



## **Alexion Joins NORD, EURORDIS and Other Patient Advocacy Groups in Supporting the Goals of Rare Disease Day 2010**

### **- Global Day of Recognition Underscores Need to Develop, and Provide Access to, Orphan Therapies -**

CHESHIRE, Conn. & SMITHFIELD, R.I., Feb 26, 2010 (BUSINESS WIRE) -- Alexion Pharmaceuticals, Inc., (Nasdaq: ALXN) has joined the National Organization for Rare Disorders (NORD) and the European Organization for Rare Diseases (EURORDIS) in supporting the goals of the third annual Rare Disease Day, February 28, 2010.

Rare Disease Day, established by EURORDIS in 2008, seeks to increase the visibility of rare diseases, give hope and information to patients, coordinate policy actions in different countries, and ensure equal access to quality care and treatment. More information on Rare Disease Day is available at [www.rarediseaseday.org](http://www.rarediseaseday.org).

"Rare Disease Day enables the rare disease community to raise awareness about the clinical needs and personal struggles faced by these patients and families. Our goal is to highlight the importance of ongoing therapeutic breakthroughs and explain the need to facilitate access to medicines once they become available," said Peter Saltonstall, President of NORD.

This year's Rare Disease Day theme, "Bridging Patients and Researchers," highlights the importance of collaboration between the clinical, advocacy, and patient communities for the discovery and development of rare disease treatments. Activities held worldwide marking this year's Rare Disease Day include educational events, podcasts and social networking to help patients around the world to share their struggles, stories and hopes for future treatments.

"The theme of this year's Rare Disease Day is especially meaningful to Alexion," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "Through our focus on developing and delivering treatments for patients with ultra-rare disorders, we know first-hand the possibilities for transforming patients' lives through medical innovation. We are continuing to bring Soliris to patients with PNH around the world while developing treatments for patients with other ultra-rare, debilitating and life-threatening disorders."

Alexion is the developer of Soliris(R) (eculizumab), the first treatment approved in the U.S., European Union and other countries as a treatment for all patients with paroxysmal nocturnal hemoglobinuria (PNH), a severe, debilitating and life-threatening blood disorder defined by hemolysis (red blood cell destruction). Among other research programs, Alexion is currently investigating the potential of Soliris as a treatment for patients with atypical Hemolytic Uremic Syndrome (aHUS), an ultra-rare and life-threatening genetic disorder, and acute humoral rejection in kidney transplant patients. The Company is recognizing Rare Disease Day with events at its international facilities.

Information about NORD can be found at [www.rarediseases.org](http://www.rarediseases.org), and information about Rare Disease Day activities in the U.S. are available at [www.rarediseaseday.us](http://www.rarediseaseday.us).

### **About PNH**

PNH is a rare blood disorder that strikes people of all ages, with an average age of onset in the early 30s. (1) Approximately 10 percent of all patients first develop symptoms at 21 years of age or younger. (2) PNH develops without warning and can occur in men and women of all races, backgrounds and ages. PNH often goes unrecognized, with delays in diagnosis ranging from one to more than 10 years. (3) The estimated median survival for PNH patients is between 10 and 15 years from the time of diagnosis. (1,3) PNH has been identified more commonly among patients with disorders of the bone marrow, including aplastic anemia (AA) and myelodysplastic syndromes (MDS). (4,5,6) In patients with thrombosis of unknown origin, PNH may be an underlying cause. (1) More information on PNH is available at [www.pnhsource.com](http://www.pnhsource.com).

### **About Soliris**

Soliris has been approved by the U.S. Food and Drug Administration (FDA), the European Commission (EC) and healthcare authorities in other countries as the first treatment for all patients with PNH, an ultra-rare, debilitating and life-threatening blood disorder defined by hemolysis, or the destruction of red blood cells. The FDA and EC reviewed and approved their respective marketing applications for Soliris under their priority review or accelerated assessment procedures. In patients with PNH, hemolysis can cause life-threatening thromboses, recurrent pain, kidney disease, fatigue, impaired quality of life, anemia, pulmonary hypertension, shortness of breath and intermittent episodes of dark-colored urine (hemoglobinuria). Soliris is the only treatment that blocks this hemolysis. Prior to these approvals, there were no therapies specifically available for the

treatment of patients with PNH. PNH treatment was limited to symptom management through periodic blood transfusions, non-specific immunosuppressive therapy and, infrequently, bone marrow transplantations -- a procedure that carries its own substantial risks of mortality and morbidity. (2,8) Alexion is committed to the objective that every patient with PNH who can benefit from Soliris will have access to Soliris. More information on Soliris is available at [www.Soliris.net](http://www.Soliris.net).

### **Important Safety Information**

Soliris is generally well tolerated. The most frequent adverse events observed in clinical studies were headache, nasopharyngitis (a runny nose), back pain and nausea. Treatment with Soliris should not alter anticoagulant management because the effect of withdrawal of anticoagulant therapy during Soliris treatment has not been established.

The product labels for Soliris in the U.S., Canada and Australia also include a boxed warning: "Soliris increases the risk of meningococcal infections. Vaccinate patients with a meningococcal vaccine at least two weeks prior to receiving the first dose of Soliris; revaccinate according to current medical guidelines for vaccine use. Monitor patients for early signs of meningococcal infections, evaluate immediately if infection is suspected, and treat with antibiotics if necessary." During clinical studies, two out of 196 vaccinated PNH patients treated with Soliris experienced a serious meningococcal infection. In the European Union, the product label for Soliris includes special warning statements that include: "Due to its mechanism of action, the use of Soliris increases the patient's susceptibility to meningococcal infections (*Neisseria meningitidis*)." "To reduce the risk of infection, all patients must be vaccinated at least 2 weeks prior to receiving the first dose of Soliris and must be revaccinated according to current medical guidelines for vaccine use." And, "All patients should be monitored for early signs of meningococcal infections, evaluated immediately if infection is suspected, and treated with antibiotics if necessary. Patients should be informed of these signs and symptoms and steps taken to seek medical care immediately."

Prior to beginning Soliris therapy, all patients and their prescribing physicians are enrolled in the Soliris Safety Registry, which is part of a special risk-management program that involves initial and continuing education and long-term monitoring for detection of new safety findings.

### **About Alexion**

Alexion Pharmaceuticals, Inc. is a biopharmaceutical company working to develop and commercialize life-changing drug therapies for patients with serious and life-threatening medical conditions. Alexion is engaged in the discovery, development and commercialization of therapeutic products aimed at treating patients with a wide array of severe disease states, including hematologic, kidney and neurologic diseases, transplant rejection, cancer and autoimmune disorders. Soliris is Alexion's first marketed product, approved in the U.S., European Union and other countries as a treatment for all patients with PNH. Alexion is evaluating other potential indications for Soliris, and is pursuing development of other antibody product candidates in early stages of development. Further information about Alexion Pharmaceuticals, Inc. can be found at [www.alexionpharma.com](http://www.alexionpharma.com).

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### **Safe Harbor Statement**

This news release contains forward-looking statements, including statements related to potential health and medical benefits from Soliris. Forward-looking statements are subject to factors that may cause Alexion's results and plans to differ from those expected, including for example, decisions of regulatory authorities regarding marketing approval or material limitations on the marketing of Soliris, the possibility that initial results of commercialization are not predictive of future rates of adoption of Soliris, 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 period ended December 31, 2009, and in Alexion's 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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