European Commission and U.S. FDA Grant Alexion's Soliris(R) (eculizumab) Orphan Drug Designation for the Treatment of Atypical Hemolytic Uremic Syndrome (aHUS)

Alexion Recruiting Patients in Four Soliris Clinical Studies in Europe, United States and Canada

CHESHIRE, Conn., Aug 06, 2009 (BUSINESS WIRE) -- Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN) announced today that Soliris(R) (eculizumab), its first-in-class complement inhibitor, has been granted Orphan Medicinal Product Designation by the European Commission for the treatment of patients with atypical Hemolytic Uremic Syndrome (aHUS). AHUS is an ultra-rare, inherited, and life-threatening complement-inhibitor deficiency disease that often progresses to end-stage kidney disease or failure. The U.S. Food and Drug Administration granted orphan drug designation to Soliris for the same indication in May 2009. Soliris is not approved for the treatment of patients with aHUS.

Alexion is currently enrolling patients at the initial sites in four clinical studies of Soliris as an investigational treatment for adolescent and adult patients with aHUS. Clinical studies are also currently being planned to investigate the use of Soliris as a treatment for children with aHUS. If Soliris is approved for the treatment of patients with aHUS in Europe or the U.S., orphan-drug status would entitle Alexion to 10 years of market exclusivity in Europe and seven years of market exclusivity in the U.S. for this use of Soliris.

Soliris is approved in the United States, European Union, Australia and Canada as a treatment for patients with paroxysmal nocturnal hemoglobinuria (PNH), an ultra-rare, debilitating, and life-threatening blood disorder. Soliris has also been designated as an orphan drug in these countries, as well as in Japan, for the treatment of patients with PNH.

"These additional orphan drug designations for Soliris underscore the unmet need faced by patients living with aHUS, an ultra-rare, genetic and life-threatening disease that destroys patients' kidneys," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "In response to the work of researchers and inquiries from practicing physicians, we are increasingly focusing our resources to investigate Soliris as a treatment for patients with aHUS."

About the aHUS Clinical Studies

Atypical Hemolytic Uremic Syndrome is characterized by chronic inflammation, hemolysis (red blood cell destruction), thrombocytopenia (reduced circulating platelets), and microangiopathy (damage in small blood vessels), particularly in the kidney and brain, often progressing to end-stage kidney disease or failure. Like PNH, aHUS is caused by a deficiency in normally occurring complement inhibitor proteins. Typically, patients with aHUS have genetic mutations in one of several complement inhibitor proteins that lead to uncontrolled complement activation. Excessive complement activation may contribute to severe inflammation of the blood vessels and blood clotting through the activation of white blood cells, platelets, and the endothelial cell lining of blood vessels. (1)

The prognosis for patients with aHUS is generally poor. Approximately 70 percent of patients with the most common mutation experience chronic renal insufficiency, chronic dialysis, or death within one year of the first clinical episode. (2) Despite current best supportive care, following kidney transplantation, recurrent aHUS causes kidney transplant failure in up to approximately 60 to 90 percent of patients. (3)

Alexion is currently enrolling patients at the initial sites in four prospective, open-label clinical studies of eculizumab as a treatment for patients with aHUS in North America and multiple European countries: two studies of patients who are plasma therapy sensitive (one in adults and one in adolescents) and two studies of patients who are plasma therapy resistant (one in adults and one in adolescents). In addition to the ongoing trials, clinical studies are currently being planned to investigate the use of eculizumab as a treatment for children with aHUS. Physicians, patients and care givers who are interested in participating in these clinical trials can learn more by contacting Alexion by e-mail at clinicaltrials@alxn.com, or by visiting the Alexion website at www.alexionpharma.com and clicking on the clinical trials link. The ongoing trials are also posted to the www.clinicaltrials.gov website maintained by the U.S. National Institutes of Health.

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, an ultra-rare, debilitating and life-threatening blood disorder defined by chronic hemolysis, or the destruction of red blood cells. Prior to these approvals, there were no therapies specifically available for the treatment of PNH. 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. Meningococcal infection may become rapidly life-threatening or fatal if not recognized and treated early. 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 and kidney diseases, transplant, cancer, and autoimmune disorders. Soliris is Alexion's first marketed product. 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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This news release contains forward-looking statements, including statements related to potential health and medical benefits from Soliris (eculizumab). 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 published reports or clinical trials are not predictive of safety and efficacy results of Soliris in broader patient populations, the risk that clinical trials may not be completed successfully, 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, 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 June 30, 2009, 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) Ståhl A, Vaziri-Sani F, Heinen S, Kristoffersson A-C, Gydell K-H, Raafat R, Gutierrez A, Beringer O, Zipfel PF, and Karpman D. Factor H dysfunction in patients with atypical hemolytic uremic syndrome contributes to complement deposition on platelets and their activation. *Blood*. 2008;111:5307-5315.

(2) Caprioli J, Noris M, Brioschi S, Pianetti G, Castelletti F, Bettinaglio P, et al. Genetics of HUS: the impact of MCP, CFH, and IF mutations on clinical presentation, response to treatment, and outcome. *Blood.* 2006 Aug 15;108(4):1267-79).

(3) Loirat C, Noris M, Fremeaux-Bacchi V. Complement and the atypical hemolytic uremic syndrome in children. *Pediatr Nephrol*. 2008 Nov;23(11):1957-72.

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