



## **First Clinical Experience With Soliris(R) in Treating Patients With Two Rare Complement-Mediated Diseases Presented at ASH Annual Meeting**

### **Effects of Complement Inhibition With Eculizumab Observed in Patients with Atypical Hemolytic Uremic Syndrome (aHUS) and Cold Agglutinin Disease (CAD)**

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CHESHIRE, Conn., Dec. 7 /PRNewswire-FirstCall/-- Soliris(R) (eculizumab), a terminal complement inhibitor developed by Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN) for the treatment of paroxysmal nocturnal hemoglobinuria (PNH), was associated with an improvement in hematologic parameters in a limited number of patients with two other rare and severe complement-mediated diseases treated outside of a clinical trial, according to patient cases presented today at the 50th Annual Meeting of the American Society of Hematology (ASH).

Physicians reported on the first clinical experience with Soliris in patients with atypical Hemolytic Uremic Syndrome (aHUS) and Cold Agglutinin Disease (CAD), two rare and serious diseases. Like patients with PNH, patients with aHUS are missing or have defective complement inhibitors that help to regulate the body's immune system. These patients suffer from hemolysis, blood clotting which can be measured in part as reduced number of circulating platelets, and kidney damage. In two patients with aHUS, Soliris administration was associated with improvements in platelet levels and reduction in hemolysis. Patients with CAD suffer from an autoimmune attack on their red blood cells leading to severe complement activation and hemolysis (destruction of those blood cells), anemia, and poor quality of life. In one patient with CAD, Soliris treatment was associated with reduced hemolysis, absence of the need for blood transfusions and improved symptoms of fatigue and anemia. Eculizumab appeared to be well tolerated in these patients, with safety observations that were consistent with those reported from controlled trials in patients with PNH.

"We are encouraged by this initial clinical experience with eculizumab in a very limited number of aHUS and CAD patients. These observations and other knowledge of the mechanisms at work in these disorders lead us to believe that there are strong scientific rationales for undertaking controlled clinical trials to investigate the role of complement inhibition in these two conditions," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "As previously announced, we are working closely and urgently with clinical experts from Europe and North America to design clinical trials of eculizumab for the treatment of aHUS and CAD."

#### **Initial Experience with Eculizumab in aHUS**

A poster titled "Successful Treatment of Atypical Hemolytic Uremic Syndrome with the Complement Inhibitor Eculizumab" was presented today at the ASH annual meeting by Dr. Jens Nuernberger of the Department of Nephrology at University Duisburg-Essen in Essen, Germany.

Dr. Nuernberger and his colleagues investigated the potential effect of eculizumab in two patients with aHUS, a rare disease usually caused by a genetic mutation in the complement system. In patients with aHUS, this defect results in hemolysis, formation of tiny blood clots (microvascular thrombosis) and inflammation of blood vessels, often causing acute kidney injury and progressing to end-stage kidney disease. Those who survive aHUS often live with irreversible kidney disease and are dependent upon dialysis to stay alive.

"Our first clinical experience with eculizumab in patients with aHUS is very promising, especially given the devastating nature of this disease and the lack of effective treatments," said Dr. Nuernberger. "Initial data suggest that eculizumab reduced hemolysis, microvascular thrombosis, and blood vessel inflammation, thereby appearing to improve the course of aHUS. If clinical trials confirm these results, eculizumab may then improve our ability to care for these patients."

#### **Initial Experience with Eculizumab in CAD**

A poster titled "Long-term Efficacy of the Terminal Complement Inhibitor Eculizumab in a Patient with Cold Agglutinin Disease" was presented today at the ASH annual meeting by Dr. Alexander Roeth of the Department of Hematology at the University Hospital of Essen in Essen, Germany.

Dr. Roeth and his colleagues investigated the potential effect of eculizumab therapy in one patient with CAD, a rare and severe disease characterized by the production of circulatory cold agglutinins, a type of monoclonal antibody that lead to hemolysis.

Conventional treatments for CAD, including corticosteroids and immunosuppressive drugs, are ineffective in many patients.

"We currently lack effective treatment options for patients with CAD," noted Dr. Roeth. "Given these promising initial observations, clinical research is needed to determine the therapeutic potential of eculizumab in patients with CAD."

#### 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. Soliris is not approved for the treatment of atypical Hemolytic Uremic Syndrome (aHUS) and Cold Agglutinin Disease (CAD).

#### 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, 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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