



UNMET NEEDS

in **Neuromyelitis
Optica Spectrum
Disorders**
in Europe



Introduction

Across Europe, 30 million people live with a rare disease.¹ For many, the journey to accurate diagnosis is complex and frustrating — mostly because awareness of their disease is low, even among providers.

One such rare disease is an autoimmune condition of the central nervous system called Neuromyelitis Optica spectrum disorders (NMOSD). A rare autoimmune condition of the central nervous system, NMOSD causes deterioration to a person's optic nerve and spinal cord. Deterioration can result in permanent vision loss, paralysis, loss of bowel and bladder control, and muscle weakness.²

NMOSD affects over 10,000 people in Europe but is most common in women in their 30s and 40s. It also disproportionately affects people of African or Asian descent.^{3,4}

Despite availability of diagnostic tests for NMOSD, it is frequently

misdiagnosed as multiple sclerosis.⁵ Symptoms across both diseases are similar. But misdiagnosis of NMOSD is of great consequence to patients.

NMOSD is a serious condition. Patients who go untreated, or inadequately treated, are at high risk of permanent disability — or worse, death.

To mitigate the severity of NMOSD, patients must receive a timely diagnosis. It is the launch off point for effective treatment and quality care. Several key barriers hinder patients' taking control of their NMOSD. These are:

- **Lack of awareness**
- **Shortage of specialists**
- **Outdated treatment guidelines**
- **Inequitable access to innovative treatments**
- **Inadequate support for caregivers**

Lack of Awareness

NMOSD is unknown by the general public, by and large. Even health care providers seldom recognise it, especially outside specialty centres.

After a first attack—marked by sudden vision loss and inability to control the bowels—a patient’s first stop is often the emergency room. There, clinicians run tests to look for any abnormalities that can shed light on the patient’s condition. Most centres, though, rarely have a physician on hand knowledgeable enough of NMOSD’s symptoms to accurately identify it.

As a result, NMOSD patients will experience relapses and make repeated trips to the emergency room in attempts to learn what is impacting their life.

Lack of NMOSD awareness, especially among emergency clinicians and primary care providers, is a critical unmet need. When left undiagnosed and untreated, NMOSD symptoms can worsen over time.² What begins as sudden vision loss or loss of sensation can lead to paralysis or even death.

Steps Forward

Disease awareness is necessary for optimal patient care. Education campaigns targeting emergency clinicians and primary care providers can broaden awareness of NMOSD and improve timely, accurate diagnosis.



“ With more knowledge of the disease, health care providers will diagnose and treat patients earlier, or direct them to the right centres to be cared for adequately. ”

Matthias Fuchs,
Patient, Germany

Shortage of Specialists

Patients also need medical care from a specialist who's familiar with NMOSD. Patients access to these providers, however, is limited by a number of factors.

Accurate referral depends on a primary care provider's interpretation of their patient's symptoms. Due to NMOSD's range of symptoms, primary care providers may serially refer patients to improper specialists — ophthalmologists, radiologists or gastroenterologists, among others — before landing the right one. This ping-ponging can delay accurate diagnosis several years.

Across Europe, there is a shortage of neurologists who specialise in inflammatory demyelinating autoimmune conditions. Patients are burdened with day-long drives, sometimes flights, to see the right specialist. And because the number of specialists who treat NMOSD is slim, many patients must endure a waiting list before ever walking through the door.

A knowledgeable specialist will proactively conduct a variety of specialised tests, including MRI, CT scans and AQP4-IgC antibodies screening, which are highly specific for NMOSD.⁶ Specialists can also prescribe optimal treatments that prevent future attacks and reduce disability risks associated with NMOSD.

Steps Forward

As availability of specialty neurologists is limited, NMOSD patients should have alternatives, to stay consistent with medical appointments. Telehealth, for example, should be offered to patients who travel far distances for specialist care. Additionally, after diagnosing a patient with NMOSD, specialists should coordinate care and inform all members of the patient's health care team.



“ We regularly see the impact that a lack of trained specialists in NMO has: long waiting times, great distances to travel to see the right doctor and sometimes a delay to diagnosis because the disease is not always recognised by the first specialist you see. ”

Camilla Bohm Coleman, Patient, Sweden

Outdated Treatment Guidelines

Treatment guidelines and consensus statements align the medical community on how to best diagnose and treat diseases. They are also important to policymakers and insurers as they develop coverage policies and formularies.

