New Research Presented at EHA Congress Shows That Soliris(R) Significantly Reduced Hemolysis in Never-Transfused Patients with PNH

Separate Analysis of Patients with PNH and Thrombocytopenia Found Sustained Platelet Recovery Following Treatment with Soliris

CHESHIRE, Conn., Jun 07, 2009 (BUSINESS WIRE) -- Clinical investigators observed that Soliris(R) (eculizumab), a first-in-class terminal complement inhibitor developed by Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN), reduced hemolysis (red blood cell destruction) and improved symptoms in nine patients with paroxysmal nocturnal hemoglobinuria (PNH) who had received no blood transfusions prior to initiating Soliris therapy.

In a separate study of 11 patients with PNH, researchers observed sustained platelet recovery with Soliris treatment in a subset of seven patients with thrombocytopenia (reduced platelet levels), indicating a likely reversal of platelet consumption with Soliris in these thrombocytopenic PNH patients. These and other data sets were presented on June 6 and 7 at the European Hematology Association Congress in Berlin. Soliris is the only therapy approved in the European Union, United States, Australia and Canada for the treatment of patients with PNH, an ultra-rare, debilitating, and life-threatening blood disorder.

"All patients with PNH, including those who do not require transfusion and may appear to be stable, are at increased risk for blood clots, kidney dysfunction, pulmonary hypertension and disabling fatigue caused by hemolysis," noted Leonard Bell, M.D., Chief Executive Officer of Alexion. "Research presented at EHA is a sobering reminder of the clinical consequences of this progressive, ultra-rare disease. These data further underscore the clinical impact of Soliris for the treatment of patients with PNH, and also provide insight into the potential role of complement inhibition in addressing other complement-mediated diseases."

Never-Transfused and Minimally Transfused Patients with PNH

Abstract 0581 titled "Efficacy of the Complement Inhibitor Eculizumab in Paroxysmal Nocturnal Hemoglobinuria Patients Never Transfused," was presented at a poster session at the EHA Congress on Saturday, June 6 by Dr. Antonio Risitano, Research Associate at the University of Naples in Italy.

Published research shows that Soliris reduces hemolysis in patients with PNH who require minimal transfusions. (1) However, many patients with PNH do not receive blood transfusions and continue to experience hemolysis and its clinical consequences. In this analysis, investigators assessed the safety and efficacy of Soliris in the treatment of nine patients with PNH who required no transfusions prior to starting Soliris therapy. These "never-transfused" patients were enrolled in the Italian Early Access Program with at least one of the following conditions: severe anemia due to intravascular hemolysis; frequent paroxysmal crises; severe symptoms due to hemolysis; or thrombosis (life-threatening blood clots).

All patients experienced a dramatic reduction in hemolysis following treatment with Soliris for a median of 16 months, as measured by a median reduction in LDH from 1,500 U/L before treatment to 356 U/L after treatment (p=0.008). Overall, hemoglobin levels increased significantly from 9.0 g/dL before treatment to 10.7 g/dL after treatment (p=0.0003), with a median increase of 2.0 g/dL. The investigator noted that patients reported a marked improvement in quality of life. No serious adverse events were reported.

Investigators compared these results to a subset of 21 patients previously enrolled in eculizumab clinical trials who had received zero or one transfusion during the year prior to eculizumab treatment. These patients experienced a significant reduction in hemolysis following six months of Soliris therapy, with a median reduction in LDH from 2,030 U/L before treatment to 336 U/L after treatment ($p < 0.001$). Hemoglobin levels increased significantly from 9.0 g/dL before treatment to 10.7 g/dL after treatment (p=0.0003), with a median increase of 1.7 g/dL. Fatigue was also significantly improved ($p<0.001$).

"PNH is a debilitating and life-threatening disease, even among patients who do not require blood transfusion," noted Dr. Risitano. "Based on clinical data, eculizumab therapy inhibited hemolysis in these never-transfused patients, leading to immediate clinical benefit and potentially reducing the long-term morbidity and mortality associated with this ultra-rare disease."

Patients with PNH and Thrombocytopenia

Abstract 0584 titled "Effect of Eculizumab Therapy on Thrombocytopenia in Patients with Paroxysmal Nocturnal Hemoglobinuria," was presented at a poster session at the EHA Congress on Saturday, June 6 by Ilene Ceil Weitz, M.D.,

Assistant Clinical Professor of Medicine, Jane Anne Nohl Division of Hematology, Keck School of Medicine of the University of Southern California.

Dr. Weitz presented additional results from an ongoing prospective study to measure the effect of Soliris therapy on measures of inflammation and thrombin generation in patients with PNH. The study uses highly sensitive laboratory tests to analyze blood samples collected from patients with PNH prior to treatment with Soliris and prior to each dose during the following 90 days.

