Soliris(R) Receives Orphan Drug Designation in Japan

CHESHIRE, Conn., Jan 12, 2009 (BUSINESS WIRE) --

Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN) today reported that Soliris(R) (eculizumab), its first-in-class complement inhibitor, has been designated as an orphan drug by the Ministry of Health, Labour and Welfare of Japan. As a result of the designation, a New Drug Application (NDA) for Soliris would receive priority review from the Japanese regulatory authorities once it is submitted, and the drug would have 10 years of market exclusivity as a treatment for patients with paroxysmal nocturnal hemoglobinuria (PNH) in Japan.

Soliris was previously granted orphan drug status in the United States and European Union, and has been in use in the U.S. and European countries following regulatory approvals in 2007. AEGIS, an open-label registration study examining Soliris as a treatment for Japanese patients with PNH, was conducted during 2008, and on December 8, 2008 Alexion reported positive results from AEGIS. Alexion expects to include data from the AEGIS study in an NDA to be submitted in Japan in 2009. The period of market exclusivity under the orphan drug designation would commence upon approval of that application. The Company has begun to establish its commercial organization in Japan in anticipation of a commercial launch of Soliris in that country in 2010.

"The orphan drug designation reflects the therapeutic value of Soliris as a treatment for Japanese patients suffering with PNH, who currently lack an effective drug therapy for their disease," said Dr. Mitsuhiro Omine, Visiting Professor, Showa University Division of Hematology, Internal Medicine, Japan.

"Japanese scientists conducted much of the early research in PNH," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "In light of the long-standing awareness of PNH in Japan, and compelling results from the AEGIS trial, we look forward to the potential to provide the clinical benefits of Soliris to significant numbers of Japanese patients with PNH."

About PNH

PNH is a rare blood disorder that strikes people of all ages, with an average age of onset in the early 30s. (1) 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. (3) The estimated median survival for PNH patients is between 10 and 15 years from the time of diagnosis. (1,3)

PNH has been identified more commonly among patients with disorders of the bone marrow, including aplastic anemia (AA) and myelodysplastic syndromes (MDS). (4,5,6) In patients with thrombosis of unknown origin, PNH may be an underlying cause. (2)

Prior to approval of Soliris in the U.S. and European Union, 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. (2,7)

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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Alexion Pharmaceuticals, Inc.
Irving Adler, 203-272-8210
Sr. Director, Corporate Communications
or
Media
Makovsky + Company
Mark Marmur, 212-508-9670
or
Investors
Rx Communications
Rhonda Chiger, 917-322-2569