Alexion Submits NDA in Japan for Soliris(R) (eculizumab) as a Treatment for Patients with PNH

April 1, 2009

CHESHIRE, Conn., Apr 01, 2009 (BUSINESS WIRE) -- Alexion Pharmaceuticals, Inc., (Nasdaq: ALXN) today announced that it has submitted a New Drug Application (NDA) for Soliris? (eculizumab) as a treatment for patients with paroxysmal nocturnal hemoglobinuria (PNH) to Japan's Pharmaceuticals and Medical Devices Agency (PMDA). PNH is a rare, debilitating and life-threatening blood disorder defined by chronic red blood cell destruction, or hemolysis.

AEGIS Study in Japan

The NDA included data from AEGIS, an open-label registration study examining Soliris as a treatment for Japanese patients with PNH. (1) AEGIS was conducted to confirm the safety and efficacy of Soliris in Japanese patients with PNH relative to the previously reported SHEPHERD and TRIUMPH (2,3) Phase III Soliris trials, which were conducted in the United States, Europe, and Australia. AEGIS was conducted during 2008 and included 29 patients at nine institutions throughout Japan. Patients in AEGIS were dosed in accordance with the Soliris product labels approved in the United States and European Union in 2007. (1)

On December 8, 2008, Alexion reported positive results from AEGIS. The prespecified primary efficacy endpoint of change in hemolysis was achieved with an 86 percent reduction ($P<0.001$). Key secondary endpoints including improvement in fatigue ($P<0.001$) and reduction in transfusions ($P<0.001$) were also achieved. The drug appeared to be safe and well tolerated in study patients; most adverse events were mild to moderate in severity, with the most frequent being headache (52 percent), nasopharyngitis (41 percent), and nausea (21 percent). No serious adverse events related to study drug were reported following treatment with Soliris. (1)

Orphan Drug Designation

In January 2009, Soliris was designated as an orphan drug by Japan's Ministry of Health, Labour and Welfare. As a result of the designation, the NDA will receive priority review from the PMDA, and the drug would have 10 years of market exclusivity as a treatment for patients with paroxysmal nocturnal hemoglobinuria (PNH) in Japan upon approval.

"This NDA submission is an important step toward improving the lives of patients suffering from PNH in Japan," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "Given Japan's long-standing leadership in PNH research, the debilitating nature of this rare and serious disease, and the markedly positive results of the AEGIS study, we know that the Japanese medical community is eager to provide Soliris to patients as quickly as the regulatory process can allow."

Alexion 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 strikes people of all ages, with an average age of onset in the early 30s. (4) Approximately 10 percent of all patients first develop symptoms at 21 years of age or younger. (5) 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. (6) It is estimated that approximately one-third of patients with PNH do not survive more than five years from the time of diagnosis. (6) PNH has been identified more commonly among patients with disorders of the bone marrow, including aplastic anemia (AA) and myelodysplastic syndromes (MDS). (7,8,9) In patients with thrombosis of unknown origin, PNH may be an underlying cause. (5) 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, a rare, debilitating and life-threatening blood disorder defined by hemolysis, or the destruction of red blood cells. All four jurisdictions reviewed and approved their respective marketing applications for Soliris under their priority review or accelerated assessment procedures, and all four have designated Soliris as an orphan drug. Prior to these approvals, 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 its own substantial risks of mortality and morbidity. (5,10) 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. 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 diseases, cancer, and autoimmune disorders. Soliris is Alexion's first marketed product, approved in the U.S. and Europe in 2007, and Canada and Australia in 2009. 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 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 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-K for the period ended December 31, 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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