



Eculizumab is Well Tolerated and Demonstrates Significant Improvement During 52 Weeks of Treatment in Open-Label Phase III SHEPHERD Study in PNH Patients

- Results Presented at American Society of Hematology Annual Meeting -

CHESHIRE, Conn., Dec. 9 -- Eculizumab, a novel monoclonal antibody investigational drug developed by Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN - News), appeared to be safe and well tolerated and provided clinically and statistically significant improvements in intravascular hemolysis, anemia, fatigue and quality of life in patients with paroxysmal nocturnal hemoglobinuria (PNH) during the 52 weeks of treatment in the Phase III SHEPHERD open-label clinical trial. These results were presented Saturday at the 48th Annual Proceedings of the American Society of Hematology in Orlando, Florida.

PNH, a rare and life-threatening form of hemolytic anemia, is an acquired genetic blood disorder characterized by destruction of red blood cells by the body's complement system (a component of the immune system). Patients with PNH lack naturally-occurring complement inhibitors on the surface of their red blood cells that normally prevent red blood cell destruction. Patients with PNH may experience severe hemolysis (red blood cell destruction), anemia, disabling fatigue, recurrent pain, and intermittent episodes of dark colored urine, known as hemoglobinuria. Importantly, PNH patients are at increased risk of forming life-threatening blood clots, or thromboses, which are a significant cause of death. Eculizumab, a long-acting C5 complement inhibitor, is a humanized monoclonal antibody drug designed to selectively block terminal complement activation, thereby preventing destruction of red blood cells by complement in patients with PNH. There currently is no therapy specifically available for the treatment of PNH. 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 to 10,000 people in North America and Europe suffer from PNH.

"The SHEPHERD results presented today broaden our earlier findings in the Phase III TRIUMPH study, further defining the efficacy and safety profile of eculizumab in patients diagnosed with PNH," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "Data from the open-label SHEPHERD study show that eculizumab may provide significant clinical benefit to a broader, more diverse population of PNH patients than previously observed in TRIUMPH. We are grateful to the dedicated physicians and other healthcare providers, patients and their families and caregivers who contributed to the successful execution of this global trial for this life-threatening and rare blood disorder."

About SHEPHERD

SHEPHERD was an open-label, global Phase III study in which patients received eculizumab for 52 weeks. The pre-specified primary endpoints of the trial were safety and a reduction in intravascular hemolysis as measured by the surrogate endpoint lactate dehydrogenase (LDH). Pre-specified secondary endpoints included fatigue and intravascular hemolysis; other endpoints assessed included patient reported outcomes and measures of anemia including transfusion requirements.

During the study, eculizumab was found to be well tolerated with an adverse event profile similar to that of placebo patients in the Phase III TRIUMPH study. Further, no serious adverse events were deemed probably or definitely related to treatment. The most common adverse events reported with eculizumab treatment in SHEPHERD were: headache, nasopharyngitis, nausea and upper respiratory tract infection. These events were generally consistent with those observed with eculizumab during six months of treatment in TRIUMPH and with six months of placebo treatment in TRIUMPH.

As presented at the ASH scientific meetings, eculizumab therapy reduced intravascular hemolysis, as shown by a reduction in the median LDH area under the curve (-632,264 U/L W day; $P < 0.001$). LDH levels during the study were reduced 87% from a median of 2051 U/L at baseline to 269 U/L after 12 months of treatment ($P < 0.001$). Clinically and statistically significant improvements in fatigue were observed as measured by change from baseline using the Functional Assessment of Chronic Illness Therapy-Fatigue (FACIT-Fatigue) instrument ($P < 0.001$) and the fatigue scale of the European Organization for Research and Treatment of Cancer Quality of Life Questionnaire (EORTC QLQ-C30) instrument ($P < 0.001$). Treatment with eculizumab also significantly improved overall health and patient functioning as measured by the EORTC QLQ-C30 instrument including the global health status scale ($P < 0.001$) and all five aspects of patient functioning: role ($P < 0.001$), social ($P < 0.001$), cognitive ($P < 0.001$), physical ($P < 0.001$) and emotional ($P < 0.001$). Treatment also significantly improved 7 of 9 EORTC QLQ-C30 symptom scales and single item measures including pain ($P < 0.001$), dyspnea ($P < 0.001$), appetite loss ($P < 0.001$), and insomnia ($P < 0.001$). Clinically and statistically significant improvements in anemia were also observed with eculizumab therapy as evidenced by the reduction in transfusion requirements from a median of 8.0 packed red cells in the 12 month pre-treatment period to 0.0 units during the 12 months of treatment ($P < 0.001$). Additionally, 51% of patients were transfusion independent for the entire 12 month treatment period ($P < 0.001$). Other improvements in anemia included a 44% increase in the endogenous PNH red blood cell mass ($P < 0.001$) and an increase in hemoglobin levels from 9.2g/dl at baseline to 10.2g/dl after 12 months of

treatment (P<0.001).

