

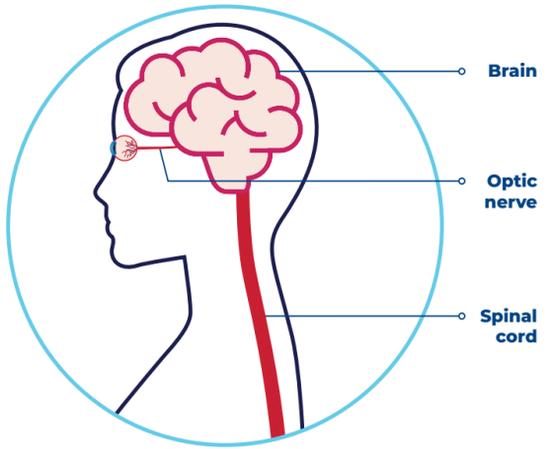
# Neuromyelitis Optica Spectrum Disorder (NMOSD)



## WHAT IS NEUROMYELITIS OPTICA SPECTRUM DISORDER?

NMOSD is a **rare disease** in which the immune system is inappropriately activated to target healthy tissues and cells in the central nervous system (CNS).<sup>1</sup>

**Approximately three-quarters of people with NMOSD are anti-AQP4 antibody-positive**, meaning they produce antibodies that bind to a specific protein, aquaporin-4 (AQP4). This binding can inappropriately activate the **complement system** to **destroy cells** in the **optic nerve, spinal cord** and **brain**.<sup>2,3</sup>



Diagnosed prevalence in adults is



NMOSD most commonly **affects women** and begins in the **mid-30s**. **Men and children** may also develop NMOSD, but it is even more rare.<sup>6,8-10</sup>

## Patients with NMOSD may experience<sup>11</sup>



**Vision problems**



**Intense pain**



**Loss of bladder/bowel function**



**Abnormal skin sensations (e.g., tingling, pricking or sensitivity to heat/cold)**



**Impact on coordination and/or movement**

Most people living with NMOSD experience **unpredictable attacks, known as relapses**. Each relapse can result in cumulative disability including **vision loss, paralysis** and sometimes **premature death**.<sup>12,13</sup>

## HOW IS NMOSD DIAGNOSED?

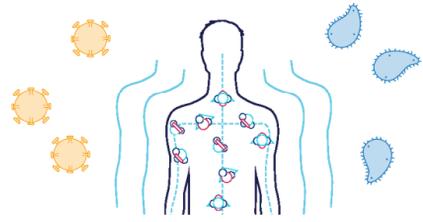
The journey to diagnosis can be long, with the disease **sometimes misdiagnosed**. NMOSD is a **distinct disease from other CNS diseases**, including multiple sclerosis (MS).<sup>14</sup>

A **neurologist or neuro-ophthalmologist** diagnoses NMOSD by one or more of the following:<sup>18</sup>

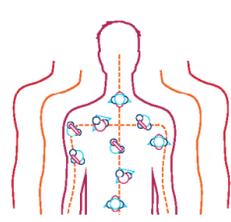


- Evidence of a blood test for the NMOSD-specific biomarker**
- At least 1-2 core manifestations of the disease (e.g., inflammation of the optic nerve or spinal cord)**
- Magnetic resonance imaging (MRI) of the brain, spinal cord or optic nerve**
- Identification of certain patterns in how the disease presents (such as length and location of the lesions caused by tissue damage)**

## THE COMPLEMENT SYSTEM



The complement system is a part of the immune system and is **essential to the body's defense against infection**.<sup>15</sup>



When the system is **thrown out of balance**, or dysregulated, these proteins can **trigger a dangerous, uncontrolled cascade of reactions** that attack cells and tissues resulting in **harmful inflammation** and the **destruction of healthy cells**.<sup>16</sup>

## WHAT ROLE DOES COMPLEMENT INHIBITION PLAY IN TREATING NMOSD?



Alexion's clinical studies in NMOSD have shown that **inhibiting the complement system** (by blocking the C5 protein) **reduces the risk of relapses**.

**Alexion's leadership in complement inhibition** has set the course for the continued study and development of innovative treatments for certain rare complement-mediated neurological diseases, including NMOSD.

## WHAT TREATMENT APPROACH IS BEING STUDIED BY ALEXION?



In addition to **developing the first approved therapy for adults with anti-AQP4 antibody-positive NMOSD**, we continue to advance research and other clinical trial programs in the disease, including an ongoing **Phase 3 study involving our long-acting complement inhibitor**.



We remain focused on **accelerating the discovery and development of new, life-changing therapies** for people living with NMOSD.

### References:

- Jarius, S., Wildemann, B. (2013). The History of Neuromyelitis Optica. *J Neuroinflammation*, 10, 797.
- Hamid SHM, et al. (2017) What Proportion of AQP4-IgG-negative NMO Spectrum Disorder Patients are MOG-IgG Positive? A Cross Sectional Study of 132 Patients. *J Neurol.*, 264(10), 2088-2094.
- Wingerchuk, D. M., et al. (2017). Neuromyelitis Spectrum Disorders. *Mayo Clinic Proceedings*, 92(4), 663-679.
- Papp, V., et al. (2020). A population-based epidemiological study of neuromyelitis optica spectrum disorder in Hungary. *European Journal of Neurology*, 27, 308-317.
- Flanagan, E. P., et al. (2016). Epidemiology of aquaporin-4 autoimmunity and neuromyelitis optica spectrum. *Ann Neurol.*, 79, 775-783.
- Bukhari W., et al. (2017). Incidence and Prevalence of NMOSD in Australia and New Zealand. *J Neurol Neurosurg Psychiatry*, 88(8), 632-638.
- Miyamoto K., et al. (2018). Nationwide Epidemiological Study of Neuromyelitis Optica in Japan. *J Neurol Neurosurg Psychiatry*, 89(6), 667-668.
- Wingerchuk, D. M., et al. (2006). Revised diagnostic criteria for neuromyelitis optica. *Neurology*, 66(10), 1485-1489.
- Drori, T., et al. (2014). Diagnosis and classification of neuromyelitis optica (Devic's syndrome). *Autoimmunity Reviews*, 13(4-5), 531-533.
- Eaneff, S., et al. (2017). Patient perspectives on neuromyelitis optica spectrum disorders: Data from the PatientsLikeMe online community. *Multiple Sclerosis and Related Disorders*, 17, 116-122.
- Mutch K, et al. (2014). Life on Hold: The Experience of Living with Neuromyelitis Optica. *Disabil Rehabil.*, 36(13), 1100-7.
- Kessler, R. A., et al. (2016). Updated Treatment of Neuromyelitis Optica Spectrum Disorder: Acute, Preventive, and Symptomatic. *Curr Treat Options Neurol.*, 18(1), 2.
- Jiao, Y., et al. (2013). Updated Estimate of AQP4-IgG Serostatus and Disability Outcome in Neuromyelitis Optica. *Neurology*, 81(14), 1197-1204.
- Mealy, M. A., et al. (2019). Assessment of Patients with Neuromyelitis Optica Spectrum Disorder Using the EQ-5D. *Int J MS Care*, 21(3), 129-134.
- Merly, N. S., et al. (2015). Complement System Part II: Role in immunity. *Front Immunol.*, 6, 257.
- Garred, P., Tenner, A. J., & Mollnes, T. E. (2021). Therapeutic Targeting of the Complement System: From Rare Diseases to Pandemics. *Pharmacological Reviews*, 73(2), 792-827.