MYASTHENIA GRAVIS
FOUNDATION OF AMERICA, INC.®

JUNE 9, 2016

REVISED -- MGFA RESEARCH UPDATE: ALEXION RELEASES TOP LINE RESULTS OF REGAIN TRIAL

On June 6, 2016, Alexion Pharmaceuticals, Inc. announced topline results from its Phase 3 REGAIN Study of Eculizumab (Soliris®) in Patients with Refractory Generalized Myasthenia Gravis (gMG). In the study, the primary efficacy endpoint of change from baseline in Myasthenia Gravis Activities of Daily Living Profile (MG-ADL) total score, a patient-reported assessment, at week 26, did not reach statistical significance. However, the first prospectively defined secondary efficacy endpoint of change from baseline in Quantitative Myasthenia Gravis (QMG) total score, a physician-administered assessment of MG clinical severity, did achieve a statistically significant change, as did other secondary endpoints.

While there is disappointment that the primary endpoint of the trial was not achieved, the clinical meaningful improvement in secondary endpoints is encouraging and confirms that eculizumab's mechanism of inhibiting complement activation is a novel treatment strategy for gMG patients. Alexion has confirmed its commitment, in conversations with investors and with MGFA staff immediately after the press release, to move forward with their development plan and to seek approval for eculizumab as a treatment for refractory MG, based on the "totality of the data." Next steps will include a presentation of a more data from the REGAIN study, and discussions with regulatory agencies in the US and Europe (FDA, EMA).

Alexion's press release LINK quoted James F. Howard, Jr., MD, Distinguished Professor of Neuromuscular Disease, Professor of Neurology, Medicine and Allied health and Chief, Neuromuscular Disorders Section, The University of North Carolina School of Medicine, and MGFA M/SAB member as saying "While the REGAIN study missed its primary endpoint, I am encouraged by the clinically meaningful improvement in MD-ADL and QMG measures in patients treated with eculizumab compared with placebo. The magnitude of effect on QMG observed in this large, prospective registration trial is unprecedented in my more than 30 years of clinical investigation of refractory MG patients, and I look forward to presenting additional outcomes at ICNMD [International Congress of Neuromuscular Diseases]." The ICNMD will take place in Toronto and Dr. Howard's presentation, reporting more fully on the REGAIN study results, will be take place on July 7, 2016.

MGFA M/SAB member and former chair, and Chair of the Department of Neurology at the George Washington University School of Medicine and Health Sciences, Henry Kaminski, MD states, "As an investigator in complement, I am encouraged by the positive secondary results. We can hope that in-depth analysis of the results of this trial and other research will lead to better understanding and ultimately approved treatments for refractory gMG."

Participants in the REGAIN study were gMG patients who had not responded to current standard MG therapies, having failed at least two immunosuppressive agents or failed with one immunosuppressive agent and required chronic plasma exchange or IVIG and also had an MG-ADL score of greater than 6 (i.e., their activities of daily living were moderately/severely impacted by their MG). [You can find information on the MG-ADL at this LINK.]

Detailed analysis of the results, as reported, showed that 10 out of 12 endpoints considered in the study were achieved with statistically significant improvements in disease severity. Alexion also reports that 94% (117 of 125) of the REGAIN trial participants continued into an open-label extension study.

During the study, common adverse events included headache, upper respiratory tract infection, nasopharyngitis, MG and nausea without meaningful differences between patients receiving eculizumab and placebo. Four patients discontinued eculizumab because of an adverse event while none discontinued their placebo treatment due to adverse events. Eculizumab has Orphan Drug Designation in the U.S. and Japan for MG, but not for Refractory Generalized Myasthenia Gravis, gMG.

“There is a critical need for therapies addressing refractory gMG which causes immeasurable suffering among those struggling with MG, and their families, as the affected person’s most basic functions are sorely compromised by muscle weakness and fatigue,” said Nancy Law, MGFA CEO. “MGFA is grateful to Alexion, the scientists and clinicians, and most of all to the patients with MG who volunteered to be part of the REGAIN trial. We look forward to learning more as information emerges from further analysis of the data and from the open-label trial extension.”

About The Myasthenia Gravis Foundation of America

Founded in 1952, the Myasthenia Gravis Foundation of America (MGFA) is the only national volunteer health agency dedicated solely to the fight against the debilitating disease, myasthenia gravis. MGFA is committed to finding a cure for myasthenia gravis and closely related diseases, improving treatment options and providing information and support to people with myasthenia gravis through research, education, community programs and advocacy. For more information please visit http://www.myasthenia.org/.