



## **PNH Patients Treated with Soliris(TM) Experienced Dramatic Reduction in Blood Clots During Clinical Trials**

### **Analysis published in the journal Blood found 92 percent reduction in incidence of thromboses**

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Patients with a rare blood disorder called PNH experienced a 92 percent reduction in the incidence of life-threatening blood clots (thromboses) following treatment with Soliris(TM) (eculizumab), according to an analysis of clinical studies recently published online in *Blood*, the journal of the American Society of Hematology.

Soliris, developed by Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN), is the first therapy approved for paroxysmal nocturnal hemoglobinuria (PNH), a rare, debilitating and life-threatening blood disorder defined by the destruction of red blood cells, or hemolysis. In patients with PNH, hemolysis can cause 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 thromboses. (1,2)

"Thrombosis is the leading cause of premature death in PNH patients and the most feared complication of PNH," said Dr. Peter Hillmen, senior author of the paper and Consultant Haematologist of the General Infirmary at Leeds, Leeds, UK. "In several clinical trials, Soliris reduced hemolysis in all PNH patients and significantly improved anemia, fatigue and quality of life. The comprehensive analyses demonstrate that Soliris substantially reduced the risk of thrombosis in a diverse population of PNH patients, including those who might be expected to have less severe disease."

Approximately 40 percent of patients with PNH experience thrombosis during the course of the disease.(3,4,5,6) An initial thrombosis increases the relative risk of death by five- to ten-fold for PNH patients.(3,7) Thrombosis is related to hemolysis in patients with PNH, and patients with both smaller and larger PNH clones are reported to experience this complication. (8,9,10,11,12) Research involving a small group of PNH patients, including those without a history of blood transfusion, found that 60 percent had sub-clinical thrombosis, which could later develop into blood clots.(13)

"For decades, people with PNH have lived with debilitating symptoms, including the threat of blood clots and their complications," said Leonard Bell, MD, Chief Executive Officer of Alexion. "This publication reinforces the importance of our goal to ensure that every PNH patient who can benefit from Soliris will have access to it."

"Many PNH patients face not only devastating symptoms, but a sharply increased risk for developing life-threatening blood clots as a result of their disease," said John Huber, Executive Director of the Aplastic Anemia & MDS International Foundation. "This news provides additional hope for patients struggling with PNH and the danger of PNH-related blood clots. We look forward to seeing the impact this new analysis has on treatment of patients with PNH."

#### **Clinical Data**

The publication, titled "Effect of the complement inhibitor eculizumab on thromboembolism in patients with paroxysmal nocturnal hemoglobinuria," analyzed data from 195 patients studied in the Soliris Phase II and Phase III trials, including the TRIUMPH and SHEPHERD studies. Patients were enrolled in the United States, Europe, Australia and Canada and treated with Soliris for up to 54 months. Soliris reduced hemolysis in all treated patients and reduced thrombosis by 92 percent, with three events during Soliris treatment compared to 39 events during the same period of time prior to treatment ( $P < 0.0001$ ). The collection of thrombotic events was defined prospectively by clinical trial protocols. The article is available online at <http://bloodjournal.hematologylibrary.org/cgi/content/abstract/blood-2007-06-095646v1>. The analysis will also be published in a future print edition of *Blood*.

#### **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.(13) PNH often strikes people in the prime of their lives, with an average age of onset in the early 30's.(3) Approximately 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.(4) The estimated median survival for PNH patients is

between 10 and 15 years from the time of diagnosis.(13,4)

PNH has been identified more commonly among patients with disorders of the bone marrow, including aplastic anemia (AA) and myelodysplastic syndrome (MDS).(14,15,16,17) In patients with thrombosis of unknown origin, PNH may be an underlying cause.(2,18)

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 procedure that carries considerable mortality risk.(2,18)

#### 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, the European Commission (EC) also approved the use of Soliris(TM) (eculizumab) for the treatment of patients with PNH. Soliris is the first therapy approved in Europe for the treatment of PNH and was the first medicinal product to receive EU approval under the EMEA Accelerated Assessment Procedure.

#### 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." 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](http://www.soliris.net).

#### About Alexion

Alexion Pharmaceuticals, Inc. is a biotechnology company working to develop and deliver life-changing drug therapies for patients with serious and life-threatening medical conditions. The Company 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 the Company's first product, Soliris for all patients with PNH and the Company began commercial sale of Soliris in the U.S. during April 2007. In June 2007, the European Commission granted marketing approval for Soliris in the European Union for all patients with PNH. The Company is evaluating other potential indications for Soliris as well as other formulations of eculizumab for additional clinical indications, and is actively 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: <http://www.alexionpharm.com>.

This news release contains forward-looking statements, including statements related to potential benefits and commercial potential for Soliris, and interest about Soliris in the patient, physician and payor communities. 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 us 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 Soliris will not generate interest among physicians, the risk that estimates regarding the number of PNH patients are inaccurate, the risk that pending litigation may be resolved adversely, 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 June 30, 2007 and in our 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.

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