FDA Approves Alexion's Soliris(TM) for All Patients With PNH

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Terms:

- First Therapy Approved for This Rare and Life-Threatening Blood Disease -

Alexion Pharmaceuticals, Inc., (Nasdaq: ALXN) announced today that it has received marketing approval from the U.S. Food and Drug Administration (FDA) for Soliris(TM) (eculizumab). Soliris is the first therapy approved for paroxysmal nocturnal hemoglobinuria (PNH), a rare, disabling and life-threatening blood disorder defined by chronic red blood cell destruction, or hemolysis. Soliris is indicated for the treatment of patients with PNH to reduce hemolysis.

Hemolysis can cause one or more of the following symptoms in patients with PNH: severe anemia, disabling fatigue, recurrent pain, shortness of breath, pulmonary hypertension, intermittent episodes of dark colored urine (hemoglobinuria), kidney disease, impaired quality of life and blood clots (thromboses).(1,2) PNH often strikes people in the prime of their lives, with an average age of onset in the early 30's.(3) The estimated median survival for PNH patients is between 10 and 15 years from the time of diagnosis.(3,4)

Patients with PNH are missing a specific protein that normally protects red blood cells from destruction by a component of the immune system called terminal complement. Soliris, the first complement inhibitor approved in the United States for the treatment of any disease, prevents hemolysis by selectively blocking terminal complement.

"Soliris brings real hope to people who live daily with the devastating effects of PNH. With the approval of Soliris, we now have a therapy that dramatically improves the lives of patients suffering from this disease. Importantly, all patients with this life-threatening disease will be eligible for treatment," said Leonard Bell, MD, chief executive officer of Alexion Pharmaceuticals.

"Soliris directly targets the underlying disease process responsible for debilitating symptoms that may contribute to shortened life spans of PNH patients," said Wendell F. Rosse, MD, Florence McAlister Professor of Medicine Emeritus, Duke University. "Having cared for more than 300 patients with PNH over my career, I believe this is the most important advance that has been made in the treatment of this disease. Treatment with Soliris markedly decreases the hemolysis responsible for anemia, fatigue, poor patient functioning and blood clots in PNH patients."

Clinical Data

Soliris has proven to be a safe and effective therapy for PNH in three multi-national clinical studies: TRIUMPH, a placebo-controlled 26 week Phase 3 study involving 87 PNH patients,(5) SHEPHERD, an open-label 52 week Phase 3 trial involving 97 PNH patients,(6) and E05-001, a long term extension study.(7)

These studies showed that Soliris reduced hemolysis in every treated patient. Hemolysis was dramatically reduced from a baseline LDH of 2,032 U/L to 239 U/L at week 26 ($p<0.001$). The reductions in hemolysis occurred within one week of initiating treatment and were sustained for periods of up to 54 months with continued dosing of Soliris. The reduction in hemolysis expands the number of circulating PNH cells and, thereby, increases the hemoglobin level. Hemoglobin stabilization and the number of transfused packed red blood cell units, the pivotal study's co-primary endpoints, were both achieved; half of the Soliris-treated patients achieved hemoglobin stabilization compared with none of the patients in the placebo group, the median number of transfusions was reduced from 10 units/patient to 0 units/patient, respectively ($p < 0.001$ in both cases). Soliris patients reported less fatigue and improved health-related quality of life. There were fewer thrombotic events with Soliris treatment than during the same period of time prior to treatment.

"The Aplastic Anemia & MDS International Foundation (AA&MDSIF) is extremely pleased that PNH patients now have a treatment specifically for their disease. This is a tremendous step forward for all who suffer from PNH -- and for anyone with bone marrow failure," said Sherrie VanVliet, vice-chairperson, AA&MDSIF, and the mother of a child with aplastic anemia. "We also appreciate that Alexion, in cooperation with the National Organization of Rare Diseases, has established a program to ensure that all patients who need eculizumab have access to it."

"Patients with rare diseases face huge challenges -- first getting a proper diagnosis, then finding effective treatments and access to them," said Abbey Meyers, president, National Organization for Rare Disorders (NORD). "Now that Soliris has been approved, people with PNH will have an effective treatment, and through NORD's PNH Foundation, the uninsured and underinsured will receive help to assure they will have access. This is a sign of hope for others with orphan illnesses, that their needs will one day be met," she added.

