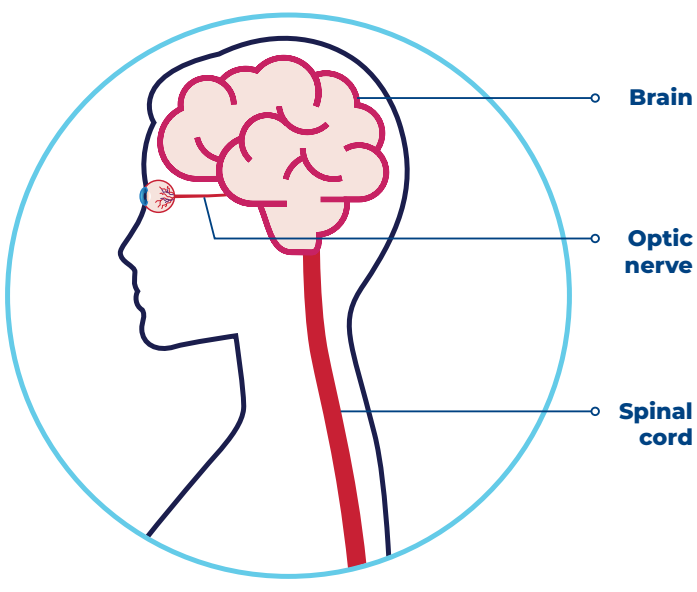
Neuromyelitis Optica Spectrum Disorder (NMOSD)

ALEXION
AstraZeneca Rare Disease

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).[1]

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**.[2,3]



Diagnosed prevalence in adults is[4,5]

~7.5K ~6K ~4K

NMOSD most commonly **affects women** and begins in the **mid-30s**. **Men and children** may also develop NMOSD, but it is even more rare.[6–9]

Patients with NMOSD may experience[10]

- **Vision problems**
- **Intense pain**
- **Loss of bladder/ bowel function**
- **Abnormal skin sensations (e.g., tingling, prickling 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**.[11,12]

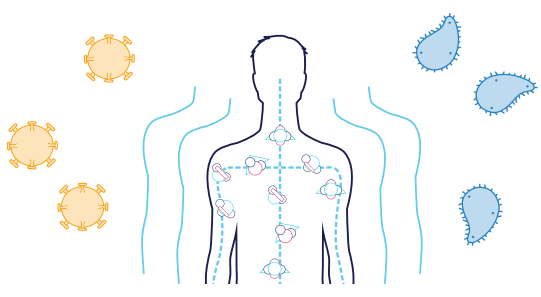
HOW IS NMOSD DIAGNOSED?

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).[1,13]

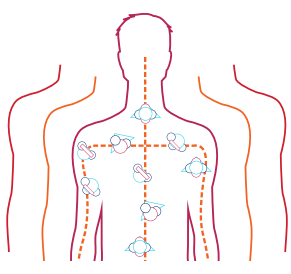
A **neurologist or neuro-ophthalmologist** diagnoses NMOSD by one or more of the following:[1,7]

- ☑ **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**.[14]



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**.[15]

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:

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