



## **Alexion Commences Dosing in AEGIS Registration Study of Soliris(R) (eculizumab) in Patients with PNH in Japan**

CHESHIRE, Conn., Jan 07, 2008 /PRNewswire-FirstCall via COMTEX News Network/ -- Alexion Pharmaceuticals, Inc. (the "Company," Nasdaq: ALXN) today announced that it has commenced dosing in the AEGIS study, a single registration study to evaluate the safety, efficacy, and pharmacology of Soliris(R) (eculizumab) as a treatment for Japanese patients with paroxysmal nocturnal hemoglobinuria (PNH). When fully enrolled, the open-label study, authorized by Japan's Pharmaceutical and Medical Device Administration (PMDA), is expected to include approximately 25 patients with PNH at clinical sites throughout Japan. Each PNH patient in the AEGIS study will be dosed with Soliris for 12 weeks after enrollment.

PNH is a rare 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. The primary efficacy endpoint of the study is reduction of hemolysis from baseline. The study will also measure the effect of Soliris on other clinical manifestations of PNH, including blood transfusion requirements, thromboses (blood clots), and kidney function. Patients' overall quality of life, including fatigue, will also be assessed.

The inclusion and exclusion criteria for the AEGIS study are similar to those used in one of Alexion's previous Phase 3 studies of Soliris as a treatment for PNH, the SHEPHERD study. SHEPHERD examined the safety and efficacy of eculizumab in a broad and diverse population of patients with PNH, including enrollment of patients with minimal transfusion requirements and/or evidence of thrombocytopenia. (1) Based on the SHEPHERD study and the TRIUMPH Phase 3 study, (2) Soliris was approved as the first treatment for patients with PNH by the U.S. Food and Drug Administration in March 2007 and by the European Commission in June 2007.

"Commencing patient dosing with eculizumab in the Japanese registration study represents an important step toward providing patients with PNH in Japan the same innovative therapy that is already available to patients in the U.S. and Europe," said Dr. Yuzuru Kanakura, M.D. Ph.D., Professor and Chairman Department of Hematology and Oncology, Osaka University Graduate School of Medicine. "We are excited to be participating in such an important study and look forward to the Soliris program progressing to registration in Japan."

"After consultation with the leading PNH clinical investigators in Japan and with the PMDA, we are pleased to have commenced dosing with Soliris in the AEGIS study, our single Japanese registration trial," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "After completing enrollment and dosing, and comparing these results in Japanese patients to our earlier PNH studies, we expect to submit a licensing application for Soliris in PNH to the Japanese regulatory authority. Initiating this Japanese registration study is an important component of reaching our global goal that every PNH patient who can benefit from Soliris will have access to it."

### **About PNH**

PNH is a rare blood disease 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. (3) PNH often strikes people in the prime of their lives, with an average age of onset in the early 30's. (4) Approximately ten 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 often 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 syndrome (MDS). (7,8,9,10) In patients with thrombosis of unknown origin, PNH may be an underlying cause. (5,11)

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. (5,11)

### **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](http://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](http://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. 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 (including the possibility that earlier clinical trials may not be representative of future results in the AEGIS study, the current Japanese registration trial), 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 us 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, 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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browser window.)

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