"The SHEPHERD results presented at the ASH meetings show that PNH patients treated with eculizumab experienced statistically and clinically important improvements in anemia and quality of life over a prolonged period of time in a controlled clinical setting on a global basis," said Dr. Robert A. Brodsky, leading SHEPHERD investigator and Director, Division of Hematology and Associate Professor of Medicine and Oncology, The Sidney Kimmel Comprehensive Cancer Center at Johns Hopkins University School of Medicine. "Importantly, the improvements with eculizumab treatment occurred in a diverse and heterogeneous population of PNH patients. Treatment options are extremely limited for PNH. The results from the 12 month SHEPHERD study and the six month TRIUMPH study both suggest that there may be great potential for eculizumab to provide an effective therapy for patients diagnosed with this life-threatening disorder."

Development Update and Expanded Access Program

Alexion also announced the initiation of an Expanded Access Program for the investigational agent eculizumab in the United States for patients with paroxysmal nocturnal hemoglobinuria (PNH), in accordance with a Treatment Protocol authorized by the U.S. Food and Drug Administration (FDA). Treatment Protocols are designed to make promising investigational agents available for patients with serious or life-threatening diseases for which there are no comparable or satisfactory alternative therapies, before general marketing commences. EMBRACE (The Paroxysmal Nocturnal Hemoglobinuria Early Access Treatment Protocol) will begin immediately.

"This is wonderful news for the PNH community. Eculizumab is the first investigational treatment specifically targeted for PNH and has the potential to significantly improve the quality of life for PNH patients. We are pleased that the FDA has allowed for expanded access of eculizumab to PNH patients," said Dr. Sarah Higgins, President of the PNH Research and Support Foundation.

It is anticipated that treatment in EMBRACE will continue until marketing approval. Alexion is also working with clinicians and appropriate authorities outside the United States in order to evaluate mechanisms for access to eculizumab in other countries where such programs are available. The number of patients eligible for enrollment in the expanded access program may be limited due to logistical considerations. In the event of oversubscription, the program is designed to enroll eligible patients on an impartial basis. Interested patients should ask their physicians to contact an Alexion representative for further information and to evaluate eligibility at 1-866-551-EMBRACE or EMBRACE@alxn.com.

The eculizumab Biologics License Application (BLA) is currently under review by the FDA for the treatment of PNH and has been granted Priority Review status. The European Medicines Evaluation Agency (EMA) has accepted the eculizumab Marketing Authorization Application (MAA) for review under the EMA's Accelerated Assessment Procedure. In addition to data from the SHEPHERD study, the marketing applications also contain data from the pivotal Phase III TRIUMPH trial, which met all pre-specified primary and secondary endpoints with statistical significance. Details regarding the TRIUMPH study results are included in an article published in the September 21, 2006 issue of the New England Journal of Medicine. Eculizumab has been granted Orphan Drug status by both the FDA and the EMA.

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 lead product candidate, Soliris(TM) (eculizumab), is currently undergoing evaluation in several clinical development programs, including 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 Phase III trials of Soliris(TM) (eculizumab) in PNH patients, known as the TRIUMPH and SHEPHERD studies. All primary and secondary endpoints in the TRIUMPH and SHEPHERD studies were achieved with statistical significance, and eculizumab appeared to be safe and well tolerated in both studies. In September, 2006, Alexion applied for marketing authorization with both the United States Food and Drug Administration and the European Medicines Evaluation Agency for the use of Soliris(TM) (eculizumab) in PNH patients. Results from the TRIUMPH and SHEPHERD trials served as the primary basis for the marketing applications filed in the United States and Europe. Alexion is engaged in discovering and developing a pipeline of additional antibody therapeutics targeting severe unmet medical needs. 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 of Soliris(TM), clinical trial results, initiation and conduct of an expanded access program, estimates of the number of PNH patients, and timing of regulatory authorities' 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 the SHEPHERD trial, delays in completion of analysis of clinical trial results, requests by the FDA or other regulatory authorities for additional information or data, timing and evaluation by regulatory agencies of our applications, the need for additional research and testing, decision of the FDA or other regulatory authorities not to approve

(or to materially limit) marketing of Soliris(TM), 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 clinical trials are not predictive of the safety and efficacy of Soliris(TM), 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 PNH patients 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 the risks discussed in Alexion's Quarterly Report on Form 10-Q for the quarter ended September 30, 2006, 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.