Preliminary results presented at the American Society of Hematology Annual Meeting in December 2008 found that patients with PNH, only one of whom had been previously diagnosed with a blood clot, exhibited a hypercoagulable state (high risk of blood clots) prior to treatment with Soliris, as indicated by elevated levels of key inflammatory and pro-thrombotic measures, including D-dimers and thrombin-antithrombin (TAT) complex. (2)

Among the 11 patients enrolled in this study, seven had thrombocytopenia with platelet counts below 100 x 10^9/L (range: 26 to 88 x 10^9/L) prior to Soliris treatment. A sustained platelet recovery above 100,000 occurred in four out of seven patients during treatment with Soliris for periods ranging from two to 20 months. Levels of D-Dimer and TAT were elevated in all of the patients with thrombocytopenia prior to treatment, and decreased following Soliris therapy.

"These results suggest that in some patients with PNH, thrombocytopenia may be due to platelet consumption and not bone marrow failure," said Dr. Weitz. "The finding that Soliris reduces thrombin-mediated platelet consumption in these PNH patients may have implications for the treatment of patients with other complement-mediated diseases complicated by thrombocytopenia."

Additional Data

The following research was presented during a poster session at the EHA Annual Meeting on Saturday, June 6:

- Abstract 0585: "Efficacy of the Terminal Complement Inhibitor Eculizumab Used Chronically in a Patient with Cold Agglutinin Diseases (CAD)," Dr. Alexander Röth.
- Abstract 0587: "Modification of the Standard Eculizumab Dose To Successfully Manage Intravascular Haemolysis Breakthrough in Patients with Paroxysmal Nocturnal Hemoglobinuria," Dr. Richard Kelly.

The following research was presented in an oral presentation at the EHA Annual Meeting on Sunday, June 7:

- Abstract 1110: "Eculizumab Reduces Pulmonary Hypertension Through Inhibition of Haemolysis-Associated Nitric Oxide Consumption in Patients with Paroxysmal Nocturnal Hemoglobinuria," Dr. Anita Hill.

The following research was presented in an oral presentation at the EHA Annual Meeting on Sunday, June 7:

- Abstract 1114a: "Successful Pregnancy Outcome in Paroxysmal Nocturnal Hemoglobinuria on Long Term Eculizumab," Dr. Richard Kelly.

About PNH

Patients with PNH suffer from hemolysis (red blood cell destruction) which leads to thromboses (blood clots), disabling fatigue, anemia, impaired quality of life, pulmonary hypertension, shortness of breath, recurrent pain, kidney disease and intermittent episodes of dark-colored urine (hemoglobinuria). (3, 4) PNH is an ultra-rare blood disorder that strikes people of all ages, with an average age of onset in the early 30s. (5) Approximately 10 percent of all patients first develop symptoms at 21 years of age or younger. (3) 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) It is estimated that approximately one-third of patients with PNH do not survive more than five years from the time of diagnosis. (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. (3) More information on PNH is available at www.pnhsource.com.

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 paroxysmal nocturnal hemoglobinuria (PNH), a rare, debilitating and life-threatening blood disorder defined by hemolysis, or the destruction of red blood cells. All four jurisdictions reviewed and approved their respective marketing applications for Soliris under their priority review or accelerated assessment procedures, and all four have designated Soliris

as an orphan drug. 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. 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, approved in the U.S. and Europe in 2007, and Canada and Australia in 2009. 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.

[ALXN-G]

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 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 March 31, 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. Bessler M, Schrezenmeier H, MaciejewskiJP, et al. Significant disease burden in paroxysmal nocturnal hemoglobinuria patients with lower levels of hemolysis, mild anemia and minimal transfusion: clinical improvement with eculizumab therapy [abstract]. *Blood*. 2007; 110 (11): A840.

2. Weitz IC, Ghods M, Rochanda L, et al. Eculizumab therapy results in rapid and sustained decreases in markers of thrombin generation and inflammation in patients with PNH [abstract]. *Blood.* 2008;112:A407.

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

4. Hill A, Richards S, Hillmen P. Recent developments in the understanding and management of paroxysmal nocturnal haemoglobinuria. *Br J Haematol*. 2007;137:181-92.

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

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.

SOURCE: Alexion Pharmaceuticals, Inc.

Alexion Pharmaceuticals, Inc.
Irving Adler, 203-271-8210
Sr. Director
Corporate Communications
or
Makovsky & Company
Media
Kristie Kuhl, 212-508-9642
or
Rx Communications
Investors
Rhonda Chiger, 917-322-2569