# SECURITIES AND EXCHANGE COMMISSION

**WASHINGTON, D.C. 20549**

# FORM 8-K

**CURRENT REPORT**
**PURSUANT TO SECTION 13 OR 15(d) OF THE**
**SECURITIES EXCHANGE ACT OF 1934**

**Date of Report (Date of earliest event reported) July 20, 2004**

# ALEXION PHARMACEUTICALS, INC.

**(Exact Name of Registrant as Specified in its Charter)**

| **Delaware** | **0-27756** | **13-3648318** |
|---|---|---|
| **(State or Other Jurisdiction of Incorporation)** | **(Commission File Number)** | **(IRS Employer Identification No.)** |

| **352 Knotter Drive, Cheshire, CT** | **06410** |
|---|---|
| **(Address of Principal Executive Offices)** | **(Zip Code)** |

**Registrant's telephone number, including area code: (203) 272-2596**

**Not Applicable**
**(Former Name or Former Address, if Changed Since Last Report)**

**Item 5. Other Events and Regulation FD Disclosure.**

On July 20, 2004, the Company announced that it received written confirmation from the U.S. Food and Drug Administration ("FDA") indicating agreement with two protocols for a clinical trial of its investigational drug eculizumab for treatment of the chronic orphan blood disorder Paroxysmal Nocturnal Hemoglobinuria under the FDA's Special Protocol Assessment process. A copy of the press release issued by the Company relating thereto is filed herewith as Exhibit 99.1.

On July 20, 2004, the Company announced that it and its collaboration partner for pexelizumab, Procter & Gamble Pharmaceuticals, Inc., have initiated patient enrollment for the PRIMO-CABG-2 trial in patients undergoing coronary artery bypass graft surgery. The Company also announced that enrollment was initiated in the APEX-AMI trial in patients experiencing acute myocardial infarction treated with primary percutaneous intervention. A copy of the press release issued by the Company relating thereto is filed herewith as Exhibit 99.2.

**Item 7. Financial Statements and Exhibits.**

**(c) Exhibits.**

99.1 Press Release dated July 20, 2004.

99.2 Press Release dated July 20, 2004.

**SIGNATURES**

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

ALEXION PHARMACEUTICALS, INC.

Date: July 20, 2004

By: /s/ Thomas I.H. Dubin

Name: Thomas I.H. Dubin
Title: Vice President and General Counsel

**Alexion Pharmaceuticals Reaches Agreement with FDA for Pivotal Phase III Eculizumab Program in the Orphan Disease Paroxysmal Nocturnal Hemoglobinuria**

- Special Protocol Assessment Process Completed for TRIUMPH Pivotal Trial -

- Conference Call Scheduled for 9:00 a.m. EDT Today -

CHESHIRE, Conn., July 20 /PRNewswire-FirstCall/ — Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN) announced today that it has received written confirmation from the U.S. Food and Drug Administration (FDA) indicating agreement with the two protocols constituting the pivotal Phase III program of its investigational drug eculizumab for the chronic orphan blood disorder Paroxysmal Nocturnal Hemoglobinuria ("PNH"). The Phase III program includes the TRIUMPH pivotal trial together with a companion safety trial called SHEPHERD. The agreement for the Phase III program was reached under the FDA's Special Protocol Assessment (SPA) process, a procedure by which the FDA provides official evaluation and guidance on proposed protocols for pivotal Phase III clinical trials. It is expected that, if successful, the trials will complete the filing package that will serve as the primary basis of review for the approval of a Biologics License Application for the PNH indication.

Alexion will host a conference call and audio webcast to discuss matters mentioned in this release today, July 20th, at 9:00 a.m., Eastern Time. To participate in this call, dial 913-981-5509, confirmation code 595961, shortly before 9:00 a.m. The audio webcast can be accessed at: http://www.alexionpharm.com. A replay of the call will be available for a limited period following the call, beginning at 12:00 p.m., Eastern Time. The replay number is 719-457-0820, confirmation code 595961.

The pivotal Phase III trial, called TRIUMPH, will examine the effects of eculizumab on the co-primary endpoints of hemoglobin stabilization and blood transfusion in hemolytic transfusion-dependent PNH patients during six months of therapy. TRIUMPH will be a double-blind, randomized, placebo-controlled multi-center clinical trial. The study is expected to enroll approximately 75 patients in the US, Canada, Europe, Australia and New Zealand. Alexion is preparing to initiate the study in the near term.