Introducing Soliris OneSource(TM)

Alexion also today introduced Soliris OneSource(TM), a treatment support service for all PNH patients and their healthcare providers. Each patient enrolled in the program receives support from an Alexion Case Manager at OneSource. Alexion Case Managers are Registered Nurses and provide education about PNH and Soliris and facilitate solutions to help patients obtain

Soliris. Alexion's goal is that all PNH patients who can benefit from Soliris will have access to it. Patients and their health care providers can learn more about OneSource by calling 1-888-SOLIRIS (1-888-765-4747) or visiting 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.(8) Treatment with Soliris should not alter anticoagulant management because the effect of withdrawal of anticoagulant therapy during Soliris treatment has not been established.

The product label for Soliris also includes a boxed warning: "Soliris increases the risk of meningococcal infections. Vaccinate patients with a meningococcal vaccine at least 2 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. "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 will be 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.

Conference Call Information

Alexion will host a conference call/webcast to discuss FDA approval of Soliris. The call is scheduled for Monday, March 19th at 9:00 a.m., Eastern Time. To participate in this call, dial 913-981-4900, confirmation code 4700435, shortly before 9:00 a.m., Eastern Time. A replay of the call will be available for a limited period following the call, beginning at 12:00 p.m., Eastern Time. The replay number is 719-457-0820, confirmation code 4700435. The audio webcast can be accessed at: www.alexionpharm.com.

About PNH

PNH is an acquired genetic blood disorder defined by hemolysis, in which patients' red blood cells are destroyed by complement, a component of the body's immune system. PNH is a rare disease that affects an estimated 8,000 to 10,000 people in North America and Europe. Ten 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 often ranging from one to more than 10 years. PNH has been identified more commonly among patients with diseases of the bone marrow, including aplastic anemia (AA) and myelodysplastic syndrome (MDS). In patients with thrombosis of unknown origin, PNH may be an underlying cause.

Prior to approval of Soliris, there were no therapies specifically available for the treatment of PNH. PNH treatment was limited to symptom management through periodic blood transfusions, non-specific immunosuppressive therapy and, infrequently, bone marrow transplantations -- a high-risk and painful procedure used as a last resort.(2)

About Alexion

Alexion Pharmaceuticals is a biotechnology company working to develop and deliver life-changing drug therapies for patients with serious and life- threatening medical conditions. Alexion's lead product, Soliris(TM) (eculizumab), is indicated for the treatment of paroxysmal nocturnal hemoglobinuria (PNH). Alexion is engaged in the discovery and development of therapeutic products aimed at treating patients with severe disease states, including hematologic diseases, cancer and autoimmune disorders. In September 2006, Alexion applied for marketing authorization with the European Medicines Evaluation Agency for the use of Soliris(TM) (eculizumab) in PNH patients. This press release and further information about Alexion Pharmaceuticals, Inc. can be found at: http://www.alexionpharm.com.

This news release contains forward-looking statements, including statements related to medical benefits and commercial potential of Soliris(TM)(eculizumab), initiation and conduct of the PNH OneSource(TM) treatment support service, and estimates of the number of people living with PNH. Forward-looking statements are subject to factors that may cause Alexion's results and plans to differ from those expected, including requests by regulatory authorities for additional information following marketing approval, delays in arranging satisfactory manufacturing capability, inability to acquire funding on timely and satisfactory terms, delays in or failure to establish internal sales and marketing capabilities, delays in developing or adverse changes in commercial relationships, the possibility that results of clinical trials are not predictive of the safety and efficacy of Soliris(TM)(eculizumab) when made available to larger number of people, the risk that third parties won't agree to license any necessary intellectual property to us on reasonable terms, the risk that third party payors will not reimburse for the use of Soliris(TM) at acceptable rates or at all, the risk that estimates regarding the number of people living with PNH 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 Alexion's Annual Report on Form 10-K. 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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(2) Parker C, Omine M, Richards S, et al. Diagnosis and management of paroxysmal nocturnal hemoglobinuria. Blood 2005; 106:3699-3709.

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

(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) Hillmen P, Young N, et al. The Complement Inhibitor Eculizumab in Paroxsysmal Nocturnal Hemoglobinuria. N Engl J Med 2006; 355:1233-1243.

(6) Young N, Antonioli E, Rotoli B, et al. Safety and efficacy of the terminal complement inhibitor eculizumab in patients with paroxysmal nocturnal hemoglobinuria: SHEPHERD phase III clinical study results. Blood 2006; 108:971.

(7) Hillmen P, Muus P, Duhrsen U, et al. The terminal complement inhibitor eculizumab reduces thrombosis in patients with paroxysmal nocturnal hemoglobinuria. Blood 2006; 108:123.

(8) Soliris(TM) (eculizumab) prescribing information. Alexion Pharmaceuticals, Inc., 2006.

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