



## Alexion Announces the EXPLORE Clinical Diagnostic Study for Paroxysmal Nocturnal Hemoglobinuria (PNH)

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CHESHIRE, Conn., June 16 -- With research suggesting that many patients with Paroxysmal Nocturnal Hemoglobinuria (PNH) go undiagnosed for years and that many patients have PNH in association with bone marrow failure disorders,(1) Alexion Pharmaceuticals (Nasdaq: ALXN) today announced the initiation of the multi-center EXPLORE study to examine the frequency of PNH in patients with aplastic anemia (AA), myelodysplastic syndrome (MDS) and other bone marrow failure disorders. Although the hemolytic blood disorder PNH is associated with bone marrow disorders, it is often misdiagnosed or undetected in these patients.

Current published guidelines recommend that patients with aplastic anemia, refractory anemia-myelodysplastic syndromes, and patients with any one of the following -- venous thrombosis involving unusual sites, hemoglobin in the urine, intravascular hemolysis without antibodies, or episodic abdominal or swallowing pain and hemolysis -- should be screened for PNH.(1) The test for PNH requires only a small sample of the patient's blood from a vein in the arm.

The announcement of the EXPLORE study coincides with the launch of an Alexion-sponsored Web site, PNHSource.com, which will serve as the first PNH-specific, on-line informational resource for physicians, patients and caregivers.

The EXPLORE (EXamination of PNH, by Level Of CD59 on REd and white blood cells, in bone marrow failure syndromes) study expects to engage approximately 200 hematologists to enroll an estimated 2,000 patients. Patients with any evidence of AA, MDS, or other bone marrow failure disorders will be asked to give informed consent to enter into the study's screening phase. The trial will mark the first systematic effort to determine the presence of the PNH blood cell clone in patients with AA, MDS, or other bone marrow failure disorders. Enrollment is expected to begin this summer in the U.S. and is then expected to expand to Europe and Asia. Healthcare providers that are interested in learning more about the EXPLORE study may register online at <http://www.PNHSource.Com>.

PNH is a rare, severe anemia that is associated with significant morbidity and early mortality. Based upon scientific investigations and presentations of the prevalence of patients diagnosed with abnormal PNH cells in their blood, it is currently estimated that approximately 8,000-10,000 people in North America and Western Europe suffer from PNH. It is caused by a hematopoietic stem cell mutation resulting in a deficiency of CD59, a complement inhibitor protein that blocks the formation of the terminal complement complex on the blood cell surface and prevents hemolysis. The mean age of onset is about 30- 40 years of age and approximately 10 percent of all newly diagnosed patients are 21 or younger.(1,2) The PNH phenotype may arise in early progenitor stem cells as a rescue attempt for failed bone marrow, and many patients are totally dependent on these cells for survival. PNH symptoms include intravascular hemolysis, hemoglobinuria and thrombosis. The most common symptoms at initial presentation include anemia, hemoglobinuria, bleeding, gastrointestinal symptoms, jaundice, or blood clots.(1)

"Alexion is committed to expanding awareness and increasing the community's knowledge about this debilitating and often life-threatening disease to physicians and patients," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "With these new initiatives, we hope to further clinical understanding of PNH and its association with disorders of bone marrow failure, and ensure that all patients with PNH receive an accurate diagnosis. At the same time, we look forward to providing a valuable resource and an opportunity for collaboration and communication through our Web site."

The EXPLORE study will enroll patients 18 and older with evidence of AA, MDS syndromes (refractory anemia, refractory anemia with ringed sideroblasts, refractory cytopenia with multilineage dysplasia, refractory cytopenia with multilineage dysplasia and ringed sideroblasts, refractory anemia with excess blasts (type 1 and 2), unclassified MDS and 5q-syndromes), as well as other bone marrow failure disorders. An initial peripheral blood draw will be used for hematological evaluation. Patients who test positive for the PNH clone may be asked to participate in a two-year follow-up to monitor symptoms and conduct hematological assessments.

1. Parker C, Omine M, Richards S, et al. Diagnosis and management of paroxysmal nocturnal hemoglobinuria. *Blood* 2005 Dec 1; 106(12): 3699-709.

2. Nishimura, J, Kanakura, Y, Ware, RE et al. Clinical course and flow cytometric analysis of paroxysmal nocturnal hemoglobinuria in the United States and Japan. *Medicine* 2004 May 83(3):193-207.

### 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 is engaged in the discovery and development of therapeutic products aimed at treating patients with a wide array of severe disease states, including hematologic diseases, cancer, and autoimmune disorders. Alexion's two lead product candidates, Soliris(TM) (eculizumab) and pexelizumab, are currently undergoing evaluation in several clinical development programs, including two Phase III trials of Soliris(TM) (eculizumab) for the treatment of paroxysmal nocturnal hemoglobinuria (PNH). Under the Special Protocol Assessment (SPA) process, the FDA has agreed to the design of protocols for the two trials of Soliris(TM) (eculizumab) in PNH patients that could, if successful, serve as the primary basis of review for approval of a licensing application for eculizumab in the PNH indication. Results from the PRIMO-CABG2 trial of pexelizumab in coronary artery bypass graft (CABG) surgery patients indicate that the trial is unlikely to support filing for licensing approval of pexelizumab in the CABG indication. The APEX- AMI trial of pexelizumab in acute myocardial infarction patients was conducted pursuant to a protocol approved under the SPA process; however, that trial has ended prior to enrolling the anticipated number of patients. Accordingly, APEX-AMI results are unlikely to be reviewed under the SPA process. The pexelizumab trials are conducted in collaboration with Procter and Gamble Pharmaceuticals. Alexion is engaged in discovering and developing a pipeline of additional antibody therapeutics targeting severe unmet medical needs, through its wholly owned subsidiary, Alexion Antibody Technologies, Inc. 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 characterization of clinical trial results, timing of announcement of clinical trial results, commercial potential of Alexion's drug candidates, the progression of Alexion's drug candidates towards commercial sales and timing for submission of, and decisions with respect to, marketing applications for Soliris(TM)(eculizumab). Forward-looking statements are subject to factors that may cause Alexion's results and plans to differ from those expected, including delays in completion of ongoing clinical trials, delays in completion of analysis of clinical trial results, timing and evaluation by regulatory agencies of the results of these and other clinical trials, the results of pre-clinical or clinical studies (including termination or delay in clinical programs), the need for additional research and testing, decision of the FDA or other regulatory authorities not to approve (or to materially limit) marketing of one or both of Alexion's two drug candidates, delays in arranging satisfactory manufacturing capability, inability to acquire funding on timely and satisfactory terms, delays in developing or adverse changes in commercial relationships, the possibility that results of earlier clinical trials are not predictive of safety and efficacy results in later clinical trials, dependence on Procter & Gamble Pharmaceuticals for development and commercialization of pexelizumab, the risk that third parties won't agree to license any necessary intellectual property to us on reasonable terms, 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 Transition Report on Form 10-K/T for the five-month transition period ended December 31, 2005 and in our other filings with the Securities and Exchange Commission. P&GP retains the development rights and the termination rights discussed in Alexion's Form 10-K/T referred to above. 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.