Phase 2 Study of Eculizumab (Soliris®) in Patients with Severe and Refractory Generalized Myasthenia Gravis Presented at MGFA Annual Meeting

Study in 14 Patients Shows Clinically Meaningful Trend in Disease Severity Score

Eculizumab Achieves Strong Disease Improvement Signal and Important Secondary Endpoints

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN) today announced that the Company's study of eculizumab (Soliris®) in a small group of 14 patients with severe and refractory generalized myasthenia gravis, an ultra-rare and debilitating form of generalized myasthenia gravis (gMG), showed a strong disease improvement signal. The exploratory 14-patient study aimed to identify a clinically meaningful benefit of eculizumab in improving Quantitative Myasthenia Gravis disease severity score (QMG score) relative to placebo. The primary endpoint showed a clinically meaningful trend, and important secondary endpoints were achieved with statistical significance. Data from the Phase 2 study were presented today at the annual Scientific Session of the Myasthenia Gravis Foundation of America, Inc. (MGFA) in San Francisco. Alexion is now planning further investigation of eculizumab as a treatment for patients with severe and refractory gMG.

The Phase 2 eculizumab study was a randomized, double-blind, placebo-controlled, cross-over study in 14 patients who had moderate to severe muscle weakness despite treatment with immunosuppressants. In the first treatment period of this cross-over study, which enrolled only 14 patients with severe and refractory gMG, 86% (6/7) of eculizumab-treated patients compared to 57% (4/7) of placebo-treated patients achieved a three-point reduction in their QMG score after 16 weeks of treatment, the primary endpoint of the study. This improvement was achieved more rapidly for eculizumab compared to placebo (p= 0.078). In the lead secondary endpoint, the study achieved its objective of demonstrating a significant clinical benefit of eculizumab in improving QMG score relative to placebo: the overall change in mean QMG total score from baseline to the last visit of the study was significantly improved more than 4 points with eculizumab compared to placebo (-7.92 vs -3.67, respectively; p=0.0144). Examining whether there would be even further differential at higher thresholds of disease improvement, an exploratory analysis of the first treatment period demonstrated that 57% of patients (4/7) treated with eculizumab obtained an eight-point improvement in total QMG score as compared to 14% (1/7) of patients receiving placebo.

"These data highlight the central role of uncontrolled complement activation in severe, refractory generalized myasthenia gravis," said James F. Howard, Jr., M.D., study investigator, Distinguished Professor of Neuromuscular Disease and Chief of Neuromuscular Disorders Section, Department of Neurology, University of North Carolina at Chapel Hill. "By blocking terminal complement, eculizumab represents a potential new treatment approach for patients with severe and refractory gMG who have failed prior therapies."

Severe and refractory gMG is an ultra-rare, debilitating, neurological disorder caused by uncontrolled complement activation due to autoantibodies directed at the neuromuscular junction (NMJ).[1] Eculizumab is a first-in-class terminal complement inhibitor. There is no known cure for myasthenia gravis.[2] Common treatments include medications (anticholinesterase agents, corticosteroids, immunosuppressive agents or cytotoxic therapy), thymectomy (surgical removal of the thymus gland) and plasma exchange.[2,3]

About the Study

Patients were randomly assigned to receive eculizumab in the first treatment period (16 weeks) followed by placebo in the second treatment period (16 weeks), or the reverse treatment sequence, placebo followed by eculizumab, respectively. The first treatment period for all patients was followed by a 5-week washout period.

Following treatment with eculizumab in the first treatment period, QMG scores in eculizumab treated patients did not return to baseline levels at the start of the second treatment period, highlighting the presence of a prolonged carry-over effect of eculizumab treatment on the reduction in total QMG scores. Changes in QMG score with eculizumab, compared to placebo, observed only in the first treatment period, were similar to those reported above for the entire study period (-6.67 vs -3.48, respectively; p=0.058).

Eculizumab appeared well-tolerated in the study with the three most common adverse events being nausea, back pain and headache. One patient in the eculizumab to placebo treatment group experienced two serious adverse events following termination of eculizumab: a myasthenia gravis exacerbation during the washout period, and a myasthenia gravis crisis during the subsequent placebo period.

"This study demonstrates that eculizumab provided a strong clinical signal for meaningful disease improvement in these investigational study patients with severe and refractory generalized myasthenia gravis, an ultra-rare disorder," said Stephen P. Squinto, Ph.D., Executive Vice President, Head of Research and Development at Alexion. "We are encouraged by the results of this Phase 2 study and will evaluate the next steps in pursuing further investigation of eculizumab for patients with this severe ultra-rare disorder."