A companion safety trial, called SHEPHERD, will be primarily aimed at generating additional safety data with eculizumab in hemolytic PNH patients with a history of transfusion. SHEPHERD will be an open-label, non-randomized, non-placebo-controlled, multi-center clinical trial. The study is expected to enroll approximately 75 patients in the US, Canada, Europe, Australia and New Zealand. Alexion expects to initiate the SHEPHERD trial after commencement of the TRIUMPH study.

The TRIUMPH trial will mark the second study performed with eculizumab in PNH patients. Results of the first trial were reported in the February 5, 2004 issue of the New England Journal of Medicine. The open-label study was conducted in the UK at two sites and included 11 patients treated with eculizumab for an initial three month period. Results showed that patients treated with eculizumab experienced a substantial decrease in the destruction of red blood cells, as lactate dehydrogenase (LDH) levels fell from a mean of 3,111 IU per liter to a mean of 594 IU per liter (P=0.002) and the mean percentage of PNH red blood cells increased from 36.7% of the total population found in the body to 59.2% (P=0.005). This reduction in red blood cell destruction helped reduce the median patient transfusion rates from 1.8 units per patient, per month, to 0.0 units per patient, per month (P=0.003). Episodes of hemoglobinuria were

reduced by an average of 96% (P<0.001) and quality of life measurements, using EORTC QLQ C-30, a standard questionnaire developed to assess quality of life in cancer patients particularly suffering from severe fatigue and anemia, substantially improved during treatment. In this trial, eculizumab appeared safe and well tolerated.

"We have now successfully concluded extensive and very constructive discussions with the FDA that have defined a clear path forward for eculizumab in this severe orphan blood disease," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "We believe that, if successful, the TRIUMPH pivotal trial, together with support from SHEPHERD, should provide a solid basis to support a biological license application for the PNH indication. With a focus on meeting the needs of the severely underserved PNH patient population, we are committed to drive forward the efficient and timely execution of this important clinical program."

If approved, eculizumab would represent the first of a new class of anti-inflammatory therapeutics-terminal complement inhibitors-as well as the first drug available specifically for patients suffering from the rare blood disease, PNH. In December 2003, Alexion announced that it had received orphan drug status for eculizumab in PNH from both the FDA and the European Agency for the Evaluation of Medicinal Products.

PNH is a blood disorder characterized by the onset of severe anemia, chronic fatigue and intermittent episodes of dark colored urine, known as hemoglobinuria. PNH patients are also at increased risk of forming life-threatening blood clots, or thromboses, which are a leading cause of death (approximately 50%) in this disease. People with PNH have an acquired deficiency of proteins that normally protect red blood cells from a component of the body's natural defense system, known as the complement cascade. Lack of these complement inhibitor proteins leaves PNH red blood cells susceptible to destruction (hemolysis), which is manifest as a reduction in blood hemoglobin, causing patients to become anemic. In some cases, these patients are dependent on blood transfusions. Currently, physicians prescribe either steroids or other immunosuppressive drug therapy to help patients cope with the symptoms of anemia, as no drugs are currently approved to specifically treat PNH. Estimates suggest that up to 2,000—10,000 people in the U.S. suffer from this condition.

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 and cardiovascular disorders, autoimmune diseases and cancer. Alexion's two lead product candidates, pexelizumab and eculizumab, are currently undergoing evaluation in several clinical development programs. Alexion has completed a Phase III clinical study with pexelizumab in coronary artery bypass graft (CABG) surgery patients undergoing cardiopulmonary bypass (CPB), and two large Phase II studies with pexelizumab in acute myocardial infarction (AMI) patients. The Phase III trial and the Phase II trials were conducted in collaboration with Procter and Gamble Pharmaceuticals. Separately, under the Special Protocol Assessment process, the FDA has agreed to protocols for the pivotal trials of pexelizumab in CABG patients undergoing CPB and in AMI patients treated with primary percutaneous intervention that could, if successful, serve as the primary basis of review for approval of Biologics License Applications for those two indications. In addition to pexelizumab, eculizumab has completed a pilot clinical trial for the treatment of paroxysmal nocturnal hemoglobinuria (PNH). Under the Special Protocol Assessment process, the FDA has agreed to protocols for the two trials of eculizumab in PNH

patients that could, if successful, serve as the primary basis of review for approval of a Biologics License Application for eculizumab in the PNH indication. Eculizumab is also in Phase II clinical development in rheumatoid arthritis and membranous nephritis. 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 on the World Wide Web at: http://www.alexionpharm.com.

