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

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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 Data In 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 x10^9/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 10^9/L at baseline to 80 and 85 x 10^9/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 10^9/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<0.001 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
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