MAYO CLINIC

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American Neurological Association

Mayo Clinic Researchers Stop Neuromyelitis Optica Attacks with New Therapy

ROCHESTER, Minn. — Mayo Clinic researchers have identified a new therapy for patients with neuromyelitis optica that appears to stop inflammation of the eye nerves and spinal cord. NMO is a debilitating central nervous system disorder that is often misdiagnosed as multiple sclerosis (MS). In the study, patients with severe symptoms of the disease, also known as NMO, were given eculizumab, a drug typically used to treat blood disorders.

While not a cure, the therapy Mayo Clinic researchers used in the study to halt attacks could potentially lead to longer attack-free periods for the thousands of NMO patients worldwide. The research is being presented Oct. 9 at the American Neurological Association Annual Meeting in Boston.

NMO manifests itself in attacks that can cause blindness in one or both eyes, weakness or paralysis in the legs or arms, painful spasms, loss of sensation, and bladder or bowel dysfunction from spinal cord damage. Attacks may be reversible, but can be severe enough to cause permanent visual loss and problems with walking. NMO can affect children as young as 2 and adults as old as 90. It is more prevalent in females than males, but affects all racial and ethnic groups. Immunosuppressants are the first line of treatment for NMO.

Mayo Clinic researchers have been international leaders in NMO diagnosis and treatment. In 2004, Mayo Clinic researchers discovered the antibody NMO-IgG — the first serum biomarker for any form of inflammatory demyelinating brain disease. A year later, they identified the target of the antibody as the water channel aquaporin 4. These discoveries helped physicians better understand the cause and potential treatments for NMO.

Mayo researchers studied 14 NMO patients with active and severe disease symptoms, defined as two attacks in the previous six months, or three within the past year. When the NMO-IgG antibody binds to its target on brain cells, it activates complement, a substance that can kill or injure these brain cells. Patients

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were treated with eculizumab, an antibody that stops complement from being activated. All 14 study participants received the treatment intravenously every two weeks for one year.

"Disability in NMO is attack related and these attacks are usually severe. If untreated, they can have devastating, irreversible effects on function," says lead author Sean Pittock, M.D., a Mayo Clinic neurologist. "If we can stop the attacks in NMO — and it appears we can — then we can hopefully prevent disability and allow patients to maintain function and a good quality of life."

Twelve patients were symptom free throughout the year of treatment. Two patients had one attack each, but these were mild and considered by the investigators to be "possible, not definite" attacks. One experienced mild back pain but had no new findings on neurologic examination or MRI scan of the spinal cord. The other experienced mild visual blurring in the right eye but also was receiving antibiotic therapy for a presumed urinary tract.

Because NMO has only recently been identified as a syndrome distinct from MS, knowing how many people have it is difficult. Mayo is studying NMO's prevalence. Mayo Clinic's Neuroimmunology Laboratory so far has detected the antibody in roughly 3,500 U.S. patients.

"We've learned there are many people who fit within the NMO spectrum who, in the past, were believed to have MS," says co-author Dean Wingerchuk, M.D., a neurologist at Mayo Clinic in Arizona. "In addition, distinguishing NMO from MS is important for preserving the validity of therapeutic trials for MS by not enrolling patients with NMO."

The study was funded by Alexion Pharmaceutical, which makes eculizumab.

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