Kamilla, living with MG

Collaborating **for the myasthenia gravis community**

Identifying & addressing unmet needs for people living with myasthenia gravis in Europe, through Rare 2030 and beyond

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

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The UN Resolution on "Addressing the challenges of persons living with a rare disease and their families" – unanimously adopted in November 2021 – is a clear marker of progress in how we must tackle rare diseases, globally. It brings together recent calls from the rare disease community for better recognition, equity and action across national boundaries. It also signals a new era of rare disease advocacy – moving from the catalyst provided by orphan drug designations toward a holistic approach to rare disease management.

Rare 2030 is the standard bearer for this new era – elevating recommendations for a new policy framework that is fit-for-purpose, in light of technological advances and the true lived experience of people affected by rare disease. This new mandate is a positive step, yet a pervasive long-term challenge exists: to improve awareness and recognition of specific rare diseases and to address health and care needs.

Such lack of awareness has multiple impacts: a struggle to achieve diagnosis (noted in this report as the "diagnostic odyssey"), challenges in being referred to appropriate clinicians, access to treatments (where they are available), and a lack of societal understanding, including in the workplace.

Myasthenia gravis (MG) is a rare disease where people affected face all of these challenges. As a chronic autoimmune condition, where the body's immune system mistakenly attacks the connections between nerves and muscles, MG leaves patients with unpredictable symptoms. This can include fatigue, muscle weakness, and difficulty breathing and swallowing. 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 global prevalence of around 700,000 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]

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.

> A pervasive long-term challenge exists: to improve awareness and recognition of specific rare diseases and to address health and care needs

That is where this report picks up the baton. We – as a group of passionate MG patient advocates – have united to explore current experiences, areas of greatest unmet need, and put forth actions that we believe will significantly improve lives and measurable health outcomes in MG by 2030.

This report summarizes dialogue that took place at a dedicated MG Patient Experience Expert Roundtable (held in October 2021) and the findings of a detailed social listening exercise organized by UCB.

From that foundation, we have highlighted three key challenges that need addressing:

1. **Delayed Diagnosis**
2. **Lack of Recognition**
3. **Barriers to Accessing Support Networks & Care**

Additionally, this report 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.

> 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.

We hope that, upon reading this report, you are inspired to join us and act.

Raquel Pardo

Johan Voerman

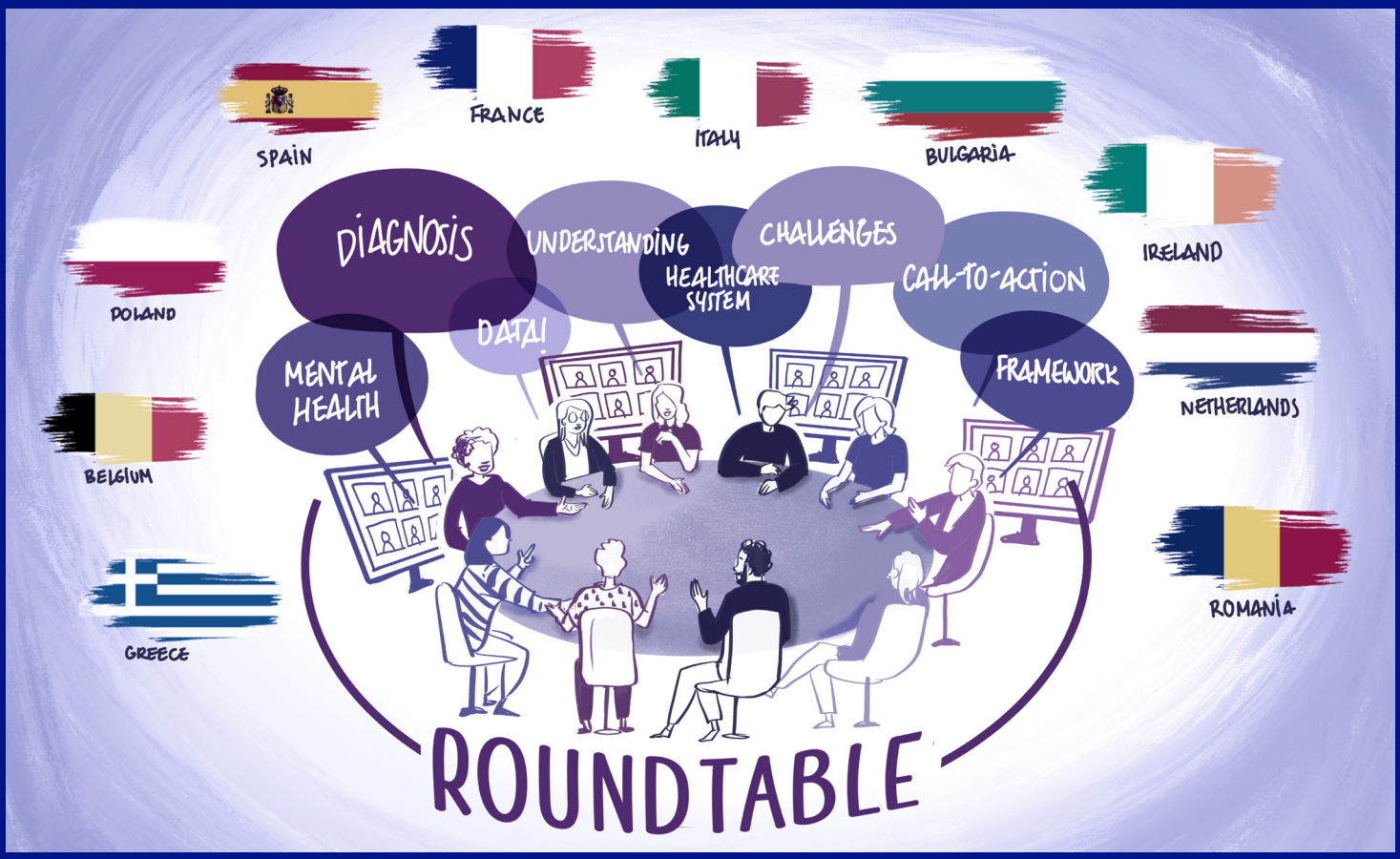
Table of Contents

Who is this report for?

We want the often-overlooked voices of people living with MG and other rare diseases to be heard in the corridors of power and 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, wherever in Europe they may live.

Acknowledgements

We give special thanks to the patient experts who participated in our MG Patient Experience Expert Roundtable, and their respective organizations whose input made this report possible.



Collaboration: An open dialogue with patient representatives from across Europe
A live illustration from the MG Patient Experience Expert Roundtable

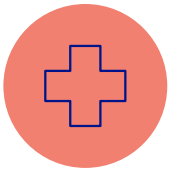
Context

Rare 2030 Foresight Study[5] – Contextualizing the unmet needs of patients living with MG

> Rare 2030 is a foresight study coordinated by some of Europe's most dedicated key opinion leaders in the field of rare disease policy. It gathered the input of hundreds of healthcare professionals and thought leaders, and thousands of people living with a rare disease, to make policy recommendations to improve the future for people living with a rare disease in Europe.'

