Soliris(R) Reduced Hemolysis, Decreased Transfusion Requirements and Improved Fatigue in Patients with PNH and Bone Marrow Insufficiency Disorders

Adding Soliris to Immunosuppressive Therapy Provided Clinical Benefit to Patients with Concomitant PNH and Aplastic Anemia in Study Presented at ASH Annual Meeting
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CHESHIRE, Conn., Dec 06, 2009 (BUSINESS WIRE) -- Soliris(R) (eculizumab), a first-in-class terminal complement inhibitor developed by Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN), reduced hemolysis (destruction of red blood cells) and transfusion requirements, and improved measures of fatigue, when added to ongoing immunosuppressive therapy (IST) in patients with both paroxysmal nocturnal hemoglobinuria (PNH) and bone marrow insufficiency (BMI), including aplastic anemia (AA).

The data were presented today at the 51st Annual Meeting of the American Society of Hematology in New Orleans in a poster session titled, "Effects of Eculizumab Therapy in Patients with Paroxysmal Nocturnal Hemoglobinuria (PNH) Receiving Concurrent Immunosuppressive Therapy for Bone Marrow Insufficiency."

In the study, patients with concomitant BMI and PNH treated with IST, prior to addition of Soliris, continued to suffer increased hemolysis and fatigue, contributing to PNH-related morbidities. In addition, the study showed that patients with BMI and PNH, in which Soliris treatment was added to ongoing immunosuppressive therapy, experienced a substantial reduction in hemolysis and fatigue, and subsequent morbidities.

"PNH and bone marrow insufficiency diseases like aplastic anemia have distinctive disease morbidities and mortality and require specific treatments," said Hubert Schrezenmeier, M.D., lead author of the study and Professor of Medicine at the Institute of Transfusion Medicine at the University of Ulm in Germany. "This clinical analysis showed patients already receiving immunosuppressive therapy can be safely treated with eculizumab and experience the expected sharp reduction in hemolysis, the underlying cause of PNH-related morbidities."

"In many patients with PNH, physicians must also manage aplastic anemia or other bone marrow insufficiencies," said Stephen Squinto, Ph.D., Executive Vice President and Head of Research and Development at Alexion. "The results presented today demonstrate the need to identify and treat each of these conditions effectively and at the same time in order to provide patients with optimal care."

Soliris is the first therapy approved for the treatment of patients with PNH, a rare, debilitating and life-threatening blood disorder, to reduce hemolysis. In patients with PNH, hemolysis can cause thromboses, kidney disease, liver dysfunction, disabling fatigue, impaired quality of life, recurrent pain, shortness of breath, pulmonary hypertension, intermittent episodes of dark-colored urine (hemoglobinuria), and anemia.

Clinical Data

Researchers conducted a post-hoc analysis of data in the Soliris PNH controlled clinical trials database to evaluate the safety and efficacy of Soliris in a population of patients receiving concomitant IST. The database included a total of 195 patients, of which 17 were receiving IST.

Patients were subdivided into two treatment groups - those patients who commenced Soliris treatment during ongoing IST and patients who commenced IST during ongoing Soliris treatment. The clinical endpoints measured included lactase dehydrogenase (LDH) level as a measurement of hemolysis, number of transfusions, hemoglobin (Hgb) level, and Functional Assessment of Chronic Illness Therapy-Fatigue (FACIT-Fatigue) Score. Patients were followed and results were reported for months prior to and during Soliris treatment.

Twelve patients (11 with AA; 1 pancytopenic) were treated with the immunosuppressive treatment cyclosporine A (CSA) from 9.8 months to 13 years before Soliris treatment. Despite treatment with CSA and prior to introduction of Soliris, patients in this group continued to experience PNH related morbidities as measured by increased hemolysis (measured by LDH; mean 1,368 U/L), severe fatigue (mean FACIT-Fatigue Score of 26.9) and substantial transfusion requirements (mean 17 U/year per patient) at 3 months prior to Soliris.

Commencement of Soliris treatment in patients also treated with ongoing IST was associated with a significant reduction in hemolysis (from a mean LDH of 1,368 U/L at 3 months prior to Soliris treatment to LDH of 389 U/L at 3 months following initiation

of Soliris treatment and maintained at 379 U/L for at least 1 year following initiation of Soliris treatment; p=0.002 at 3 and 12 months respectively). The reduction in hemolysis associated with Soliris treatment also reduced transfusion requirements (from a mean of 17 transfusion units/year at 12 months prior to Soliris treatment to a mean of 6 transfusion units/year at 12 months following initiation of eculizumab treatment; p=0.02) and maintained hemoglobin levels (mean of 9.2 Hgb g/dL at 12 months prior to Soliris treatment to 9.1 Hgb g/dL at 12 months following initiation of Soliris treatment). Soliris treatment also significantly improved fatigue scores (from a mean FACIT-Fatigue Score of 26.8 at 12 months prior to Soliris treatment to a mean FACIT-Fatigue Score of 36.3 following initiation of Soliris treatment; p=0.02) despite the stabilization of hemoglobin and reduction in transfusions. The overall and infection-related adverse event rates and serious adverse event rates were similar to the overall clinical trial population and did not increase over time.

About PNH

PNH is an ultra-rare blood disorder that strikes people of all ages, with an average age of onset in the early 30s. (1) Patients with PNH suffer from hemolysis (red blood cell destruction) which leads to thromboses (blood clots), disabling fatigue, anemia, impaired quality of life, pulmonary hypertension, shortness of breath, recurrent pain, kidney disease and intermittent episodes of dark-colored urine (hemoglobinuria). (2,3) Approximately 10 percent of all patients first develop symptoms at 21 years of age or younger. (2) 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. (4) It is estimated that approximately one-third of patients with PNH do not survive more than five years from the time of diagnosis. (4) PNH has been identified more commonly among patients with disorders of the bone marrow, including aplastic anemia (AA) and myelodysplastic syndromes (MDS). (5,6,7) In patients with thrombosis of unknown origin, PNH may be an underlying cause. (1) More information on PNH is available at www.pnhsource.com.

About Soliris

Soliris has been approved by the U.S. Food and Drug Administration (March 2007), the European Commission (June 2007), Health Canada (January 2009) and Australia's Therapeutic Goods Administration (February 2009) as the first treatment for all patients with 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 PNH. Soliris is not expected to affect the aplastic component of anemia in patients with PNH.

More information on Soliris is available at www.soliris.net.

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. 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 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, cancer, and autoimmune disorders. 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

This news release contains forward-looking statements, including statements related to potential health and medical benefits

from Soliris (eculizumab). 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 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 Quarterly Report on Form 10-Q for the period ended September 30, 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.

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