About Myasthenia Gravis

Myasthenia Gravis is a rare, debilitating, neurological disorder caused by uncontrolled complement activation. The uncontrolled complement activation, resulting from auto-antibodies that recognize a specific target in the nerve-muscle junction, causes tissue damage and interference with signaling between nerve and muscle fibers.[1,4] Patients with myasthenia gravis initially experience weakness in their ocular (eye) muscles, and the disease typically progresses to the more severe and generalized form to include head, spinal, limb and respiratory muscles. Symptoms can include drooping eyelid, blurred vision, slurred speech, difficulty chewing or swallowing, weakness in the arms and legs and difficulty breathing.

About Eculizumab (Soliris®)

Soliris is a first-in-class terminal complement inhibitor developed from the laboratory through regulatory approval and commercialization by Alexion. Soliris has been approved in the U.S., European Union, Japan and other territories as the first treatment for patients with PNH, a debilitating, ultra-rare and life-threatening blood disorder defined by chronic uncontrolled complement activation which causes chronic red blood cell destruction (hemolysis), leading to blood clots, organ failure, and shortened survival. Prior to these approvals, there were no therapies specifically available for the treatment of patients with PNH. Soliris (eculizumab) is not approved for any indication other than PNH. Alexion's breakthrough approach to complement inhibition has received some of the pharmaceutical industry's highest honors: the 2008 Prix Galien USA Award for Best Biotechnology Product with broad implications for future biomedical research and the 2009 Prix Galien France Award in the category of Drugs for Rare Diseases. More information on Soliris is available at www.soliris.net.

Important Safety Information

Soliris is generally well tolerated in patients with PNH. The most frequent adverse events observed in clinical studies of patients with PNH were headache, nasopharyngitis (runny nose), back pain and nausea. Treatment with Soliris should not alter anticoagulant management because the effect of withdrawal of anticoagulant therapy during Soliris treatment has not been established.

The U.S. product label for Soliris also includes a boxed warning: "Soliris increases the risk of meningococcal infections. Meningococcal infection may become rapidly life-threatening or fatal if not recognized and treated early. Vaccinate patients with a meningococcal vaccine at least two weeks prior to receiving the first dose of Soliris; revaccinate according to current medical guidelines for vaccine use. Monitor patients for early signs of meningococcal infections, evaluate immediately if infection is suspected, and treat with antibiotics if necessary." During PNH clinical studies, two out of 196 vaccinated PNH patients treated with Soliris experienced a serious meningococcal infection. Prior to beginning Soliris therapy, all patients and their prescribing physicians are encouraged to enroll in the PNH Registry, which is part of a special risk-management program that involves initial and continuing education and long-term monitoring for detection of new safety findings.

About Alexion

Alexion Pharmaceuticals, Inc. is a biopharmaceutical company focused on serving patients with severe and ultra-rare disorders through the innovation, development and commercialization of life-transforming therapeutic products. Alexion is the global leader in complement inhibition, and has developed and markets Soliris® (eculizumab) as a treatment for patients with PNH, a debilitating, ultra-rare and life-threatening blood disorder. Soliris is currently approved in more than 35 countries for the treatment of PNH. Alexion is evaluating other potential indications for Soliris and is pursuing development of other innovative biotechnology product candidates in early stages of development. This press release and further information about Alexion Pharmaceuticals, Inc. can be found at: www.alexionpharma.com.

Safe Harbor Statement

This news release contains forward-looking statements, including statements related to anticipated clinical development, regulatory and commercial milestones and potential health and medical benefits of Soliris® (eculizumab) for the potential treatment of patients with severe and refractory generalized myasthenia gravis. Forward-looking statements are subject to factors that may cause Alexion's results and plans to differ from those expected, including for example, decisions of regulatory authorities regarding marketing approval or material limitations on the marketing of Soliris for its current or potential new indications, and a variety of other risks set forth from time to time in Alexion's filings with the Securities and Exchange Commission, including but not limited to the risks discussed in Alexion's Quarterly Report on Form 10-Q for the period ended June 30, 2011, and in Alexion's other filings with the Securities and Exchange Commission. Alexion does not intend to update

any of these forward-looking statements to reflect events or circumstances after the date hereof, except when a duty arises under law.

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