Through the methodology of foresight, Rare 2030 consolidated stakeholder perspectives on a number of issues important to rare disease action and policy. Alongside these discussions, a survey[6] gathered the views of thousands of people living with a rare disease on this topic. Findings included the following strongly supported ideas:

- To improve coordination of healthcare.



- Better integration of health and social care to improve quality of life.

- Implementation of new-born screening for rare conditions.

- Active involvement of patient organizations in research.

Rare 2030 recommendations most pertinent to MG:

A European policy framework guiding the implementation of national plans and strategies, regularly monitored and assessed by a multistakeholder body.

Earlier, faster, and more accurate diagnosis via consistent standards and programs across Europe, new technologies, and innovative approaches driven by patient needs.

A specialized healthcare ecosystem, with political, financial, and technical support at European and national levels across the patient pathway.

Rare disease research maintained as a priority across research disciplines.

Optimized use of data to improve health and well-being.

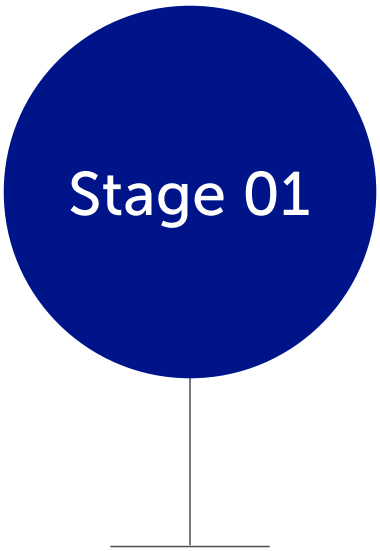
Improve the **availability, accessibility, and affordability of rare disease treatments** for all.

> Rare 2030 provides a powerful mandate for people living with MG, their families, and other stakeholders to advocate for a new future. Throughout this report, we use these recommendations as an anchor for specific actions that must be taken to improve the lives of those with MG.

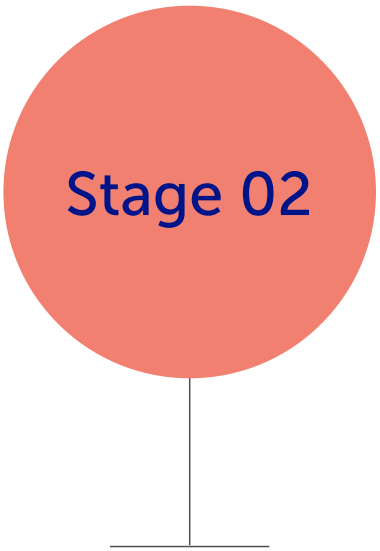
Uncovering authentic experiences for those living with MG

In 2021, following publication of the Rare 2030 Foresight Study conclusions, UCB carried out extensive patient experience insight gathering to illuminate the true burdens faced by people with MG in Europe.

Methodology



Stage One was an in-depth social listening exercise to explore individual perspectives and anecdotal insights shared by people affected by MG across countries and demographics via social platforms.



Stage Two was an MG Patient Experience Expert Roundtable in October 2021, which brought together patient organizations from across Europe – to evaluate the social listening findings, identify country-specific nuances, and discuss recommendations to address them.

> An artist joined the roundtable meeting – interpreting the discussion and perspectives shared. Throughout this report you will find examples of those illustrations to help bring to life the main issues.

Additional Context

The Lived Experience of Myasthenia Gravis: A Patient-Led Analysis[4]

In October 2021, while this report was being developed, a new analysis was published in *Neurology* and *Therapy*. It explored the real-life experience of MG, through the eyes of the patient. 'The Lived Experience of Myasthenia Gravis: A Patient-Led Analysis', was led by a UCB MG Patient Council – nine individuals living with MG who serve as patient advocates across Europe and the United States, many of whom also contributed to this report. The paper is the result of detailed patient input and an exhaustive literature search, offering an unprecedented level of first-hand information for healthcare providers into the challenges facing people living with the condition.

Five key themes emerged from this analysis including:

- 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

This paper adds to the expanding base of literature on the physical, psychological, social and day-to-day experience of living with a rare disease, and is unique in its patient-led, co-authored exploration of the nuanced reality of MG. The themes, insights and calls-to-action are reflected and built upon in this report, which aims to provide another pivotal step forward in elevating the voice of MG patients, to help navigate change in Europe.

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, USA and Kelly Davio from London, UK, in collaboration with UCB. The study was published posthumously following Nancy's death in September 2021.

Nancy's invaluable contribution to this paper and leadership of the MG patient advocacy community is hugely appreciated by all involved.

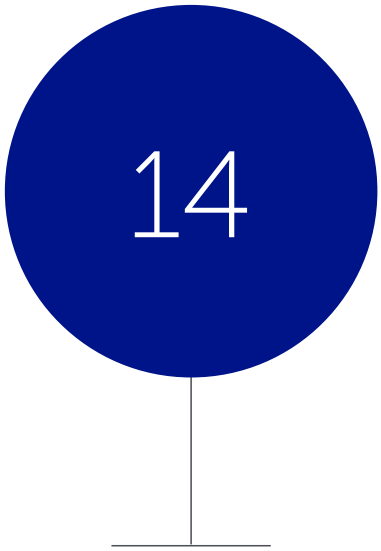
The experience of living with MG[7]

To delve deeper into the experiences of people living with MG through the lens of Rare 2030, UCB used a robust assessment of unprompted comments made on social media platforms from across the US, France, Germany, Spain, Italy and the UK to gain a representative picture of the lived experience of MG. Four prevalent themes were identified.

1. A failure to be recognized

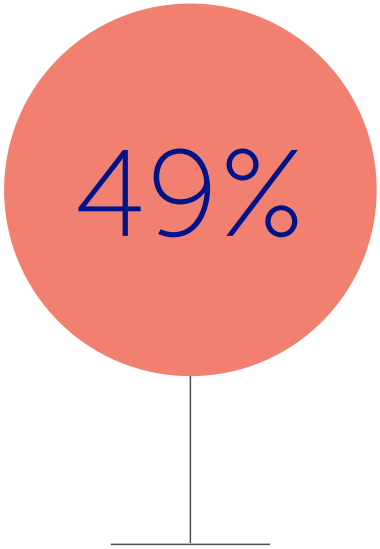
Frequent misdiagnoses and confusion related to the condition are reported. Feelings of exasperation and being misunderstood are also reported, in addition to physical symptoms being overlooked.

