Soliris® Reversed Thrombocytopenia in Patients with Both PNH and Pre-Existing Thrombocytopenia in Study Presented at ASH Annual Meeting

Data Highlight Likely Ongoing Platelet Consumption in Untreated PNH Patients
Abstract 4030, Poster Board III-966

CHESHIRE, Conn., Dec 07, 2009 (BUSINESS WIRE) -- Soliris® (eculizumab), a first-in-class terminal complement inhibitor developed by Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN), reversed thrombocytopenia (abnormally low platelet count) in a significant proportion of patients with both paroxysmal nocturnal hemoglobinuria (PNH) and pre-existing thrombocytopenia, according to an analysis of data from 49 patients enrolled in clinical trials of Soliris in PNH. Soliris increased platelet count by inhibiting terminal complement-mediated platelet activation and consumption, potentially reducing the risk of developing thrombosis. Improvement in platelet count was observed in thrombocytopenic PNH patients irrespective of history of thrombosis or bone marrow failure. The data were presented today at the 51st Annual Meeting of the American Society of Hematology (ASH) in a poster titled, "Terminal Complement Inhibitor Eculizumab Improves Complement-Mediated Platelet Consumption and Thrombocytopenia in Patients with Paroxysmal Nocturnal Hemoglobinuria.""Based on this analysis, unregulated terminal complement activity in patients with PNH can lead to ongoing platelet activation and consumption and contribute to thrombocytopenia, potentially increasing the risk of thrombosis in these patients," said Gerard Socie, M.D., Ph.D., of Hospital Saint-Louis, Paris, France and lead author of the study. "Terminal complement inhibition with Soliris can significantly reduce platelet consumption, which may account for the lower rate of thrombosis observed in patients with PNH treated with Soliris in clinical trials." "This research increases our understanding of the relationship between complement activation and platelet consumption in thrombocytopenic PNH patients," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "The finding that Soliris reduces platelet consumption in patients with PNH may have implications for the treatment of patients with other diseases complicated by complement-mediated thrombocytopenia." Clinical DataIn this analysis, researchers examined whether chronic inhibition of terminal complement activation with Soliris increases platelet counts in patients with PNH and thrombocytopenia. The study population consisted of 49 patients with PNH and thrombocytopenia (defined as platelet count <100 x109/L) prior to eculizumab treatment, identified from the 195 patients in Soliris PNH clinical trials. Platelet counts were measured at baseline, 26 and 52 weeks during Soliris treatment. Patients with thrombocytopenia were more likely to have a history of thromboembolic events than patients with normal platelet counts (45% vs 27%; P=0.02). Among thrombocytopenic patients with PNH treated with Soliris, median platelet counts increased significantly from 68 x 109/L at baseline to 80 and 85 x 109/L (P<0.001) at 26 and 52 weeks, respectively. Soliris treatment was associated with a reversal of thrombocytopenia in a significant proportion of patients studied, with 33% of previously thrombocytopenic patients improving to a non-thrombocytopenic condition (defined as platelet count >100,000 x 109/L) at week 26, and 36% at 52 weeks. Although patients showed significant improvements in platelet counts, there was no change in absolute neutrophil count from baseline to week 26 or week 52, suggesting that the improvements in platelet counts with Soliris are likely not due to improvement in underlying marrow blood cell production, but rather to reduced platelet consumption associated with terminal complement inhibition. Treatment with Soliris also markedly inhibited terminal complement activity in all thrombocytopenic patients, as measured by a significant reduction in LDH at 26 and 52 weeks (P<0.0001 for each time point vs. baseline). Soliris treatment with eculizumab significantly increased platelet counts irrespective of a history of bone marrow failure (P<0.05 vs. baseline at 52 weeks) or history of thromboembolic events (P<0.03 vs. baseline in both history and no history of thrombosis). About PNHPNH is an ultra-rare blood disorder that strikes people of all ages, with an average age of onset in the early 30s. (1) 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). (2,3) Approximately 10 percent of all patients first develop symptoms at 21 years of age or younger. (2) 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. (4) It is estimated that approximately one-third of patients with PNH do not survive more than five years from the time of diagnosis. (4) PNH has been identified more commonly among patients with disorders of the bone marrow, including aplastic anemia (AA) and myelodysplastic syndromes (MDS). (5,6,7) In patients with thrombosis of unknown origin, PNH may be an underlying cause. (1) More information on PNH is available at www.pnhsource.com. About SolirisSoliris 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 InformationSoliris 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 AlexionAlexion 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.Safe HarborThis 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 September 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. 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. 2. Parker C, Omine M, Richards S, et al. Diagnosis and management of paroxysmal nocturnal hemoglobinuria. Blood. 2005;106 (12):3699-3709. 3. Hill A, Richards S, Hillmen P. Recent developments in the understanding and management of paroxysmal nocturnal haemoglobinuria. Br J Haematol. 2007;137:181-92. 4. Hillmen P, Lewis SM, Bessler M, Luzzatto L, Dacie JV. Natural history of paroxysmal nocturnal hemoglobinuria. N Engl J Med. 1995; 333:1253-1258. 5. 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. 6. 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. 7. 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.

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