



Alexion Joins Eurordis, NORD and Patient Organizations Worldwide in Celebrating Rare Disease Day 2013

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-- Global Effort to Raise Awareness and Improve Diagnosis and Treatment of Rare Disorders --

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 patient organizations worldwide in celebrating Rare Disease Day 2013, a global effort to focus attention on rare diseases, their profound impact on patients, and the need for improved diagnosis and treatment. The theme of this year's celebration, "Rare Disorders without Borders," aligns with Alexion's mission of developing and delivering life-transforming therapies for patients worldwide who suffer from severe, life-threatening diseases that are ultra-rare.

"On Rare Disease Day, we are breaking isolation and raising awareness. Patients worldwide are not alone. We urge all stakeholders to reach across borders and find common solutions to living with serious, chronic and life-threatening rare diseases," said Yann Le Cam, Chief Executive Officer, EURORDIS. "Working together we can promote rare diseases as a public health priority, so to improve patients' access to diagnosis and treatment."

Many rare and ultra-rare diseases are chronic, progressive and marked by continuing pain, severe disability and high mortality rates. Diagnosing and managing these rare diseases is often made difficult by a lack of scientific knowledge, research and medical innovation. Few physicians are familiar with diagnosing and treating these illnesses, which frequently leads to missed, delayed or inaccurate diagnoses even when an approved, effective therapy is available.¹ Because of this, it is important to educate the medical community through disease awareness programs and diagnostic initiatives to identify patients suffering from rare and ultra-rare diseases as early as possible.

"Like many patients coping with a rare or ultra-rare disease, it took several months for our daughter to get an accurate diagnosis," said Denise Schmidt, mother of a young adult diagnosed with atypical hemolytic uremic syndrome (aHUS), a chronic, ultra-rare and life-threatening disease that can progressively damage vital organs. "Increasing awareness among physicians and patients is a vital first step to ensuring our loved ones receive the best treatment and care."

Alexion developed Soliris[®] (eculizumab), a first-in-class terminal complement inhibitor, from the laboratory through regulatory approval and commercialization. Soliris is approved in the US, European Union, Japan and other countries as the first and only treatment for patients with paroxysmal nocturnal hemoglobinuria (PNH), a debilitating, life-threatening and ultra-rare blood disorder. Soliris is also approved in the US and the European Union as the first and only treatment for patients with atypical hemolytic uremic syndrome (aHUS), a debilitating and life-threatening ultra-rare genetic disorder.

"We understand that every day is Rare Disease Day for patients and families who suffer from severe and life-threatening ultra-rare disorders and often live without hope because an effective treatment option is not available," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "The employees of Alexion are committed to developing and delivering therapies that can transform the lives of these patients. We now serve patients in 50 countries by focusing on disease education to help patients with PNH and aHUS receive an accurate diagnosis and appropriate treatment. At the same time, we continue to invest in research and development with the goal of providing highly innovative therapies to patients with additional severe and life-threatening disorders, which also happen to be extremely rare."

Bringing Hope Across the Globe

Alexion is currently developing five highly innovative therapeutics, including eculizumab (Soliris[®]), which are being investigated in nine severe and life-threatening ultra-rare disorders. The company's development programs are solely focused on:

- Severe disorders with devastating and life-threatening medical consequences
- Disorders with ineffective, or no treatment options
- Disorders that are ultra-rare and affect very small numbers of patients

To learn more about Rare Disease Day, visit www.rarediseaseday.us for U.S. activities and www.rarediseaseday.org for global activities.

About Rare and Ultra-Rare Disorders

In the United States, a disease is defined as rare if it affects fewer than 650 patients per million of population.² The European Union definition of a rare disease is one that affects fewer than five patients per 10,000 of population.³ In contrast, a disease is generally considered to be ultra-rare if it affects fewer than 20 patients per million of population⁴ (one patient per 50,000) – and most ultra-rare diseases affect far fewer people than this.

Despite the very small numbers of patients they affect, the impact of these rare and ultra-rare diseases on patients, their families, and society is profound, as many are severe, chronic and progressive, with high mortality rates. Patients with severe and life-threatening ultra-rare diseases often live without hope, have no effective treatment options and may face premature death.