Notable findings:



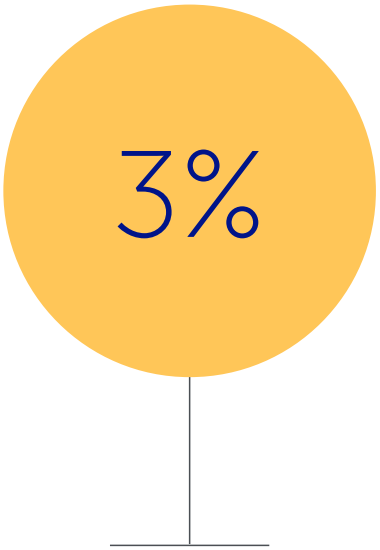
MG was reported to have been misdiagnosed as **14 different diseases** including stress, fibromyalgia, and diabetes.[7]

Base: 65 mentions



49% of these misdiagnoses are mental health conditions.[7]

Base: 65 mentions



Yet just 3% of online discussion is attributed to mental health-related symptoms in relation to MG.[7]

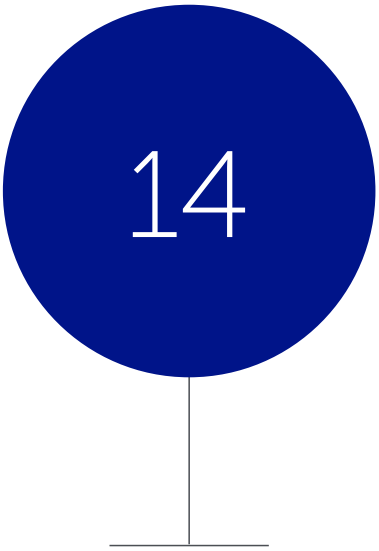
Base: 315 mentions

The experience of living with MG

2. Lack of adequate support

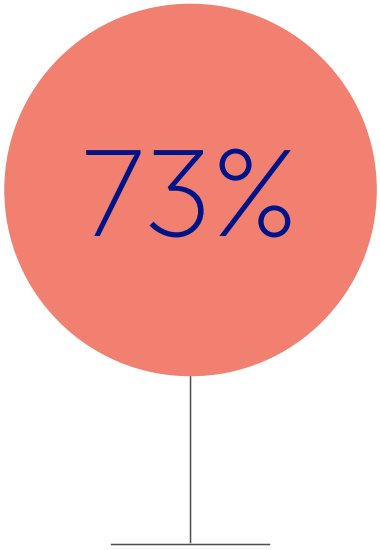
Barriers to accessing care in a complex specialist landscape, with no clear support system to rely on compounds negative experiences. A lack of awareness and defined pathway through the healthcare system makes it difficult for patients and clinicians to interact consistently and productively.

Notable findings:



MG patients reported contacting over **14 different types of specialists** at some point in their care.[7]

Base: 118 mentions



Whilst 73% of patients mention being referred to neurologists, 1 in 10 are referred to other specialists such as;
Rheumatologists (2%)
Psychologists (2%)
ENT specialists (1%)
Pulmonologists (1%)
Psychiatrists (1%)
and paediatricians (1%)[7]

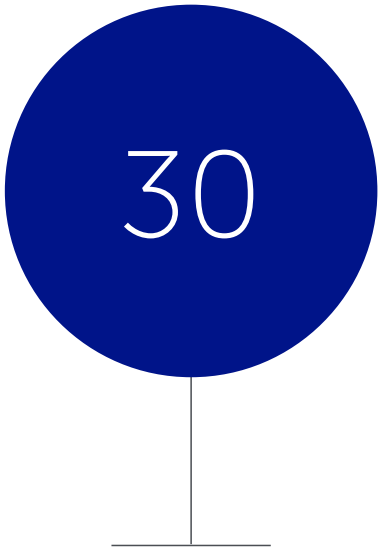
Base: 118 mentions

The experience of living with MG

3. High self-sufficiency

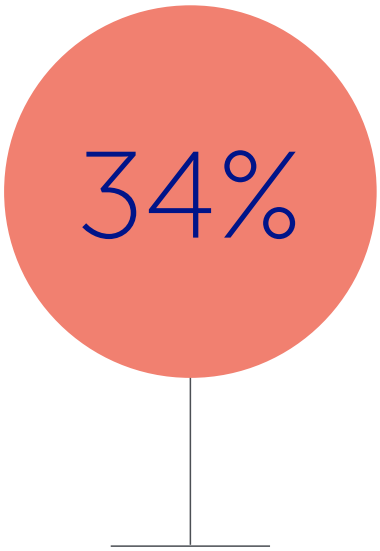
Coping with MG requires self-education and adaption to everyday life, often without professional support. Patients feel isolated and alone, struggling to cope with the unpredictable impacts of MG.

Notable findings:



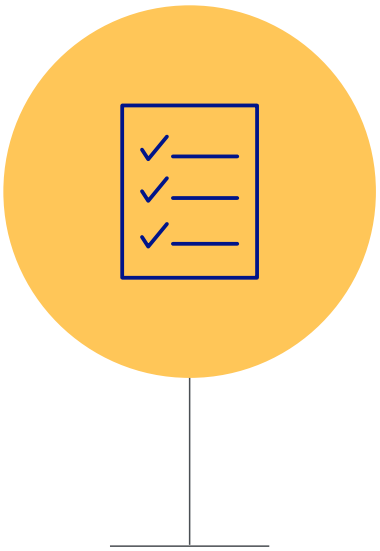
Patients describe deploying nearly **30 different coping mechanisms** to manage the physical and psychological burden of disease.[7]

Base: 397 mentions



Self-education (34%) was the most frequently mentioned.[7]

Base: 397 mentions



Patients make practical adaptations every day to cope with symptoms such as when washing, dressing, and undertaking caring duties.[7]

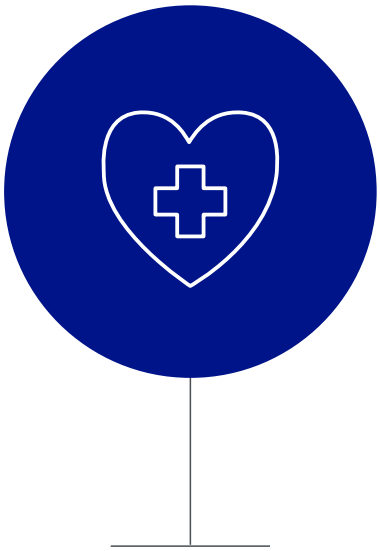
Base: 397 mentions

The experience of living with MG

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.

Notable findings:



