Alexion's Soliris(R) (eculizumab) Receives Marketing Approval in Canada for All Patients with PNH

CHESHIRE, Conn., Jan 29, 2009 (BUSINESS WIRE) -- Alexion Pharmaceuticals, Inc., (Nasdaq: ALXN) today announced that Canada's national healthcare regulatory agency, Health Canada, has approved the use of Soliris(R) (eculizumab) for the treatment of all patients in Canada with paroxysmal nocturnal hemoglobinuria (PNH), a rare, debilitating and life-threatening blood disorder defined by chronic red blood cell destruction, or hemolysis. Soliris is the first therapy approved in Canada for the treatment of PNH.

"Soliris has had a life-changing impact on patients with PNH and represents the only effective and safe drug therapy available for their disease," said Dr. Loree M. Larratt, M.D., Department of Medicine, Divisional Director Clinical Hematology, University of Alberta at Edmonton, and one of the researchers who participated in clinical trials of Soliris. "Hemolysis underlies the significant morbidities and mortality of PNH, and Soliris reduced hemolysis in every patient treated in clinical studies."

Soliris was approved under Priority Review by Health Canada's Biologics and Genetic Therapies Directorate (BGTD). The marketing application submitted to the BGTD included safety and efficacy data from three multi-national clinical studies: TRIUMPH, a placebo-controlled 26-week Phase 3 study involving 87 PNH patients, (1) SHEPHERD, an open-label 52-week Phase 3 trial involving 97 PNH patients, (2) and E05-001, a long term extension study. (3) Soliris was approved by the United States Food and Drug Administration and the European Commission in 2007 using data from the same studies and is currently being used to treat patients with PNH in the U.S. and more than 15 additional countries.

"This approval paves the way for patients with PNH across Canada to begin receiving the clinical benefits of Soliris," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "We are currently developing our organization in Canada and we will now work with Canada's public and private healthcare organizations to provide access to Soliris, patient-by-patient, as rapidly as possible."

"We are very excited that Health Canada has been able to move quickly to approve Soliris. We have been hearing from Canadian patients affected by PNH who have been waiting for access to this innovative drug," said Durhane Wong-Rieger, Ph.D., President of the Canadian Organization for Rare Disorders. "We call upon the private, provincial and federal drug plans to act without delay to provide this life-saving therapy to patients as soon as possible."

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) The estimated median survival for PNH patients is between 10 and 15 years from the time of diagnosis. (4,6) 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)

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. (5,10)

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 Canadian 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 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.

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.

[ALXN-G]

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 Canada. 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-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.

(1) Hillmen P, Young NS, Schubert J, et al. The complement inhibitor eculizumab in paroxysmal nocturnal hemoglobinuria. N *Engl J Med*. 2006;355:1233-1243.

(2) Brodsky RA, Young, NS, Antonioli E., et al. Multicenter phase 3 study of the complement inhibitor eculizumab for the treatment of patients with paroxysmal nocturnal hemoglobinuria. *Blood*. 2008;111:1840-1847.

(3) Socie G, Hillmen P, Muus P et al. Sustained improvements in transfusion requirements, fatigue and thrombosis with eculizumab treatment in paroxysmal nocturnal hemoglobinuria [abstract]. *Blood.* 2007; 110: A3672.

(4) Socie G, Mary J Yves, de Gramont A, et al. Paroxysmal nocturnal haemoglobinuria: long-term follow-up and prognostic factors. *Lancet*. 1996: 348:573-577.

(5) Parker C, Omine M, Richards S, et al. Diagnosis and management of paroxysmal nocturnal hemoglobinuria. *Blood*. 2005;106 (12):3699-3709.

(6) Hillmen P, Lewis SM, Bessler M, Luzzatto L, Dacie JV. Natural history of paroxysmal nocturnal hemoglobinuria. N *Engl J Med*. 1995; 333:1253-1258.

(7) Wang H, Chuhjo T, Yasue S, Omine M, Naka S. Clinical significance of a minor population of paroxysmal nocturnal hemoglobinuria-type cells in bone marrow failure syndrome. *Blood.* 2002;100 (12):3897-3902.

(8) Iwanga M, Furukawa K, Amenomori T, et al. Paroxysmal nocturnal haemoglobinuria clones in patients with myelodysplastic syndromes. *Br J Haematol*. 1998;102 (2):465-474.

(9) Maciejewski JP, Risitano AM, Sloand EM, et al. Relationship between bone marrow failure syndromes and the presence of glycophosphatidyl inositol-anchored protein-deficient clones. *Br J Haematol.* 2001;115:1015-1022.

(10) Hill A, Richards S, Hillmen P. Recent developments in the understanding and management of paroxysmal nocturnal haemoglobinuria. *Br J Haematol*. 2007;137 (3):181-192.

SOURCE: Alexion Pharmaceuticals, Inc.

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

Copyright Business Wire 2009