

## Soliris(TM) (eculizumab) Demonstrates Significant Improvement in PNH Study

- -Phase III TRIUMPH Study Published in New England Journal of Medicine-
- Alexion Pharmaceuticals compound found to reduce symptoms, improve quality of life in clinical study -

## Soliris(TM) (eculizumab) Demonstrates Significant Improvement in PNH Study

CHESHIRE, Conn., September 20, 2006 - Soliris<sup>TM</sup> (eculizumab), a novel monoclonal antibody drug developed by Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN), significantly reduced symptoms of paroxysmal nocturnal hemoglobinuria (PNH), a severe life-threatening hemolytic anemia, as compared with placebo, according to the results of a study, TRIUMPH, published today in The New England Journal of Medicine. The data show that anemia was improved in the group of patients receiving Soliris<sup>TM</sup>. Soliris<sup>TM</sup> treated patients required a median of 0 units of red blood cells transfused per patient as compared with a median of 10 units per patient required in the placebo group (p<0.001). The data also show significant stabilization of hemoglobin over a six month period, with 49% of Soliris<sup>TM</sup> treated patients achieving stabilization as compared to 0% for patients receiving placebo (p<0.001). The number of red blood cell units transfused and hemoglobin stabilization are two key measures of anemia. The study enrolled 87 patients at 45 sites in the US, Canada, Europe and Australia.

"Today's publication of the TRIUMPH results shows that patients treated with Soliris experienced statistically and clinically important improvements in anemia and quality of life as compared to placebo in a controlled clinical setting on a global basis," said Dr. Peter Hillmen senior author, lead investigator and chairman of the TRIUMPH steering committee and Consultant Haematologist of the General Infirmary at Leeds, Leeds, UK. "Importantly, the improvements with Soliris treatment occurred in patients with severe anemia and markedly diminished quality of life at baseline. Treatment options are extremely limited for PNH. While we continue to study Soliris treatment in the Phase III SHEPHERD safety trial and in the E05-001 extension study, the TRIUMPH results suggest that there may be great potential for Soliris to provide an effective therapy for patients diagnosed with this life-threatening disorder.

"Results of an initial open-label, 11 patient pilot study of Soliris™ in PNH were reported in the February 5, 2004 issue of The New England Journal of Medicine in which patients treated with Soliris™ experienced a substantial decrease in intravascular hemolysis and transfusion requirements and an improvement in quality of life. In June 2006, Alexion announced that the sixmonth interim results from the ongoing SHEPHERD Phase III safety study showed that Soliris™ appeared to be safe and well tolerated and that all primary and secondary efficacy endpoints were achieved with statistical significance. SHEPHERD is an open-label, 12-month Phase III study primarily focused on examining safety, as well as efficacy measures, with Soliris™ in approximately 95 PNH patients in the US, Canada, Europe and Australia. The TRIUMPH and SHEPHERD Phase III studies will serve as the primary basis of review for approval of a licensing application for Soliris™ in the PNH indication. SHEPHERD is scheduled to complete in 2006.

"The TRIUMPH results published today provide a strong foundation as we look to establish the efficacy and safety profile with Soliris in patients diagnosed with PNH," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "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 pivotal trial for this life-threatening and rare blood disorder. As announced today, we have submitted the Biologics License Application for Soliris to the Food and Drug Administration in the United States. We are equally committed to submitting the Marketing Application for Soliris to the EMEA in the European Union this year and to subsequent regulatory submissions on a global basis."

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 blood cells which normally prevent red blood cell destruction. Patients with PNH may experience severe hemolysis (red blood cell destruction), anemia, chronic fatigue, recurrent pain, pulmonary hypertension 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. Soliris™ (eculizumab), a long-acting C5 complement inhibitor, is a humanized monoclonal antibody drug designed to selectively block terminal complement activation and thereby restore complement inhibition in the blood of patients with PNH. There currently is no therapy specifically available for the treatment of PNH. It is currently estimated that approximately 8,000 to 10,000 people in North America and Europe suffer from PNH.

"Soliris is a potentially important step forward in the search for the first ever treatment for PNH. Data from the TRIUMPH study suggest that many patients may benefit from its use. Treatments like this can give hope to patients and their families. More

research on the disease and its treatment is needed, and we applaud Alexion for all that it has done to help patients with PNH," stated Marilyn Baker, President of the Aplastic Anemia and MDS International Foundation.

## **About TRIUMPH**

In the TRIUMPH study, patients received either placebo or eculizumab at a dose of 600 mg intravenously for the first 4 weeks, 900 mg at week 5, and then 900 mg every 2 weeks through the 6 month trial period. The two pre-specified primary endpoints, which measured anemia, were the number of units of packed red blood cells transfused and the stabilization of hemoglobin levels. Pre-specified secondary endpoints were intravascular hemolysis, fatigue, and the avoidance of red blood cell transfusions. During the study, a median of 0 units of packed red cells was administered in the eculizumab group, as compared with 10 units in the placebo group (P<0.001). Stabilization of hemoglobin levels in the absence of transfusions was achieved in 49% (21 of 43) of the patients assigned to eculizumab and none (0 of 44) of those assigned to placebo (P<0.001). Eculizumab reduced intravascular hemolysis, as shown by the 85.8% lower median area under the curve for lactate dehydrogenase in the eculizumab group, as compared with the placebo group (58,587 vs. 411,822 U per liter x day; P<0.001). Clinically and statistically significant improvements in fatigue were observed as measured by scores on the Functional Assessment of Chronic Illness Therapy-Fatigue instrument (P<0.001) and the fatigue subscale of the European Organization for Research and Treatment of Cancer Quality of Life Questionnaire (EORTC QLQ-C30) (P<0.001). Treatment with eculizumab also significantly improved overall health and patient functioning as measured by the EORTC QLQ-C30 instrument including global health status (P<0.001) and all five aspects of patient functioning: role (P<0.001), social (P=0.003), cognitive (P=0.002), physical (P<0.001) and emotional (P=0.008). Treatment also significantly reduced EORTC QLQ-C30 disease-related symptoms including pain (P=0.002), dyspnea (P<0.001), appetite loss (P<0.001), and insomnia (P=0.014). The most common adverse events reported in the eculizumab group were headache, nasopharyngitis, back pain, and nausea; of these, headache and back pain occurred more frequently in the eculizumab group than in the placebo group. Of the 87 randomized patients, 4 in the eculizumab group and 9 in the placebo group experienced serious adverse events, none of which were considered to be treatment-related.

## **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<sup>TM</sup> (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<sup>TM</sup> (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. In January, 2006, Alexion announced that the first of those two PNH trials, the TRIUMPH study, achieved its co-primary endpoints with statistical significance. In June 2006, Alexion announced that interim results from the second of those two PNH trials, the SHEPHERD study, showed that eculizumab appeared to be safe and well tolerated and that all primary and secondary efficacy endpoints were achieved with statistical significance. 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.alxn.com/.

This news release contains forward-looking statements, including statements related to potential benefits and commercial potential of Soliris, clinical trial results, the timing of completion of additional clinical trial results, the likely basis of review of Alexion's BLA for Soliris, estimates of the number of PNH patients, and timing for submission of, and regulatory authorities' decisions with respect to, marketing applications for Soliris™ (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 either prior to their acceptance of our submission for filing or following their review of our applications, 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, 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<sup>TM</sup>, 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 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 June 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.

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