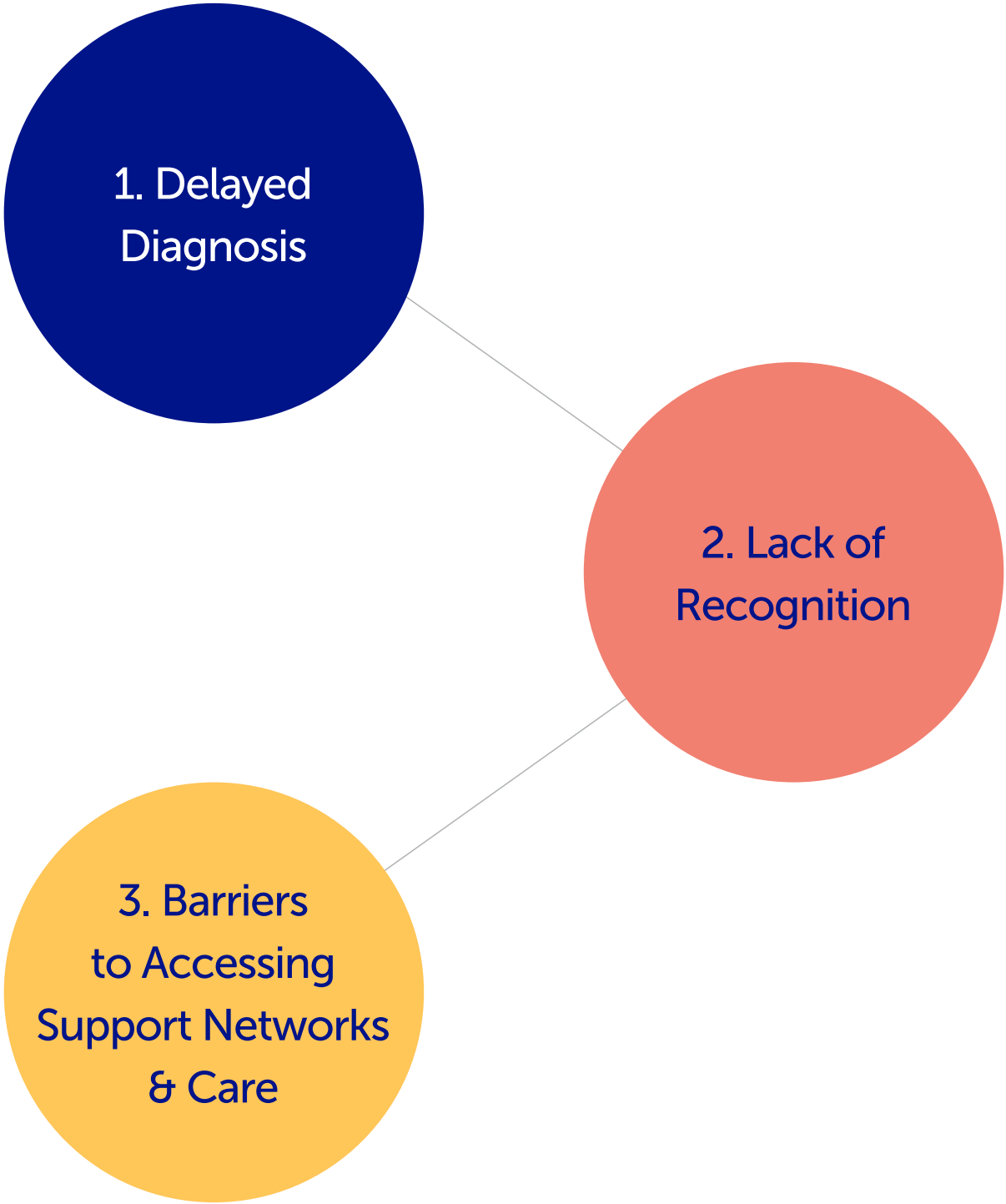
The number one driver of **patient happiness** is recognition of MG as a significant condition.[7]

Base: 301 mentions

These findings were used as the basis for the initial discussion at the Patient Experience Expert Roundtable, which led to a consolidated list of three areas of unmet need being identified.

The Three Challenges

The activities established three overarching challenges to tackle as a priority to improve the lives of people affected by MG:



This report explores the research on each challenge separately, as well as key stakeholder actions to address them.

The Three Challenges

A live-drawn illustration based on dialogue at the MG Patient Experience Expert Roundtable

1. Delayed Diagnosis

> In Rare 2030, the second recommendation is for: "Earlier, faster, and more accurate diagnosis of rare diseases through better and more consistent use of harmonized standards and programs across Europe".[8]

It is well known that people living with rare diseases often face a long and arduous journey to initial diagnosis. This so-called "diagnostic odyssey" can generally be attributed to a lack of sufficient knowledge and awareness across multiple clinical centers, specialties, and practitioners, with wide ranging needs[9] (e.g. primary care doctors need a basic level of knowledge, whereas specialists need the tools to diagnose MG quickly when presented).

Such delays place a substantial burden on healthcare resources, but the patient experts also highlighted the real and emotional impact of delayed diagnosis on patients and their families – echoing the sentiment expressed in the social listening exercise.

It is well known that people living with a rare disease often face a long and arduous journey to initial diagnosis.

Despite advances in rare disease policy and treatment innovation, there remains an enormous deficit in diagnosing rare diseases.[9]

Diagnosing MG has many challenges

Like many other rare diseases, MG presents significant challenges for an accurate and timely diagnosis. MG patients report experiencing nearly 50 different symptoms.[7] Symptoms are diverse, fluctuating and can be non-specific, such as weakness and fatigue, with each patient experiencing unique combinations. Mental health challenges are commonly co-occurring symptoms, which can also lead to misdiagnoses.

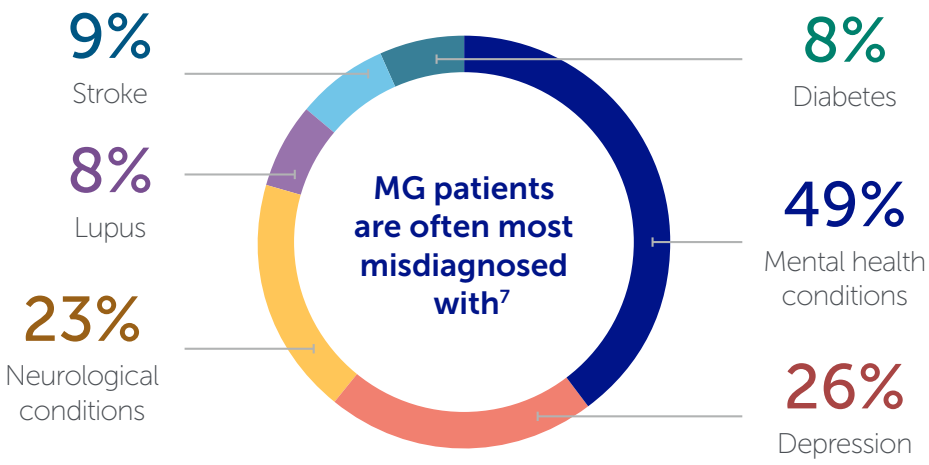
This can then send patients on frustrating and ineffective paths around specialists (the diagnostic odyssey), or even lead to them being sent home with a prescription for anti-depressant or anxiety medication, rather than an MG diagnosis.

Our social listening data reported MG patients being most often **misdiagnosed with mental health conditions (49%)**, chief among them **depression (26%).**[7] Patient experts reinforced this finding, stating that mental health symptoms are often recognized first by doctors, despite other MG symptoms being present.

Another common form of misdiagnosis discussed was MG being mistaken for **neurological conditions (23% of misdiagnosis mentions),** led by **lupus (8%)**.[7] **Stroke (9%)** and **diabetes (8%)** also received significant mentions.[7]

> People living with MG reported 14 different medical specialists being involved in their care, many of whom are irrelevant to the appropriate treatment and management of MG.[7,4]

Attendees at the Expert Roundtable also reported that even among relevant specialists, such as neurologists and ophthalmologists, many clinicians had inadequate experience with MG or are not confident or equipped to prescribe the most appropriate treatments when patients present with more severe symptoms.



