New England Journal of Medicine Publishes Case Reports on the Investigational Use of Soliris(R) (eculizumab) in Patients with Atypical Hemolytic Uremic Syndrome (aHUS)

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CHESHIRE, Conn., Jan 29, 2009 (BUSINESS WIRE) -- Two separate case reports published today in the *New England Journal of Medicine* (*NEJM*) examine the investigational use of Soliris (R) (eculizumab), a terminal complement inhibitor developed by Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN), in patients with a rare and severe inflammatory disease called atypical Hemolytic Uremic Syndrome (aHUS). In both cases, physicians observed a significant reduction in the destruction of red blood cells, reduced platelet consumption and improved kidney function following Soliris therapy.

Separately, Alexion announced today that it is currently initiating clinical trials of eculizumab in patients with aHUS. Soliris is approved in the United States, European Union, and Canada for the treatment of patients with paroxysmal nocturnal hemoglobinuria (PNH).

Atypical Hemolytic Uremic Syndrome is characterized by hemolysis, thrombocytopenia and clotting of blood vessels (microangiopathy), particularly in the kidney and brain, often progressing to end-stage kidney disease. Like PNH, aHUS is caused by a deficiency in normally occurring complement inhibitors. 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 poor. Approximately 70% of patients with the most common mutation experience chronic renal insufficiency, chronic dialysis, or death by one year after the first clinical episode. (2) Following kidney transplantation, recurrent aHUS causes kidney transplant failure in approximately 62 - 88% of patients. (3)

Case Reports

In a case report submitted to the NEJM by Ralph A. Gruppo, M.D., Director of the Comprehensive Hemophilia and Thrombosis Center at the Cincinnati Children's Hospital Medical Center, an 18-month-old infant was admitted to the hospital following a fourth clinically severe relapse of congenital aHUS. During this episode, the patient did not respond to daily plasmapheresis, a procedure whereby proteins are removed from the blood by circulating the patient's blood through a machine. When the patient's condition deteriorated further, the physician administered Soliris. Complete blockade of terminal complement was observed in this patient, and hematologic and renal improvement began within 48 hours after initiation of treatment. Plasmapheresis was discontinued within the first week of eculizumab treatment and clinical remission was sustained throughout the 60 day observation period. The report further notes that eculizumab therapy is ongoing for over four months, to date, with sustained disease remission and no further plasma therapy intervention.

"It is well known that aHUS is a devastating disease without effective treatment options," explained Dr. Gruppo, lead author of the report. "Further, after clinical worsening, there may be virtually nothing a physician can do to prevent further kidney damage and eventual kidney failure. Based on this initial and very limited experience, further studies of terminal complement inhibition with eculizumab are warranted."

In a second case reported in the same issue of the NEJM by Dr. Jens Nuernberger of the Department of Nephrology at University Duisburg-Essen in Essen, Germany, a 37-year-old woman with a history of kidney failure due to aHUS and loss of her first kidney transplant due to recurrent aHUS, was admitted to the hospital with progressive and severe aHUS shortly after her second kidney transplant. The patient's aHUS condition continued to clinically worsen despite extensive plasma treatments, indicating a high probability that the second kidney transplant would fail. After the administration of eculizumab, hemolysis quickly resolved, platelet count rebounded and kidney transplant function recovered. The patient's renal graft function has remained stable.

Eculizumab appeared to be well tolerated in these two patients, with safety observations that have been consistent with those reported from controlled trials with eculizumab in patients with PNH.

"There is a profound need to improve the management of aHUS. We are encouraged by the initial clinical experience with eculizumab in a very limited number of aHUS patients, and we are undertaking prospective clinical trials to investigate the role of complement inhibition in this condition," said Leonard Bell, M.D., Chief Executive Officer of Alexion.

Upcoming Clinical Studies

Alexion is currently initiating 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). Information on the trials can be requested by e-mail using the address clinicaltrials@alxn.com, or by visiting the Alexion website at www.alexionpharma.com and clicking on the clinical trials link. The trials also will be posted to the www.clinicaltrials.gov website maintained by the U.S. National Institutes of Health.

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 and January 2009, respectively, the European Commission (EC) and Health Canada also approved the use of Soliris for the treatment of patients with PNH. Soliris is the first therapy approved in the U.S., Europe and Canada 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 in the U.S., Europe, Canada or elsewhere for the treatment of atypical Hemolytic Uremic Syndrome (aHUS).

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.

Please see full U.S. prescribing information at 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, and in January 2009, Health Canada granted marketing approval in Canada 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.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. 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 or published case reports 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) Stahl 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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