FDA approves Soliris to treat generalized myasthenia gravis

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Alexion Pharmaceuticals announced on Oct. 23 that the U.S. Food and Drug Administration (FDA) has approved eculizumab (brand name Soliris) as a treatment for adult patients with generalized myasthenia gravis (MG) who are anti-acetylcholine receptor antibody-positive. Soliris is the first in a new class of drugs to be approved for MG in the U.S.

Soliris is not a cure for MG, but it may lessen the symptoms experienced by people living with generalized MG. Soliris was tested in clinical trials in patients who had previously failed immunosuppressive treatment and continued to suffer from significant unresolved disease symptoms such as difficulties seeing, walking, talking, swallowing and breathing. Patients taking Soliris had improved scores on scales designed to assess quality of life and symptom burden, including double vision, ptosis (drooping of the eyelids), swallowing, speech, breathing, and use of arms and legs.

MDA President Lynn O'Connor Vos said in a press release, "MDA celebrates the approval of Soliris to treat generalized myasthenia gravis—the first in a new class of drugs to be approved for MG in the U.S.—and we offer our deepest thanks to the dedicated researchers, and the individuals and families who participated in clinical trials to make this development possible.

"This is truly an unprecedented time, when more experimental therapies than ever before are reaching late-stage development and moving through the regulatory review process," she said. "In just the past few years, our community has seen six FDA approvals for drugs to treat neuromuscular diseases in MDA's program—including periodic paralysis, Duchenne muscular dystrophy, ALS, spinal muscular atrophy and now myasthenia gravis. The origins of four of those six drugs can be traced directly to MDA research dollars.

"We are closing in on solutions for people living with neuromuscular diseases and will continue to fund critical scientific research, facilitate clinical trial participation, and advocate for policies that enable safe and effective therapy options to be made available as quickly as possible," she said.

Patients with generalized MG experience muscle weakness in the head, neck, trunk, limb and respiratory muscles. An estimated 10 to 15 percent do not respond to treatments that are typically helpful in other MG patients.

Profound weakness throughout the body often is accompanied by slurred speech, impaired swallowing and choking, double vision, upper and lower extremity weakness, disabling fatigue, shortness of breath due to respiratory muscle weakness, and episodes of respiratory failure. Patients with generalized MG may require hospitalization, often involving intensive-care unit stays.

Soliris is a terminal complement inhibitor that targets a part of the immune system called the complement system, which is responsible for helping antibodies clear damaged cells and potentially toxic microbes that could cause infections. In MG, antibodies whose job it is to target these toxic pathogens, instead inappropriately recruit the complement system and target the space across, which nerve fibers transmit signals to muscle fibers, called the neuromuscular junction (NMJ). In patients with anti-acetylcholine receptor antibody-positive MG, the body's own immune system turns on itself to produce antibodies against the acetylcholine receptor, a receptor located on muscle cells at the NMJ, activating the complement system. Soliris is thought to work in MG by inhibiting the complement pathway to prevent destruction of the neuromuscular junction.

MDA has not been directly involved in the development of Soliris for MG, but it has invested in previous research investigating complement inhibition as a therapeutic strategy for MG using donations from NALC members and other groups. In addition, your donations are currently funding research focused on developing improved complement inhibitors like Soliris.

End-of-year reporting and the 2017 NALC Honor Roll

Money should be turned into MDA within two days after a fundraising event has taken place. A copy of the NALC Activity Report should be included with the check/money order being sent to the local MDA office. All funds must be mailed to the MDA office by Dec. 15 each year to be counted toward the calendar year and to be included in NALC's Honor Roll. Outstanding pledges where money has not yet been received cannot count toward your year-end total. MDA can provide a receipt, called the SE-14 Income Verification Form, for all cash or checks upon request. Please mail a copy of any receipts for all cash or checks, along with the NALC Activity Report, to NALC Headquarters.

Please reach out if you have questions about special circumstances or events that conclude after the Dec. 15 deadline. If you wish to know where your branch stands with documented MDA donations for this year to ensure proper credit, please give me a call at 202-756-7403 or send me an e-mail at mda@nalc.org well before the end of the year. This will allow the time needed to research any concerns.

Deadline for the 2017 MDA Honor Roll is Dec. 15

The deadline for sending funds raised and MDA activity forms to your local MDA office is Dec. 15. Please send them by this date to be counted in NALC's 2017 Honor Roll list.