Base: 65 mentions

A Diagnostic Odyssey in Poland

"In Poland, people experience similar problems in securing a diagnosis to those experienced in other European countries. Patients end up visiting many specialists including ophthalmologists, ENT doctors, and those focused on mental health issues. Even once a person has an MG diagnosis, many specialists in secondary care feel underqualified to treat the condition when it has already intensified, creating greater delays as patients wait for an appointment with a clinician who is adequately qualified to prescribe optimal treatments.

Throughout this process, people suffering these symptoms are faced with the challenge of describing their symptoms accurately as they fluctuate considerably, and many do not know which terminology to use to help their clinician understand what is happening to them. It is therefore imperative that more is done to make sure that the initial misdiagnosis does not occur, or is corrected quickly, and that people are able to get in front of an appropriate specialist as soon as possible."

Marlena Pasternak,
Polish Association of Patients with Myasthenia Gravis
"Gioconda",
Poland

Tackling the specific diagnostic challenge of fluctuating symptoms

"Many MG symptoms fluctuate, which makes diagnosis and recognition more difficult. A person living with MG may have a more severe ocular or fatigue-related symptom one day and then, by the time they see their doctor the following day, their symptoms have changed, both clinically and in terms of severity.

We therefore need to find solutions that make it easier for people who suspect they have MG to explain this experience to clinicians and for this to be recognized in MG diagnosis."

Annie Archer,
Groupe d'intérêt Myasthénie de l'AFM Téléthon,
France

Improving diagnosis timelines

Tackling the diagnostic odyssey is imperative as patients subjected to it suffer from diminished quality of life.[9]

Included below are foundational recommendations that could improve diagnosis timelines:

Investment in specialized centers of excellence and reference centers where they do not currently exist and ensuring all relevant health care professionals are aware of when to utilize them.

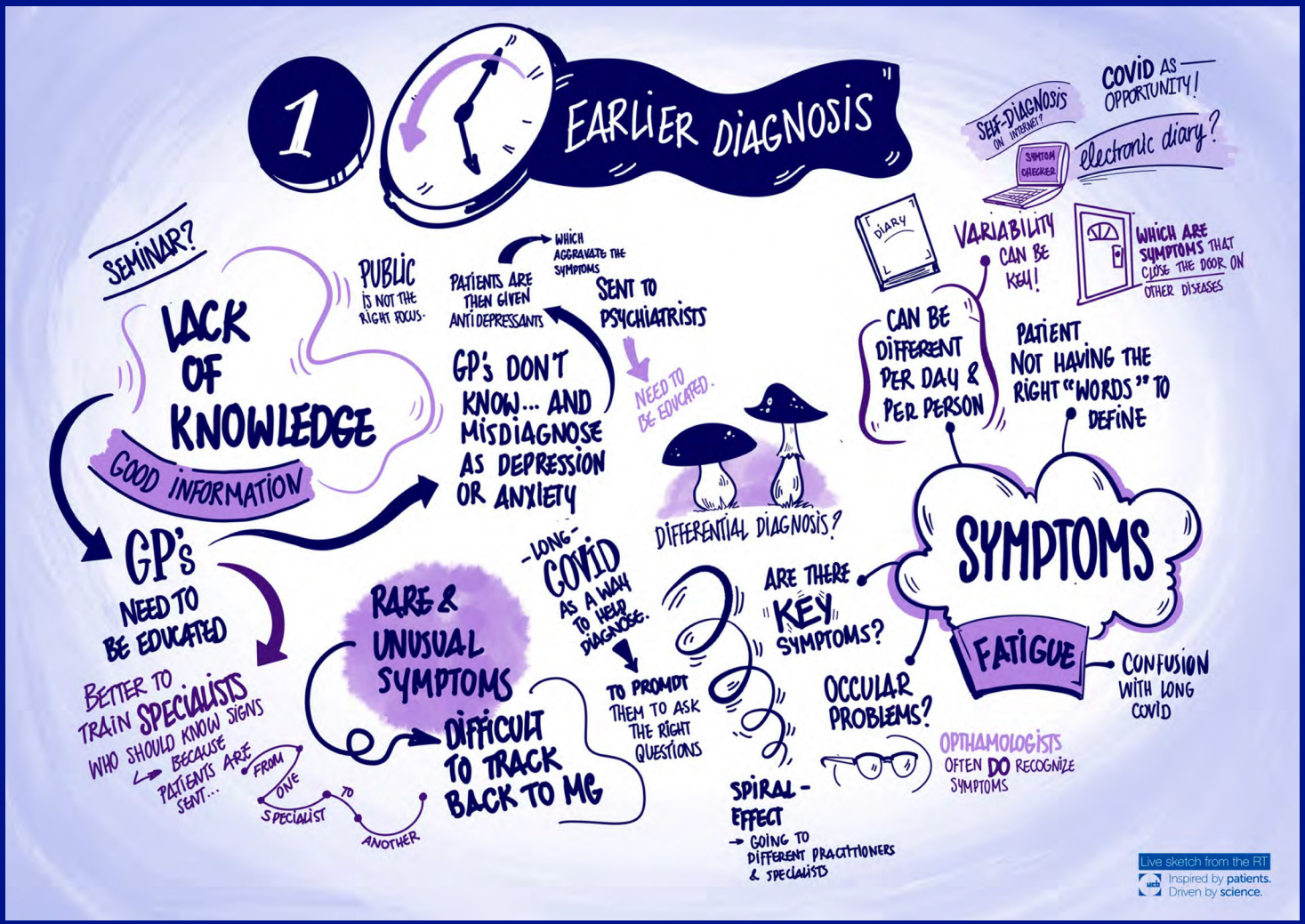
Training and awareness raising among primary care professionals, in particular to tackle the common misdiagnosis of MG as depression or anxiety.

Training and awareness raising among specialities that people living with MG are often referred to as part of an initial misdiagnosis, such as psychiatrists.

Providing symptom tracking tools for people who suspect they may have MG in order to facilitate more accurate diagnosis.

Collateral such as interactive guides or smart resources to facilitate better patient-clinician conversations, as well as better target the diagnosis process (when appropriate).

Improving diagnosis timelines



Diagnostic odyssey: A web of MG diagnosis challenges
A live illustration from the MG Patient Experience Expert Roundtable

2. Lack of Recognition

> In Rare 2030, the fourth recommendation includes a focus on "inclusion of integrated and person-centred, long-term care for people living with a rare disease and their families".[8]

Alongside issues with diagnosis, insufficient awareness and knowledge on the lived-experience of MG has far-reaching impacts.

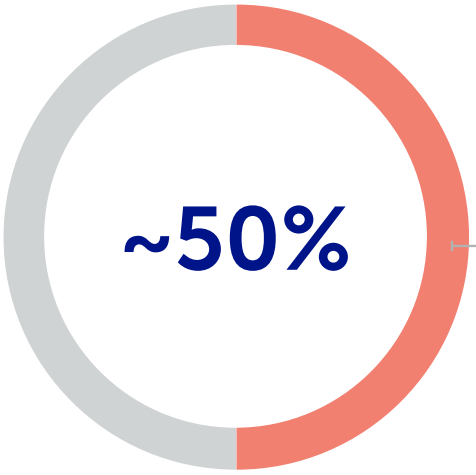
> Stigma, discrimination, and exclusion – direct or otherwise – amongst employers, health and social services, and society at large are part of life for many living with a rare disease.[10]

Rare disease patients and their families often experience not being taken seriously when trying to access support. Furthermore, limited access to adequate care and sparse information about the disease often results in negative experiences, such as frustration and self-doubt.[7]

This vicious circle is compounded by the fact that a lack of general awareness means that many people remain unaware of their risk of suffering from a rare disease – as such, patients can experience significant morbidity for many years before receiving an accurate diagnosis.

