Alexion Completes Enrollment in All Four Clinical Trials of Soliris(R) (eculizumab) in Patients with Atypical Hemolytic Uremic Syndrome (aHUS)

Trials Include Adult and Adolescent Patients with aHUS

CHESHIRE, Conn., Apr 20, 2010 (BUSINESS WIRE) -- Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN) today announced that it has completed enrolling patients in all four previously announced prospective, open-label clinical studies investigating Soliris (R) (eculizumab) as a potential treatment for patients with atypical Hemolytic Uremic Syndrome (aHUS) being conducted in North America and multiple European countries. The four studies have achieved the Company's enrollment targets, with a total of approximately 35 adult and adolescent patients with aHUS.

About the Studies

All patients in the studies had evidence of thrombotic microangiopathy prior to enrollment. The studies include patients who had been treated chronically with plasma therapy and others who were resistant to plasma therapy. For patients who have been treated chronically with plasma therapy, patients were screened for an initial 8 week period prior to initiating treatment with Soliris. Once enrolled, patients in the trials are treated with Soliris for a period of 26 weeks. All patients have now commenced treatment.

"Given the potential for rapid and life-threatening deterioration observed in patients with aHUS, and the inability to predict sudden worsening in an individual, we are increasingly focused on diligently advancing our development efforts. We look forward to presenting preliminary results from these studies later in 2010," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "In addition, because a significant number of patients with aHUS are children, we are committed to expanding our aHUS program to include studies of Soliris in pediatric patients with aHUS."

Alexion is working with the regulatory authorities in the U.S. and European Union to finalize protocols for studies of Soliris in patients younger than 12 years of age. The Company expects to begin a trial in pediatric aHUS patients approximately mid-year.

About Soliris

Soliris is not approved for the treatment of patients with aHUS and is being provided to patients in clinical studies on an investigational basis. Soliris is a first-in-class terminal complement inhibitor developed from the laboratory through regulatory approval by Alexion. Soliris has been approved in the U.S., European Union and other countries as the first treatment for patients with paroxysmal nocturnal hemoglobinuria (PNH), an ultra-rare, debilitating and life-threatening blood disorder defined by chronic hemolysis, or the destruction of red blood cells. Prior to these approvals, there were no therapies specifically available for the treatment of patients with PNH. Alexion's innovative 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.

About aHUS

Atypical hemolytic-uremic syndrome (aHUS) is a rare and severe genetic disorder characterized by sudden clinical deterioration - life-threatening blood clots throughout the body leading to renal failure, thrombocytopenia, and hemolytic anemia. These clinical abnormalities are the hallmark of thrombotic microangiopathy.

Patients with aHUS experience poor outcomes, including dialysis, kidney failure and death, often occurring within the first year following diagnosis. As in PNH, aHUS is caused by a deficiency in normally occurring complement inhibitor proteins. Typically, patients with aHUS have genetic mutations in one of several complement inhibitor proteins that lead to uncontrolled complement activation. Excessive complement activation may contribute to severe inflammation of the blood vessels and blood clotting through the activation of white blood cells, platelets, and the endothelial cell lining of blood vessels. (1)

The prognosis for patients with aHUS is generally poor. Approximately 70 percent of patients with the most common mutation experience renal failure, dialysis, or death within one year of the first clinical episode. (2, 3) Following kidney transplantation, recurrent aHUS causes kidney failure in up to 60 to 90 percent of patients. (4)

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 working to develop and deliver life-changing drug therapies for patients with serious and life-threatening medical conditions. Alexion is engaged in the discovery, development and commercialization of therapeutic products aimed at treating patients with a wide array of severe disease states, including hematologic and kidney diseases, transplant, other inflammatory disorders, and cancer. Soliris is Alexion's first marketed product. Alexion is evaluating other potential indications for Soliris as well as other formulations of eculizumab for additional clinical indications, and is pursuing development of other antibody product candidates in early stages of development. This press release and further information about Alexion Pharmaceuticals, Inc. can be found at: www.alexionpharma.com.

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Safe Harbor Statement

This news release contains forward-looking statements, including statements related to anticipated clinical development milestones and potential health and medical benefits of Soliris (eculizumab) for the potential treatment of patients with atypical hemolytic uremic syndrome (aHUS). 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, delays in arranging satisfactory manufacturing capability and establishing commercial infrastructure, the possibility that results of published reports or clinical trials are not predictive of safety and efficacy results of Soliris in broader patient populations, the risk that clinical trials may not be completed successfully, the possibility that initial results of commercialization are not predictive of future rates of adoption of Soliris, the risk that third parties won't agree to license any necessary intellectual property to Alexion on reasonable terms or at all, the risk that third party payors will not reimburse for the use of Soliris at acceptable rates or at all, 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 Annual Report on Form 10-K for the period ended December 31, 2009, 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.

References

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