



Soliris(R) Reduced Measures of Thrombosis and Inflammation, and Decreased Indicators of Pulmonary Hypertension, in Studies of Patients with PNH

New Data Presented at ASH Annual Meeting Concerning Soliris and Important Medical Complications of PNH

CHESHIRE, Conn., Dec 09, 2008 /PRNewswire-FirstCall via COMTEX News Network/ --

Soliris(R) (eculizumab), a terminal complement inhibitor developed by Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN), was observed by investigators to reduce blood measures associated with undiagnosed blood clots and inflammation in patients with PNH.

A separate study found that Soliris was observed to reduce indicators of pulmonary artery hypertension (PAH) in patients with paroxysmal nocturnal hemoglobinuria (PNH), according to a new analysis of clinical trial data. Both sets of data were presented in oral sessions yesterday at the 50th Annual Meeting of the American Society of Hematology.

"The common, severe and progressive clinical consequences of PNH are becoming more apparent as researchers gain more experience and basic knowledge with regard to this disease," said Leonard Bell, M.D., Chief Executive Officer of Alexion.

Blood Markers of Thrombin Generation and Inflammation

Research titled "Eculizumab Therapy Results in Rapid and Sustained Decreases in Markers of Thrombin Generation and Inflammation in Patients with PNH" was presented yesterday in an oral session at the ASH annual meeting by Ilene Ceil Weitz, M.D., Assistant Clinical Professor of Medicine, Jane Anne Nohl Division of Hematology, Keck School of Medicine of the University of Southern California.

Recently published research showed that patients with PNH were observed to have 92 percent fewer blood clots (thromboses) during treatment with Soliris (1) compared to the period of time prior to Soliris treatment. To better understand the mechanism for this observed reduction, researchers used highly sensitive laboratory tests to track levels of blood markers in order to determine the effect of Soliris on markers of thrombin generation and inflammation among eight patients with PNH, only one of which had been previously diagnosed with a blood clot.

Results showed that prior to treatment with Soliris, patients with PNH exhibited a hypercoagulable state as indicated by elevated levels of key inflammatory and pro-thrombotic measures. Soliris treatment was associated with statistically significant decreases in key blood measures, including LDH levels ($p=0.0001$), D-dimers ($p=0.0057$), thrombin-antithrombin complex or TAT (0.01), interleukin 6 or II-6 ($p=0.04$), and tissue factor microparticles or TFMP (0.02) during the four-week induction phase of treatment. All decreases in D-dimers, TAT, II-6, TFMP, and LDH were sustained in the maintenance phase of treatment.

The authors concluded that the study patients, most of whom did not have clinical evidence of thrombosis and were also not previously transfused, exhibited a hypercoagulable state. In these patients, Soliris treatment was observed to result in a decrease in measures of thrombin generation and inflammation. These changes appeared to be independent of the observed reduction in hemolysis.

"This data deepens our understanding of the complex interactions in the blood that result in dangerous inflammation and blood clots in patients with PNH," said Dr. Weitz. "It also suggests that many patients with PNH, even without a clinical thrombosis, exhibit a high risk for blood clotting, and provides hope for patients and physicians, since thrombosis is the leading cause of premature death in PNH and the most feared complication of the disease."

Pulmonary Hypertension

Research titled "Eculizumab Reduces Pulmonary Hypertension through Inhibition of Hemolysis-Associated Nitric Oxide Consumption in Patients with Paroxysmal Nocturnal Hemoglobinuria" was presented in an oral session yesterday at the ASH annual meeting by Anita Hill, M.D., of the Department of Haematology, Bradford Royal Infirmary, Bradford, United Kingdom.

Using data from the Phase III TRIUMPH study of Soliris, Dr. Hill and her colleagues evaluated the efficacy of Soliris in the regulation of cell-free plasma hemoglobin levels, nitric oxide depletion and subsequent cardiovascular morbidities in patients with PNH. This analysis found that 47 percent of patients with PNH (34 of 73) suffered from pulmonary hypertension before

starting the trial. In this study, PAH was measured by an elevated blood level of NT-proBNP, which has been shown to be highly predictive of PAH and an independent predictor of mortality in other hemolytic diseases. (2) At the start of the study, levels of hemolysis and nitric oxide consumption were shown to be much greater in PNH (more than 6- and 10-fold, respectively) than in patients with other hemolytic diseases.

Patients treated with Soliris experienced a 50 percent reduction in the incidence of PAH over the course of the 26-week treatment period, from 52.5 percent to 26.3 percent, while PAH did not change with placebo (39.4% to 43.8%) ($P < 0.001$). Additionally, Soliris-treated PNH patients experienced significantly improved shortness of breath compared to placebo, as measured by the EORTC QLQ-C30 quality of life survey ($P < 0.001$).

