Alexion Reports Positive Results from AEGIS Registration Study of Soliris in Japanese Patients With PNH

Primary Endpoint of Reduction of Hemolysis Achieved; Secondary Endpoints Including Fatigue, Anemia, Quality of Life Achieved; Results Consistent With Previous Long-Term Phase III Trials
Data Presented at the American Society of Hematology (ASH) Annual Meeting

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Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN) today reported positive results from AEGIS, an open-label registration study examining Soliris(R) (eculizumab) treatment of Japanese patients with paroxysmal nocturnal hemoglobinuria (PNH). The pre-specified primary efficacy endpoint of change in hemolysis was achieved with an 86 percent reduction (P<0.001). Key secondary endpoints including fatigue (P<0.001) and transfusions/anemia (P<0.001) were also achieved.

Top-line results from AEGIS were presented today at the 50th Annual Meeting of the American Society of Hematology in San Francisco in a poster session titled, "Safety and Efficacy of the Terminal Complement Inhibitor Eculizumab in Japanese Patients with Paroxysmal Nocturnal Hemoglobinuria: AEGIS Phase II Clinical Study Results." The study was conducted to evaluate the safety and efficacy of Soliris in Japanese patients with PNH relative to the previously reported SHEPHERD and TRIUMPH (1,2) Phase III Soliris trials, which were conducted in the United States, Europe and Australia.

"These results show clinical improvements from eculizumab for Japanese patients with PNH, consistent with those observed in the clinical trials conducted in the United States and Europe," said Yuzuru Kanakura, M.D., Ph.D., Professor of Hematology and Oncology at Osaka University Hospital in Suita, Japan, and lead author of the study. "Consistent with the results from the SHEPHERD and TRIUMPH trials, these patients experienced significant reductions in hemolysis, anemia, transfusion dependence, and fatigue. Perhaps most noteworthy was the improvement in kidney function observed with eculizumab treatment. These are important and potentially life-changing improvements for patients with PNH."

Clinical Data

In the AEGIS open-label clinical study, investigators examined the safety and efficacy of Soliris in Japanese patients with PNH relative to the SHEPHERD and TRIUMPH studies. The primary endpoints of the study were reduction of hemolysis and safety following 12 weeks of Soliris treatment. The AEGIS study included 29 patients at nine institutions with minimal transfusion requirements (one or more transfusion episodes in the preceding two years) and permitted enrollment of patients with significant thrombocytopenia (platelet counts greater than or equal to 30x10(9)/L). Forty-five percent of the AEGIS patients had a history of aplastic anemia or myelodysplastic syndromes. Patients were dosed in accordance with the Soliris product labels approved in the United States and European Union in 2007.

Key clinical efficacy data from the AEGIS trial presented at the ASH meeting included:

- Hemolysis, the primary trial endpoint, was rapidly and significantly reduced with Soliris treatment. A standard measure of hemolysis, lactate dehydrogenase (LDH), decreased 86 percent from a median of 1,814 U/L at baseline to a median of 244 U/L following 12 weeks of treatment (P<0.001; normal range 103-223 U/L).
- Control of hemolysis resulted in an improvement in anemia as indicated by a reduction in packed red blood cell (PRBC) transfusion requirements. Transfusions were reduced 71 percent from a mean (SE) of 5.2 (+/-1.04) PRBC units/patient during the 12-week pre-treatment period to 1.5 (+/-0.67) units/patient following 12 weeks of Soliris treatment (P<0.001 for the pre-specified median change). Transfusion independence was achieved in 67 percent (14/21) of patients who were transfusion dependent prior to treatment (P<0.001).
- The improvement in anemia with Soliris treatment was also indicated by increased hemoglobin levels. Hemoglobin levels increased from a median of 7.6 g/dL (7.9 +/- 0.3) at baseline to a median of 9.0 g/dL (8.9 +/- 0.4) following 12 weeks of Soliris treatment (P<0.001).

-- Patient fatigue levels, as measured by the FACIT-Fatigue instrument, significantly improved within one week of Soliris treatment, with a median increase of 5.0 points at 12 weeks (P<0.001). A change of three or more points is considered clinically meaningful.
-- An exploratory analysis was also performed to evaluate the effect of Soliris on Chronic Kidney Disease (CKD), measured as an improvement or worsening in CKD stage during treatment according to the KDOQI CKD published guidelines. (3) Chronic eculizumab treatment was associated with a 12-fold likelihood of an improvement in CKD level (P<0.001;CKD improved in 41 percent (12/29) of patients, CKD was unchanged in 55 percent (16/29), CKD worsened in 3.4 percent (1/29)).

The drug appeared to be safe and well tolerated in study patients. The most frequent adverse events (AEs) were headache (52 percent), nasopharyngitis (41 percent), and nausea (21 percent). Most AEs were mild to moderate in severity. No serious AEs related to study drug were reported following treatment with Soliris.

"The AEGIS results show that Soliris treats the hallmarks of PNH in Japanese patients with levels of safety and efficacy consistent with those demonstrated in U.S. and European study populations," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "This is an important step in the process of bringing Soliris to the substantial numbers of patients with PNH in Japan, which is tied to our objective that every patient who can benefit from Soliris will have access to Soliris."

The AEGIS study was authorized by Japan's Pharmaceutical and Medical Device Administration (PMDA). Alexion expects to include data from the AEGIS study in an application for marketing authorization to be submitted to the PMDA in 2009. The Company has begun to establish its commercial organization in Japan in anticipation of a commercial launch of Soliris in that country in 2010.

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. (4) PNH strikes people of all ages, with an average age of onset in the early 30s. (5) Approximately 10 percent of all patients first develop symptoms at 21 years of age or younger. (6) 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. (7) The estimated median survival for PNH patients is between 10 and 15 years from the time of diagnosis. (5,7)

PNH has been identified more commonly among patients with disorders of the bone marrow, including aplastic anemia (AA) and myelodysplastic syndromes (MDS). (8,9,10) In patients with thrombosis of unknown origin, PNH may be an underlying cause. (6,11)

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. (6,11)

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 and the timing of regulatory and commercial milestones for Soliris in Japan. 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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