Recognizing MG

People living with MG are acutely vulnerable to the detrimental impacts of low disease burden. In an analysis of MG patient experience, the priority driver of happiness is recognition of MG as a significant condition.[7]



Base: 108 mentions

The analysis also revealed that almost half of MG patients struggle to be believed when it comes to the impact of their condition – a truly devastating finding.[7]

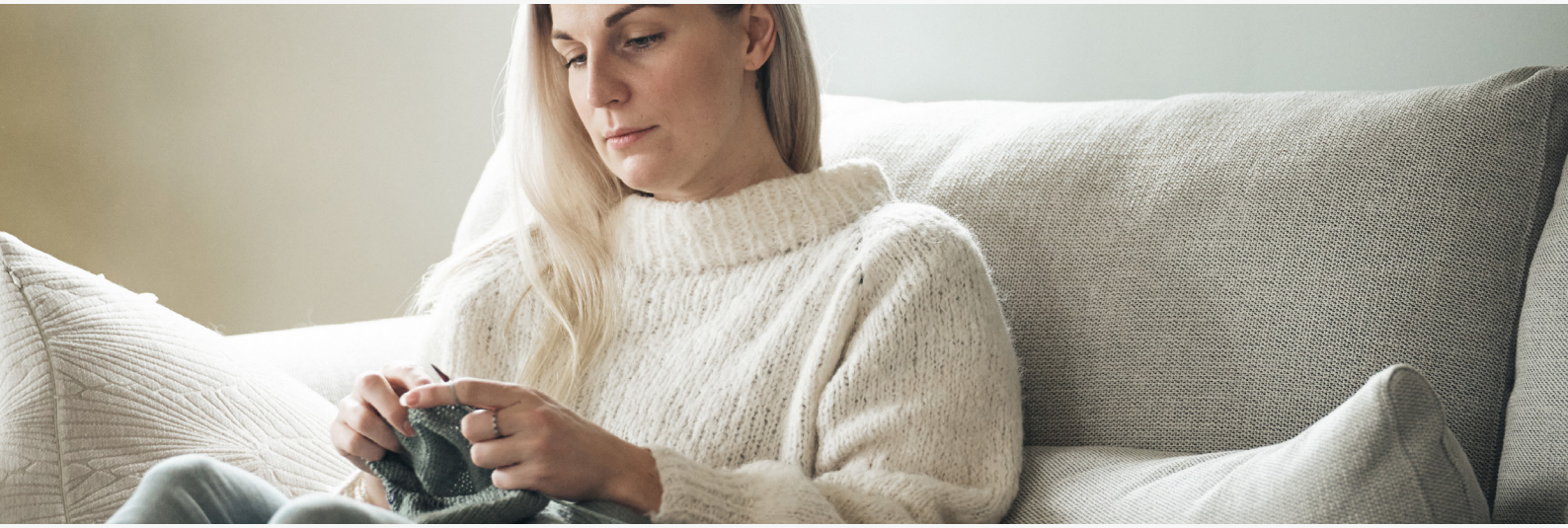
Clinical and real-world research, such as the recently published 'The Lived Experience of Myasthenia Gravis: A Patient-Led Analysis' in Neurology & Therapy,[4] is generating a better understanding of physiological challenges, but there remains a lack of recognition of the first-hand lived experience 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 MG populations.

Under-recognition of MG in Bulgaria

With a lack of appropriate clinical tools, it is far too easy for clinicians to underestimate the impact of MG and the problem of under-recognition is consequential both for treatment and diagnosis.

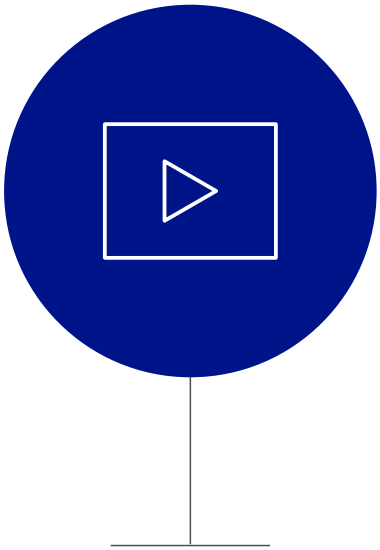
Equipping people who suspect they may have MG, as well as those moving through the patient pathway post-diagnosis, with more information on the condition could help patients present a more accurate description of their condition to their doctors, and break the negative cycle of under recognition. It is just as important however that the health system itself develops appropriate clinical tools throughout the pathway to understand a patient's experience and acknowledge the often severe impact symptoms have on patients' quality of life.

Irena Neshovska,
Foundation Myasthenia Gravis,
Bulgaria

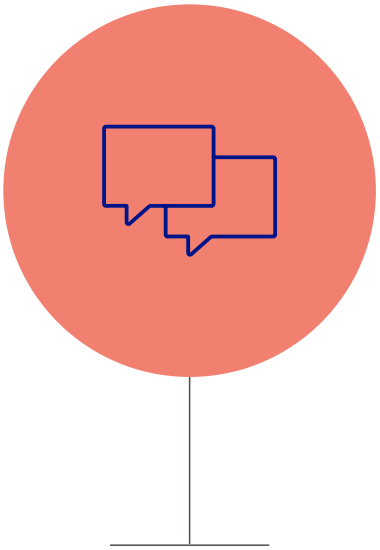


Overcoming barriers to recognition

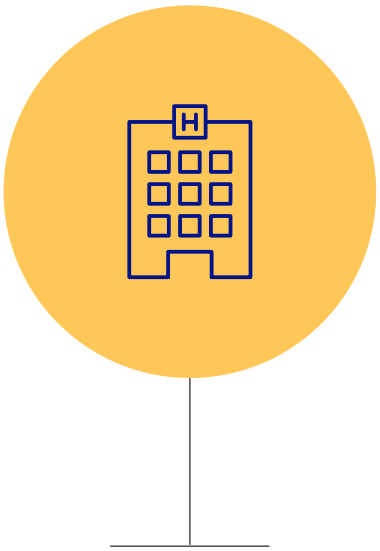
Patient experts recognized that efforts to increase broad general awareness of MG would be challenging, given the sheer number of medical conditions – rare or otherwise – vying for attention at a public level. But they did identify recommendations for the engaged MG community:



A coordinated, pan-European effort to increase the understanding of MG, **leveraging new compelling assets such as videos.**



Peer-to-peer education and advocacy – elevation of 'patient champions', including carers and families, to educate others living with MG and empower through shared knowledge and experience.