"A careful analysis of blood levels in PNH patients shows that hemolysis, the red blood cell destruction that defines the disease, consumes nitric oxide in the blood which is likely to result in an increase in cardiovascular complications as shown in other hemolytic diseases," said Dr. Hill. "This study confirms that PAH is common in hemolytic PNH patients and also suggests that the anti-hemolytic effect of Soliris treatment significantly increases nitric oxide and consequently reduces pulmonary hypertension in patients with PNH."

Pulmonary artery hypertension is a rare, progressive disorder characterized by high blood pressure (hypertension) of the pulmonary arteries, the blood vessels that carry blood from the heart to the lungs. Symptoms of pulmonary hypertension can be severe and include shortness of breath (dyspnea), chest pain, fatigue, and fainting episodes.

Additional Data

A poster titled "Modification of the Eculizumab Dose to Successfully Manage Intravascular Breakthrough Hemolysis in Patients with Paroxysmal Nocturnal Hemoglobinuria" was presented yesterday at the ASH annual meeting by Richard Kelly, M.D., of St. James's University Hospital, Leeds, United Kingdom. The authors concluded that two alternative-dosing regimens of Soliris were well tolerated and could be employed in the small percentage of patients with PNH in whom complement inhibition is not consistently maintained using the standard dose. For details, visit: <http://ash.confex.com/ash/2008/webprogram/Paper8331.html>.

A poster titled "Reducing Intravascular Hemolysis on Ferritin Homeostasis in Eculizumab Treated Paroxysmal Nocturnal Hemoglobinuria (PNH) Patients" was presented yesterday at the ASH annual meeting by Alexander Roeth, M.D. of the Department of Hematology, University Hospital Essen in Essen, Germany. The authors concluded that eculizumab was safe and well tolerated in the study patients, and that iron parameters in PNH patients treated with eculizumab should be monitored to determine if iron supplementation should be altered or iron depletion therapy should be considered. For details, visit: <http://ash.confex.com/ash/2008/webprogram/Paper11166.html>.

About PNH

PNH is a rare blood disorder that affects an estimated 8,000 to 10,000 people in North America and Europe and, using similar prevalence estimates, potentially 1,000 - 2,000 patients in Japan. (3) PNH strikes people of all ages, with an average age of onset in the early 30s. (4) Approximately 10 percent of all patients first develop symptoms at 21 years of age or younger. (5) PNH develops without warning and can occur in men and women of all races, backgrounds and ages. PNH often goes unrecognized, with delays in diagnosis ranging from one to more than 10 years. (6) The estimated median survival for PNH patients is between 10 and 15 years from the time of diagnosis. (4,6)

PNH has been identified more commonly among patients with disorders of the bone marrow, including aplastic anemia (AA) and myelodysplastic syndromes (MDS). (7, 8, 9). In patients with thrombosis of unknown origin, PNH may be an underlying cause. (5,10)

Prior to approval of Soliris, there were no therapies specifically available for the treatment of PNH. PNH treatment was limited to symptom management through periodic blood transfusions, non-specific immunosuppressive therapy and, infrequently, bone marrow transplantations -- a procedure that carries considerable mortality risk. (5,10)

About Soliris

Soliris was approved in March 2007 by the U.S. Food and Drug Administration (FDA) as the first treatment for PNH, a rare, debilitating and life-threatening blood disorder defined by hemolysis, or the destruction of red blood cells. In June 2007, the European Commission (EC) also approved the use of Soliris for the treatment of patients with PNH. Soliris is the first therapy approved in Europe for the treatment of PNH and was the first medicinal product to receive EC approval under the EMEA Accelerated Assessment Procedure.

Important Safety Information

Soliris is generally well tolerated. The most frequent adverse events observed in clinical studies were headache, nasopharyngitis (a 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. 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 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 enrolled in the Soliris Safety 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.

Please see full prescribing information at www.soliris.net.

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 diseases, cancer, and autoimmune disorders. In March 2007, the FDA granted marketing approval for Alexion's first product, Soliris, for all patients with PNH, and Alexion began commercial sale of Soliris in the U.S. during April 2007. In June 2007, the EC granted marketing approval for Soliris in the European Union for all patients with PNH. 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.alexionpharm.com.

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Safe Harbor Statement

This news release contains forward-looking statements, including statements related to potential health and medical benefits from Soliris. 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, delays in arranging satisfactory manufacturing capability and establishing commercial infrastructure, delays in developing or adverse changes in commercial relationships, the possibility that results of clinical trials are not predictive of safety and efficacy results of Soliris in broader patient populations, 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, the risk that estimates regarding the number of PNH patients are inaccurate, the risk that pending litigation may be resolved adversely, 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 September 30, 2008 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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