



Soliris(TM) Effective in PNH Patients With History of Aplastic Anemia and Myelodysplastic Syndromes

- Analysis of Phase III Data Presented at ASCO - - Alexion Expands EXPLORE Study to Evaluate PNH in Patients with Bone Marrow Disorders -

CHICAGO, June 4, 2007 /PRNewswire-FirstCall via COMTEX News Network/ -- Investigators reported today that Soliris(TM) (eculizumab), a new treatment for paroxysmal nocturnal hemoglobinuria (PNH) developed by Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN), is effective in patients diagnosed with PNH who have a history of aplastic anemia (AA) or myelodysplastic syndromes (MDS). Soliris significantly reduced red blood cell destruction (hemolysis), decreased or eliminated blood transfusion requirements, improved fatigue and quality-of-life outcomes, and reduced thrombotic events in PNH patients who have a history of either one of these bone marrow disorders, according to an analysis of Phase III data presented today at the 43rd annual meeting of the American Society of Clinical Oncology (ASCO).

"A growing body of evidence shows that all PNH patients have the potential to realize meaningful improvement with Soliris, regardless of symptoms, history of bone marrow failure, or transfusion needs prior to treatment," said Leonard Bell, MD, chief executive officer of Alexion Pharmaceuticals. "We believe these data will increase awareness of PNH among oncologists who specialize in bone marrow disorders and give them confidence to treat PNH patients with Soliris."

Alexion also announced today the expansion of an ongoing study to examine the frequency of PNH in patients with AA, MDS and other bone marrow disorders. Alexion now plans to enroll up to 10,000 patients in the EXPLORE trial (EXamination of PNH, by Level Of CD59 on Red and white blood cells, in bone marrow failure syndromes). Since beginning the EXPLORE trial in June 2006 with an initial target of 2,000 patients, Alexion has enrolled 1,500 patients.

"People with bone marrow disorders like AA and MDS are more likely to suffer from PNH," said Dr. Hugo Castro-Malaspina, Division of Bone Marrow Transplant and Hematology Services at Memorial Sloan-Kettering Cancer Center. "The EXPLORE study will help hematologists-oncologists better understand the prevalence and clinical characteristics of PNH in patients with bone marrow disorders. Many of these patients may benefit from treatment with Soliris. Data from EXPLORE may also help us define and implement screening protocols for PNH."

PNH is a rare, disabling and life-threatening blood disorder defined by chronic red blood cell destruction, or hemolysis. Hemolysis can cause one or more of the following symptoms in patients with PNH: severe anemia, disabling fatigue, recurrent pain, shortness of breath, pulmonary hypertension, intermittent episodes of dark colored urine (hemoglobinuria), kidney disease, impaired quality of life and blood clots (thromboses).(1)(2)

PNH has been identified more commonly among patients with disorders of the bone marrow, including AA and MDS.(3)(4)(5)(6) Underlying bone marrow dysfunction can complicate the clinical course of PNH, contributing to anemia, a greater dependence on transfusion support, an increased risk of infection and life-threatening hemorrhage.(7) AA occurs when the bone marrow stops making enough blood-forming stem cells. MDS occurs when the bone marrow and stem cells malfunction, resulting in the production of too many defective blood cells and not enough normal blood cells.

Data Presented at ASCO

Researchers conducted an analysis of data from the Phase III TRIUMPH and SHEPHERD clinical trials to evaluate the safety and efficacy of Soliris treatment over 26 weeks in 39 patients who were diagnosed with PNH and who had a history of AA or MDS. Among these patients, hemolysis was reduced by 84 percent, compared with an 85 percent reduction among patients without a history of AA or MDS. Anemia was also significantly reduced in these patients, as transfusions of packed red blood cells (RBCs) were reduced from a median of seven units/patient prior to treatment to zero units/patient while undergoing therapy with Soliris ($p < 0.001$). Patients with a history of AA or MDS also reported a noticeable improvement in fatigue and overall quality-of-life, similar to the clinical improvements reported by the overall PNH patient population in the TRIUMPH and SHEPHERD studies. Patients with a history of AA or MDS had an elevated rate of thromboembolic events prior to entering the studies (11.13 events per 100 patient-years; $n = 59$) and the rate with Soliris treatment was 1.04 events per 100 patient-years ($P < 0.001$). Soliris appeared to be safe and well-tolerated when administered to PNH patients with a history of AA or MDS, similar to results in the overall population in the TRIUMPH and SHEPHERD studies.(8)

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 affects an estimated 8,000 to 10,000 people in North America and Europe.(9) PNH often strikes people in the prime of their lives, with an average age of onset in the early 30's.(10) Ten 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 often ranging from one to more than 10 years.(7) The estimated median survival for PNH patients is between 10 and 15 years from the time of diagnosis.(5)(11)

About Soliris

Soliris is the first therapy approved by the U.S. Food and Drug Administration for the treatment of PNH. Soliris is indicated for the treatment of patients with PNH to reduce hemolysis. In April the Committee for Human Medicinal Products (CHMP) of the European Medicines Agency (EMA) adopted a positive opinion recommending marketing authorization for Soliris for the treatment of all patients with PNH. Final EU approval is expected in June or July 2007.

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.(2)(12)

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.(13) Treatment with Soliris should not alter anticoagulant management because the effect of withdrawal of anticoagulant therapy during Soliris treatment has not been established.

The product label for Soliris also includes a boxed warning: "Soliris increases the risk of meningococcal infections. Vaccinate patients with a meningococcal vaccine at least 2 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." 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 <http://www.soliris.net>.

About Alexion

Alexion Pharmaceuticals is a biotechnology company working to develop and deliver life-changing drug therapies for patients with serious and life-threatening medical conditions. Alexion's lead product, Soliris(TM) (eculizumab), is indicated for the treatment of paroxysmal nocturnal hemoglobinuria (PNH) to reduce hemolysis. Alexion is engaged in the discovery and development of therapeutic products aimed at treating patients with severe disease states, including hematologic diseases, cancer and autoimmune disorders, and in May 2007, received a corporate leadership award from the National Organization of Rare Disorders (NORD) for the development of Soliris. Alexion applied for marketing authorization with the European Medicines Evaluation Agency (EMA) for Soliris in September 2006, and in April 2007 the Committee for Human Medicinal Products (CHMP) of the EMA adopted a positive opinion recommending marketing authorization for Soliris for the treatment of PNH. This press release and further information about Alexion Pharmaceuticals, Inc. can be found at: <http://www.alexionpharm.com>.

This news release contains forward-looking statements, including statements related to potential benefits and commercial potential for Soliris, timing for, and potential regulatory decisions with respect to, the marketing applications for Soliris in Europe, and interest and excitement about Soliris in the physician community. Forward-looking statements are subject to factors that may cause Alexion's results and plans to differ from those expected, including for example, requests by regulatory authorities for additional information or data after their review of our submissions, the need for additional research and testing, decision of regulatory authorities not to approve (or to materially limit) marketing of Soliris in Europe or other territories, 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 risk that third parties won't agree to license any necessary intellectual property to us on reasonable terms, 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 March 31, 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.

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