This news release contains forward-looking statements. Such statements are subject to factors that may cause Alexion's results and plans to differ from those expected, including the results of pre-clinical or clinical studies (including termination or delay in clinical programs), the need for additional research and testing, 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 Annual Report on Form 10-K for the year ended July 31, 2003 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 and other filings 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.

Contacts:

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**Alexion Pharmaceuticals Initiates Enrollment in Pivotal Phase III PRIMO-CABG-2 Trial**

- Enrollment also Initiated in Pivotal Phase III APEX-AMI Trial -

CHESHIRE, Conn., July 20 /PRNewswire-FirstCall/ — Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN) announced today that they and their collaboration partner for pexelizumab, Procter & Gamble Pharmaceuticals, Inc. (P&GP), have initiated patient enrollment for the pivotal Phase III PRIMO-CABG-2 trial in patients undergoing coronary artery bypass graft surgery. Alexion reported on June 14th that they, together with P&GP, had reached agreement with the FDA on the design for the PRIMO-CABG-2 study under the Special Protocol Assessment (SPA) process. It is expected that, if successful, this trial will complete the filing package that will serve as the primary basis of review for the approval of a Biologics License Application for the CABG indication.

The "Pexelizumab for Reduction of Infarction and MOrtality in Coronary Artery Bypass Graft surgery" (PRIMO-CABG-2) pivotal Phase III trial, will examine the effects of pexelizumab on the composite endpoint of death or myocardial infarction at 30 days post procedure in moderate to high risk CABG surgery patients with or without concomitant valve surgery during cardiopulmonary bypass. The study is expected to enroll approximately 4,000 patients in North America and Europe over the next 12-15 months. PRIMO-CABG-2 represents the second Phase III clinical trial conducted in CABG patients. Alexion and P&GP are jointly executing the trial.

In addition, enrollment was initiated in the pivotal Phase III APEX-AMI trial in patients experiencing acute myocardial infarction treated with primary percutaneous intervention (PCI). The "Assessment of PEXelizumab in Acute Myocardial Infarction" (APEX-AMI) pivotal Phase III trial, will examine the effects of pexelizumab on death at 90 days post procedure in patients undergoing percutaneous intervention for acute myocardial infarction. The study is expected to enroll approximately 8,500 patients in North America, Europe, Australia and New Zealand over the next 24-36 months. APEX-AMI represents the first Phase III trial of pexelizumab in patients experiencing acute myocardial infarction. Initiation of this study triggers a milestone payment to Alexion from P&GP. Alexion and P&GP are jointly executing the trial.

"Having completed the SPA process, we are pleased to be moving quickly to the enrollment of patients in these studies," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "Of particular importance is the initiation of the PRIMO-CABG-2 study, which we will strive to complete in the second half of 2005 and which is designed to provide data which, if positive, will provide a solid basis to support a biological license application for the CABG indication."

If approved, pexelizumab would represent the first of a new class of anti-inflammatory therapeutics (terminal complement inhibitors) for patients undergoing CABG surgery and for patients undergoing PCI for acute myocardial infarction.

Alexion is engaged in the discovery and development of therapeutic products aimed at treating patients with a wide array of severe disease states, including cardiovascular and autoimmune disorders, inflammation and cancer. Alexion's two lead product candidates, pexelizumab and eculizumab, are currently undergoing evaluation in several clinical development programs. Alexion has completed a Phase III clinical study with pexelizumab in coronary artery bypass graft (CABG) surgery patients

undergoing cardiopulmonary bypass (CPB), and two large Phase II studies with pexelizumab in acute myocardial infarction (AMI) patients. The Phase III trial and the Phase II trials were conducted in collaboration with Procter and Gamble Pharmaceuticals. Separately, under the Special Protocol Assessment process, the FDA has agreed to protocols for the Phase III pivotal trials of pexelizumab in CABG patients undergoing CPB and in AMI patients treated with primary percutaneous intervention. In addition to pexelizumab, eculizumab has completed a pilot clinical trial for the treatment of paroxysmal nocturnal hemoglobinuria (PNH). Under the Special Protocol Assessment process, the FDA has agreed to protocols for the two trials constituting the pivotal Phase III program of eculizumab in PNH. Eculizumab is also in Phase II clinical development in rheumatoid arthritis and membranous nephritis. 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 on the World Wide Web at: http://www.alexionpharm.com.

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