



Alexion Joins NORD, EURORDIS and Other Patient Advocacy Groups in Supporting the Goals of Rare Disease Day 2011

Global Event Aims to Improve Diagnosis, Treatment, Access and Support for Patients and Families Facing Rare, Devastating Diseases

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN) joins the European Organization for Rare Diseases (EURORDIS), the National Organization for Rare Disorders (NORD) and families, governments, companies and medical communities around the world in observing the fourth annual Rare Disease Day today.

In the U.S., a rare disease is defined as one that affects fewer than 200,000 patients. Many diseases are considered ultra-rare, generally thought of as affecting fewer than 20 patients per one million of the population, or approximately 6,000 or fewer patients in the U.S. The goal of Rare Disease Day is to draw attention to these diseases as an important public health issue with unique challenges. This year's theme, "rare but equal," spotlights the healthcare inequalities that often exist with rare diseases. Specific objectives include equal access to health care and social services; equal access to orphan drugs and treatments; and equal access to basic social rights such as education and employment.

"Challenges faced by patients with rare diseases include delayed diagnosis, few treatment options, and difficulty finding medical experts," said Peter L. Saltonstall, President and CEO of NORD. "On Rare Disease Day 2011, millions of patients and their families will share their stories to shine a light on rare diseases and help educate and inform the public."

As a company dedicated to developing innovative therapies for patients with rare, devastating disorders, Alexion shares the mission of Rare Disease Day to elevate research, hope and equality for patients. Alexion discovered and developed Soliris[®] (eculizumab), the first treatment for patients with paroxysmal nocturnal hemoglobinuria (PNH), an ultra-rare, debilitating, and life-threatening blood disorder. Today, the company is investigating the potential of Soliris in other rare and ultra-rare disorders, including atypical Hemolytic Uremic Syndrome (aHUS), an ultra-rare genetic disorder, and acute humoral rejection (AHR) in patients undergoing kidney transplant.

"Rare Disease Day brings much-needed attention to the efforts that many advocates, physicians, policymakers and companies are engaged in every day," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "We are gratified to see how far we've come in changing the landscape of how PNH is diagnosed, treated and managed. But much work remains to help patients suffering from rare diseases, and we are accelerating our programs to develop life-transforming therapies for other rare and severe disorders."

Rare Disease Day 2011 coincides with The NASDAQ Stock Market's recognition of Alexion's 15th anniversary as a listed company at this morning's Market Open ceremony. With the goal of breaking new ground in rare diseases, Alexion recently established a translational medicine group in Cambridge, Massachusetts to accelerate the development of an expanded portfolio of innovative preclinical compounds. One such compound is an investigational treatment for newborn children with molybdenum cofactor deficiency (MoCD) Type A, a catastrophic, ultra-rare genetic disorder that strikes newborns and currently has no treatment options.

To learn more about Alexion's research and development programs, access initiatives, and mission and vision, visit our Website at www.alexionpharma.com. To learn more about Rare Disease Day, visit www.rarediseaseday.us for U.S. activities and www.rarediseaseday.org for global activities.

About PNH

PNH is an ultra-rare blood disorder in which chronic uncontrolled activation of complement, a component of the normal immune system, results in hemolysis (destruction of the patient's red blood cells). PNH strikes people of all ages, with an average age of onset in the early 30s. Approximately 10 percent of all patients first develop symptoms at 21 years of age or younger. 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. It is estimated that approximately one-third of patients with PNH do not survive more than five years from the time of diagnosis. PNH has been identified more commonly among patients with disorders of the bone marrow, including aplastic anemia (AA) and myelodysplastic syndromes (MDS). In patients with thrombosis of unknown origin, PNH may be an underlying cause. More information on PNH is available at www.pnhsource.com.

About Soliris

Soliris is a first-in-class terminal complement inhibitor developed from the laboratory through regulatory approval and commercialization by Alexion. Soliris has been approved in the U.S., European Union, Japan and other territories as the first treatment for patients with PNH, an ultra-rare, debilitating and life-threatening blood disorder defined by chronic uncontrolled complement activation which causes chronic hemolysis. Prior to these approvals, there were no therapies specifically available for the treatment of patients with PNH. Eculizumab (Soliris) is not approved for the treatment of aHUS, transplant or other indications other than PNH. Alexion's innovative approach to complement inhibition has received some of the pharmaceutical industry's highest honors: the 2008 Prix Galien USA Award for Best Biotechnology Product with broad implications for future biomedical research and the 2009 Prix Galien France Award in the category of Drugs for Rare Diseases. More information on Soliris is available at www.soliris.net.

Important Safety Information

Soliris is generally well tolerated in patients with PNH. The most frequent adverse events observed in clinical studies of patients with PNH were headache, nasopharyngitis (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 U.S. product label for Soliris also includes a boxed warning: "Soliris increases the risk of meningococcal infections. Meningococcal infection may become rapidly life-threatening or fatal if not recognized and treated early. 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 PNH clinical studies, two out of 196 vaccinated PNH patients treated with Soliris experienced a serious meningococcal infection. Prior to beginning Soliris therapy, all patients and their prescribing physicians are encouraged to enroll in the PNH 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 deliver 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 and kidney diseases, neurologic disorders, ophthalmic, transplant, other inflammatory disorders, and cancer. Soliris® (eculizumab), Alexion's first marketed product, is approved in more than 35 countries as a therapy for patients with PNH, a debilitating and ultra-rare life-threatening blood disorder. Alexion is evaluating other potential indications for Soliris and is pursuing development of other innovative biotechnology product candidates in early stages of development. This press release and further information about Alexion Pharmaceuticals, Inc. can be found at: 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 of Soliris (eculizumab) for the treatment of patients with PNH, and Alexion's plans for developing other therapies and for developing Soliris for other disorders. 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 for its current or potential new indications, 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-K for the period ended December 31, 2010, 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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