# Alexion Pharmaceuticals Submits Biologics License Application for Soliris(TM) (eculizumab)

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Product News

*- European Application on Track for Submission in 2006 -*

Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN) today announced that it has submitted a Biologics License Application (BLA) with the U.S. Food and Drug Administration (FDA) for its lead product candidate Soliris™ (eculizumab) as a treatment for patients diagnosed with paroxysmal nocturnal hemoglobinuria (PNH), a rare life-threatening genetic blood disorder.

"There currently is no treatment specifically available for PNH, which can be a debilitating disease that often shortens lives," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "We believe Soliris represents a significant advance in the management of PNH and in improving the lives of patients diagnosed with the disease. We look forward to working with the FDA as it reviews our application and we remain on track to submit a Marketing Authorization Application (MAA) in Europe by the end of this year."

The BLA submission is based on data from the pivotal Phase III TRIUMPH trial which met all pre-specified primary and secondary endpoints with high levels of statistical significance. Details regarding the TRIUMPH study results are included in an article published today in the New England Journal of Medicine. The BLA submission also includes interim data of at least six months from the open-label Phase III SHEPHERD safety trial. Final 12-month data from the SHEPHERD trial will be submitted to the application. The TRIUMPH trial included 87 PNH patients and the SHEPHERD trial has enrolled 97 PNH patients. An additional 11 PNH patients were enrolled in an earlier pilot study.

Alexion has requested Priority Review designation for this BLA from the FDA. If granted, the FDA has up to six months from submission date to take action on the BLA. Priority Review may be granted to drug products that provide significant improvement compared to existing treatments for a disease. Alexion has already been granted Accelerated Assessment by the EMEA for Soliris™ (eculizumab) in Europe once the MAA is submitted.

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 which normally prevent red blood cell destruction. SolirisTM (eculizumab), a long-acting C5 terminal complement inhibitor, is a monoclonal antibody drug that is designed to selectively block terminal complement activation and thereby restore complement inhibition in the blood of patients with PNH. Soliris™ (eculizumab) has been granted Orphan Drug Status in the PNH indication from both the FDA and European regulatory agencies. There currently is no approved therapy specifically available for 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. Patients with PNH may suffer from severe hemolysis, 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 major cause of death in this disease.

**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™ (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™ (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.alexionpharm.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 submission of additional clinical trial results, 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™, 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.

To download further materials related to Alexion and this press release, click here

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