Disease Diagnosis Care and Treatment in the US. Published 2020, Accessed November 18, 2022, https://rarediseases.org/wp-content/uploads/2020/11/NRD-2088-Barriers-30-Yr-Survey-Report_FNL-1.pdf

7. Ronicke et al. Can a decision support system accelerate rare disease diagnosis? Evaluating the potential impact of Ada DXin a retrospective study. Orphanet J RareDis. 2019;14(1):69. *https://doi.org/10.1186/s13023-019-1040-6*

8. UCB Data on file

9. UCB Data on file

10. Nicholson GA, Wilby J, Tennant C. Myasthenia gravis: the problem of a "psychiatric" misdiagnosis. Med J Aust. 1986 Jun 9;144(12):632-8. doi: 10.5694/j.1326-5377.1986.tb112345.x. PMID: 3713604.

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