Harness centers of excellence for treatment and diagnosis, to also be hubs for information and building understanding amongst different stakeholder groups.

Overcoming barriers to recognition

Lack of recognition creates a "snowball" of confusion and frustration

A live-drawn illustration based on dialogue at the MG Patient Experience Expert Roundtable

3. Barriers to Accessing Support Networks & Care

People living with rare diseases consistently describe difficulties accessing the information and support they need.[11] Many rare diseases do not have a dedicated support group – despite evidence that people affected by a rare disease can benefit greatly from peer engagement.

Social media has provided welcome opportunities for new forms of support networks, allowing rare disease patients to connect across geographical boundaries and find comfort from one another. Researchers are increasingly turning to social media for clinical trial participants, but there is still some way to go until appropriate methodologies for remote participation can offer opportunities for all rare disease patients.

Accessing professional support for MG

As a result, MG patients are independent and self-driven, as supported by Rare 2030 findings. The patient experts highlighted the inter-reliance of MG patients and beneficial role of patient-patient support networks.

> While more treatment innovation is needed, leveraging this clear inter-reliance between and with the patient community is particularly critical to help them feel supported emotionally and psychologically.

The patient experts also identified a rural-urban divide, in terms of access to and the quality of care. Considering also that Europe has a relatively large variety of health systems – organized nationally, regionally and locally – a pan-European care pathway and service reorganization for MG is likely to be elusive.

Empowering people through patient networks in the Netherlands

"While it is very important that psychological services are available to those that need them, many people recently diagnosed with MG may find that support from a patient group locally or online is able to help them cope in addition to healthcare professional advice.

For example, if a newly diagnosed person is asking questions like "what medication should I be asking my doctor about?" or "how do I handle my parenting or professional responsibilities?", a network of peers may be able to provide useful and practical advice given their direct experience."

Johan Voerman,
Spierziekten Nederland,
Netherlands

Patient-organized support groups in Romania

"Our association provides a hotline, which is frequently used by recently diagnosed people living with MG. We listen to them, try to understand them and then help them understand what is happening to them. We are then able to point them towards other resources including our own support groups.

For example, we host our main support group on Facebook with almost 1,000 members. Most of these are patients but some of these are also relatives and healthcare professionals. These family members and clinicians – mainly young neurologists and primary care doctors – are interested in everything that happens to people living with MG.

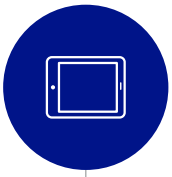
In the Facebook group, people share their experiences. The group's role is not to replace the role of healthcare professionals, or recommend medicines. Our aim rather is to understand each other and share our MG experiences so that those newly diagnosed do not feel isolated or misunderstood.

During the pandemic we organized online groups to reach as many patients as possible and may continue this in future."

Adriana Harja,
Romanian Myasthenia Gravis Association (RoMGA),
Romania

Putting in place strong support & care networks

Discussions at the Roundtable concluded that – due to differing context or country-specific factors – it was a challenge to develop recommendations that would fully harmonize complex MG care and support network needs, therefore the group focused on steps that could easily be adapted to local situations:



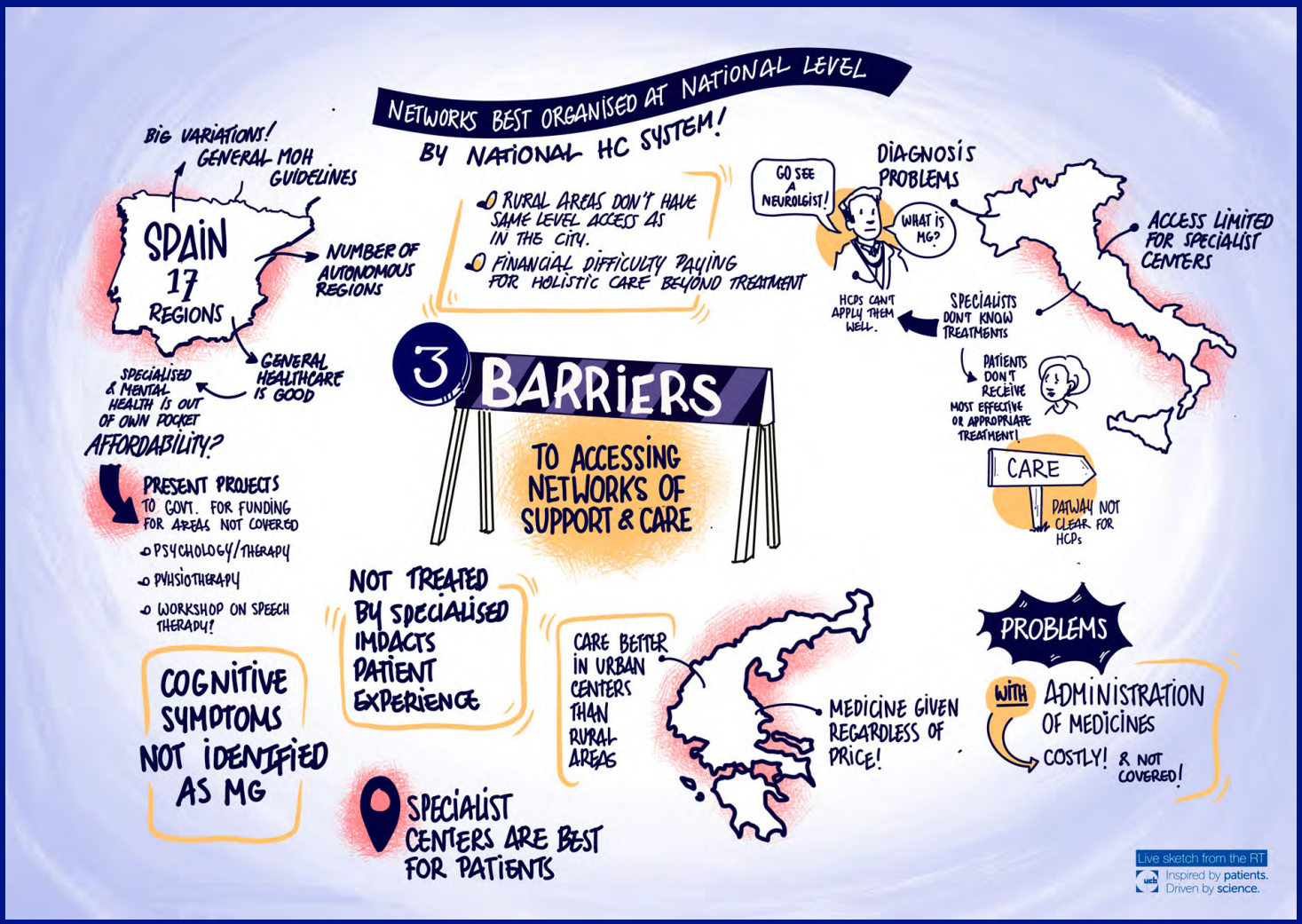
Collaborate on a pan-European, digital "space" or "forum" for those living with MG, being overseen by expert moderators, but also allowing for smaller discussions on specific topics.