Current European treatment guidelines to diagnose and treat NMOSD were most recently published in 2010.⁷ However, the European Medicines Agency has since approved therapies—one as recently as 2021—to reduce NMOSD patient relapses and prevent permanent disability.

Without updated guidelines, patients are unable to access these innovative treatments. And clinicians will remain unknowledgeable of optimal courses for diagnosing and treating NMOSD patients they encounter.

As these tools evolve to reflect scientific advances, they must be accessible to people outside the healthcare system, advocacy groups and patients especially.

Advocacy organisations and patient support groups channel educational tools and messages to patients. They are key voices in discussions with policy makers when advocating for access to patient-centred care.

Steps Forward

As European guidelines to treat NMOSD are updated to include new therapies, there is an even greater need to develop and distribute educational materials clearly understood and accessible to patients and policy makers. Including patient perspectives in guideline development will also ensure recommendations are patient centred.



“ Updating the European NMOSD treatment guidelines will ensure patients have new evidence-based medicines available to them. More importantly, including NMO patients in future guideline discussions will ensure they are patient-focused and raise awareness of NMOSD among patients, providers, policy makers and the public. ”

Professor Romain Marignier, MD,
NMO Specialist, France



Inconsistent Access to Innovative NMOSD Treatments

The European Commission's approval of new therapies does not guarantee patients can access them. Like most rare disease treatments, those for NMOSD can be costly.

Governments and healthcare systems in countries throughout Europe can decide the treatments are not cost effective. And in the event they do, patient access becomes nearly impossible.

To navigate this, patients and advocacy organisations should engage early and often with health technology assessment bodies. These bodies provide recommendations on therapies that can be financed or reimbursed by the health care system. Their processes can be difficult to follow and

aren't always receptive to advocates' and patients' input, however. Also, definitions of value and cost effectiveness vary by country, so accessibility will differ from patient to patient.

Patients provide important perspectives on treatment value and needs for NMOSD.

Steps Forward

Patient perspectives must be integrated into health technology assessment bodies' decision-making. NMOSD patients can successfully engage these

bodies if equipped with tools and resources to understand them.

Patients have the power to communicate to policy makers the value of access to care for people living with NMOSD.

“ A suitable treatment is essential to lead a fulfilling life with NMOSD. ”

Christiaan Waters,
Patient, Netherlands

Poor Support for Patient Carers

Patients with NMOSD, especially those with permanent disabilities, may face difficulties with everyday tasks that require sight and movement. This can create challenges living independently, requiring support from families and carers.

The role of families and carers, however, is often overlooked by health care systems. To adequately support NMOSD patients, carers must sometimes sacrifice their own priorities. This can include their jobs, family needs, social activities and personal obligations.

As a result, carers can face significant stress – financial and emotional. To adequately serve a patient with NMOSD disabilities, carers must also have their own needs met.

Key members of patients' health care teams, carers also require educational tools to fully understand the needs of patients with

NMOSD. Emotional support is also critical as carers navigate life's responsibilities while simultaneously caring for loved ones full time.

Steps Forward

Not only do NMOSD patients need more accessible education tools and resources, so too do families and carers. Hospitals and specialty centres must recognise them as vital members of a patient's health care team. All that can be done should be done to set them up for success. This can include:

- referring them to support groups,
- sharing easy-to-understand educational tools, and
- providing a point of contact at the hospital or specialty centre if they have questions about NMOSD.



“ Caregivers need the same access as the patient to high quality information and resources about NMO, as it is important they work together as a team. ”

Souad Mazari, Founder
NMO France Association, France



Conclusion

Patients living with NMOSD in Europe have unmet needs that prevent them from receiving adequate care. Their condition is little known outside specialty circles. Left undiagnosed and untreated, NMOSD poses a high risk of permanent disability and, in some cases, death.

Policy makers must address barriers patients face. They can:

- Target emergency clinicians and primary care providers with education campaigns about NMOSD
- Improve telehealth options so patients can access critical specialists practicing at a burdensome distance
- Update guidelines and make them clear and easily understood by patients and caregivers
- Encourage Health Technology Assessment bodies to consider patient perspectives in their decisions
- Treat caregivers as vital members of patients' treatment teams

Addressing these issues can materially improve understanding of NMOSD, expand treatment access and open care opportunities.

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The European Alliance for Patient Access is a division of the Global Alliance for Patient Access, an international platform for health care providers and patient advocates to inform policy dialogue about patient-centered care

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