PNH Patients With Lower Levels of Hemolysis, Mild Anemia and Minimal Transfusion Support Have Significant Disease Burden; Soliris® Therapy Provided Clinical Improvements in PNH Patients Regardless of Disease Severity

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Soliris(R) (eculizumab) therapy reduced hemolysis, fatigue, thromboses (blood clots) and transfusion requirements in patients with a rare blood disorder called paroxysmal nocturnal hemoglobinuria (PNH), including those who might have been expected to have less severe disease, according to data from an ongoing open-label clinical study presented today at the 49th Annual Meeting of the American Society of Hematology Meeting in Atlanta.

The data were highlighted in an oral presentation titled, "High Incidence of Progression to Significant Disease Burden in Paroxysmal Nocturnal Hemoglobinuria Patients with Lower Levels of Hemolysis, Mild Anemia and Minimal Transfusion: Clinical Improvement with Eculizumab Therapy."

In the study, (1) Soliris was associated with significant long-term clinical improvements in patients with PNH, regardless of baseline degree of hemolysis, anemia or transfusion requirements. The study also demonstrated that patients who might have been expected to have less severe disease, considering their baseline clinical characteristics, suffered from significant disease burden.

"PNH patients once thought to have less severe disease based on their clinical characteristics actually face significant disease burden from anemia, fatigue, impaired quality of life, blood transfusion requirements and blood clot risk," said Monica Bessler, MD, PhD, lead author of the study and Professor of Medicine, Professor of Pharmacology and Molecular Biology, Washington University in St. Louis School of Medicine. "The results presented today show that, in this study group, regardless of disease severity, long- term Soliris treatment provides important clinical improvements in their disease signs, symptoms and complications."

"We continue to observe that the PNH patient population includes a wide range of patients with a broad clinical profile," said Leonard Bell, MD, Chief Executive Officer of Alexion Pharmaceuticals. "The results presented today provide further evidence for the utility of Soliris therapy in patients with diverse manifestations of PNH. We remain committed to our goal that all patients who can benefit from Soliris will have access to it."

Soliris, developed by Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN), is the first therapy approved for the treatment of patients with PNH, a rare, debilitating and life-threatening blood disorder defined by the destruction of red blood cells, or hemolysis. Soliris is a complement inhibitor indicated for the treatment of patients with PNH 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. (2-4)

Clinical Data

In the ongoing open-label clinical study, investigators examined the long- term clinical effect of Soliris in patient subgroups, including those with lower levels of hemolysis, mild anemia and minimal transfusion requirements.

Data were analyzed by levels of baseline hemolysis (as indicated by baseline quartiles of lactate dehydrogenase, LDH), anemia (Hgb < 10.5 g/L vs greater than or equal to 10.5 g/L), and transfusion requirements (transfusion episodes in prior year greater than or equal to 1 vs 0 episodes). Hemolysis, fatigue and transfusion requirements were examined using a ranking test to measure efficacy during both the first and the most recent six months of Soliris therapy; patients received a median of 22 months of treatment.

Hemolysis was significantly reduced with eculizumab treatment in patients with all levels of baseline hemolysis. In patients with the lowest quartile of hemolysis (< 1490 U/L), LDH was reduced from 1077 +/- 42 U/L pre-eculizumab to 323 +/- 22 U/L during the first six months and to 347 +/- 47 U/L during the most recent six months of Soliris therapy (P<0.001 for each comparison to baseline). Hemolysis was also significantly reduced during both the first and most recent six months of Soliris therapy in patients with mild anemia (Hgb of greater than or equal to 10.5 g/L) and minimal transfusion support prior to treatment (0 or 1 transfusion episode in the previous year) (P<0.001 for all subgroups at both time intervals).

In the study, patients who might have been considered to have less severe disease were found to have an elevated risk of blood clots prior to therapy and experienced the following reductions in the incidence of blood clots during Soliris therapy: for patients with the lowest quartile of baseline hemolysis, the rate decreased from 10.8 to 4.5 events per 100 patient
years ($P=0.009$, $n=48$), for those with mild anemia from 5.2 to 0.8 events per 100 patient years ($P<0.001$, $n=55$), and for those with minimal pretreatment transfusion support from 4.9 to 0.0 events per 100 patient years ($P=0.063$, $n=22$).

Soliris treatment was associated with significant improvements in fatigue (as measured by the FACIT-Fatigue instrument), regardless of the baseline level of hemolysis, anemia, or pretreatment transfusion requirements. At baseline, patients who had the lowest levels of hemolysis, mild anemia, or minimal transfusion requirements reported fatigue. With patients in the lowest quartile of baseline hemolysis, FACIT-Fatigue scores improved by 6 points during the first six months and by 8 points during the most recent six months of Soliris therapy ($P<0.001$ for each comparison to baseline); an increase of 3 or more points is considered clinically meaningful in this instrument. (5) In patients with mild baseline anemia, fatigue scores improved by 5 points during the first six months and by 6 points during the most recent six months of therapy ($P<0.001$ for each comparison to baseline). Even in patients with minimal transfusion support prior to Soliris therapy, fatigue scores improved by 9 points during the first six months and by 15.5 points during the most recent six months of treatment ($P<0.001$ for each comparison to baseline).

In addition, in patients with history of bone marrow failure, units transfused were reduced from mean 9.2 +/- 1.2 units per patient prior to treatment to 4.4 +/- 1.4 units per patient during the most recent six months of Soliris treatment ($P<0.001$).

About PNH

PNH is an acquired genetic blood disorder defined by hemolysis, in which patients' red blood cells are destroyed by complement, a component of the body's immune system. PNH is a rare disease that affects an estimated 8,000 to 10,000 people in North America and Europe. (6) PNH often strikes people in the prime of their lives, with an average age of onset in the early 30’s. (7) Approximately ten percent of all patients first develop symptoms at 21 years of age or younger. (4) 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 often ranging from one to more than 10 years. (3) The estimated median survival for PNH patients is between 10 and 15 years from the time of diagnosis. (3,7)

PNH has been identified more commonly among patients with disorders of the bone marrow, including aplastic anemia (AA) and myelodysplastic syndrome (MDS). (8,9,10,11) In patients with thrombosis of unknown origin, PNH may be an underlying cause. (4,12)

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 transplantsations -- a procedure that carries considerable mortality risk. (4,12)

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 us 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 Soliris will not generate interest among physicians, 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, 2007 and in our 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.