Develop resources that allow for the creation of local, grassroots and/or small community networks – making those available in different languages, and easily accessible across relevant national or European platforms.

Develop ways to ensure recently diagnosed people living with MG are connected by their healthcare professionals to **patient support networks** as early as possible in their diagnosis and treatment journey.

Continue the sharing of best practices between patient associations in Europe and globally to deliver the most effective support networks and identify priority needs to address.

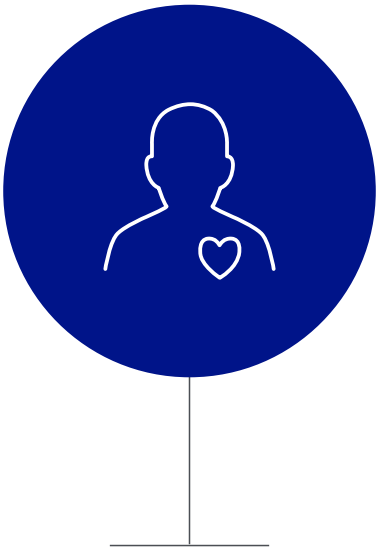
Putting in place strong support & care networks



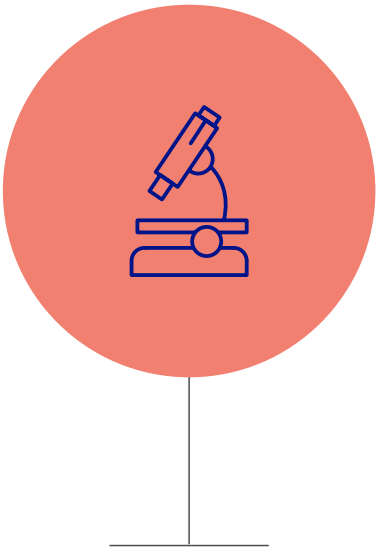
Multidimensional factors combine to create stubborn barriers to support and care

A live-drawn illustration based on dialogue at the MG Patient Experience Expert Roundtable

Conclusion



This report has been driven by the need to improve MG patient experiences to align with advancements in scientific knowledge and policy in rare disease.



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 MG patients and their families.

Insights and recommendations included in this report are anchored by this need to better understand the MG patient experience with an emphasis on what can be done – across stakeholders – to address it.

This is just the start. New evidence, continued collaboration and industry endorsement are all required to turn recommendations into action.

Priority Focus Areas

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 Roundtable representatives:

Action 01

Coordinated national and European policy strategies to address the delayed diagnosis and under recognition of MG, with particular focus on the establishment of effective reference networks in every country and increased training for relevant specialists.

Action 02

Production of **more effective diagnostic triage tools** and collateral that include prompts to recognize the fluctuating symptom profile of MG for primary and specialist clinicians.

Action 03

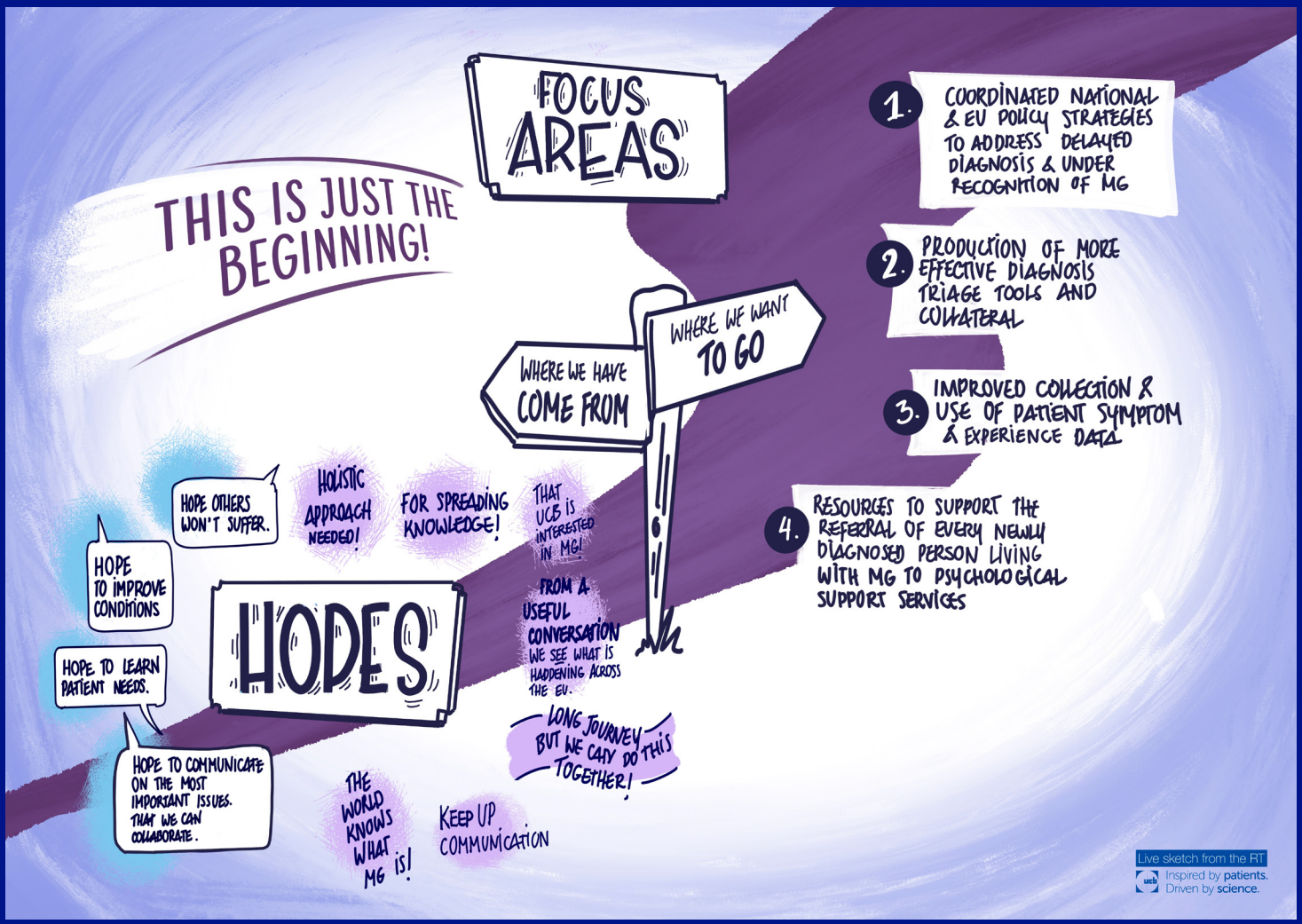
Improved collection and use of patient symptom and experience data (real world evidence) to inform diagnostic tools, patient support materials and clinical research.

Action 04

Resources to support the referral of every newly diagnosed person living with MG to psychological support services if needed and a patient support network.

> Development of this report is also an important step forward – uniting advocates and experts from across Europe 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 patient communities and these voices must remain at the heart of MG advocacy efforts.

Just the beginning



Forward-thinking collaboration will help advance progress for the MG community

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Kamilla, living with MG

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

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