About aHUS

aHUS is a chronic, ultra-rare, and life-threatening disease in which a genetic deficiency in one or more complement regulatory genes causes chronic uncontrolled complement activation, resulting in complement-mediated thrombotic microangiopathy (TMA), the formation of blood clots in small blood

vessels throughout the body.^{5,6} Permanent, uncontrolled complement activation in aHUS causes a life-long risk for TMA, which leads to sudden, catastrophic, and life-threatening damage to the kidney, brain, heart, and other vital organs, and premature death.^{5,7} Sixty-five percent of all patients with aHUS require kidney dialysis, have permanent kidney damage or die within the first year after diagnosis despite plasma exchange or plasma infusion (PE/PI).^{8,9} The majority of patients with aHUS who receive a kidney transplant commonly experience subsequent systemic TMA, resulting in a 90% transplant failure rate in these TMA patients.¹⁰

aHUS affects both children and adults.¹¹ Complement-mediated TMA also causes reduction in platelet count (thrombocytopenia) and red blood cell destruction (hemolysis). While mutations have been identified in at least ten different complement regulatory genes, mutations are not identified in 30-50% of patients with a confirmed diagnosis of aHUS.¹¹

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.¹² Approximately 10% of all patients first develop symptoms at 21 years of age or younger.¹³ 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.¹⁴ In the period of time before Soliris was available, it had been estimated that approximately one-third of patients with PNH did not survive more than five years from the time of diagnosis.¹² PNH has been identified more commonly among patients with disorders of the bone marrow, including aplastic anemia (AA) and myelodysplastic syndromes (MDS).¹⁵⁻¹⁷ In patients with thrombosis of unknown origin, PNH may be an underlying cause.¹²

About Soliris

Soliris is a first-in-class terminal complement inhibitor developed from the laboratory through regulatory approval and commercialization by Alexion. Soliris is approved in the U.S., European Union, Japan and other countries as the first and only treatment for patients with paroxysmal nocturnal hemoglobinuria (PNH), a debilitating, ultra-rare and life-threatening blood disorder, characterized by complement-mediated hemolysis (destruction of red blood cells). Soliris is indicated to reduce hemolysis.

Soliris is also approved in the U.S. and the European Union as the first and only treatment for patients with atypical hemolytic uremic syndrome (aHUS), a debilitating, ultra-rare and life-threatening genetic disorder characterized by complement-mediated thrombotic microangiopathy, or TMA (blood clots in small vessels). Soliris is indicated to inhibit complement-mediated TMA. The effectiveness of Soliris in aHUS is based on the effects on TMA and renal function. Prospective clinical trials in additional patients are ongoing to confirm the benefit of Soliris in patients with aHUS. Soliris is not indicated for the treatment of patients with Shiga toxin *E. coli* related hemolytic uremic syndrome (STEC-HUS).

Alexion's breakthrough approach in complement inhibition has received 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, including the full prescribing information on Soliris, is available at www.soliris.net.

Important Safety Information

The US product label for Soliris includes a boxed warning: "Life-threatening and fatal meningococcal infections have occurred in patients treated with Soliris. Meningococcal infection may become rapidly life-threatening or fatal if not recognized and treated early. Comply with the most current Advisory Committee on Immunization Practices (ACIP) recommendations for meningococcal vaccination in patients with complement deficiencies. Immunize patients with a meningococcal vaccine at least 2 weeks prior to administering the first dose of Soliris, unless the risks of delaying Soliris therapy outweigh the risk of developing a meningococcal infection. (See Serious Meningococcal Infections (5.1) for additional guidance on the management of meningococcal infection.) Monitor patients for early signs of meningococcal infections and evaluate immediately if infection is suspected. Soliris is available only through a restricted program under a Risk Evaluation and Mitigation Strategy (REMS). Under the Soliris REMS, prescribers must enroll in the program (5.2). Enrollment in the Soliris REMS program and additional information are available by telephone: 1-888-soliris (1-888-765-4747)."

In patients with PNH, the most frequently reported adverse events observed with Soliris treatment in clinical studies were headache, nasopharyngitis (runny nose), back pain and nausea. Soliris treatment of patients with PNH should not alter anticoagulant management because the effect of withdrawal of anticoagulant therapy during Soliris treatment has not been established. In patients with aHUS, the most frequently reported adverse events observed with Soliris treatment in clinical studies were hypertension, upper respiratory tract infection, diarrhea, headache, anemia, vomiting, nausea, urinary tract infection, and leukopenia. Please see full prescribing information for Soliris, including boxed WARNING regarding risk of serious meningococcal infection.

About Alexion

Alexion Pharmaceuticals, Inc. is a biopharmaceutical company focused on serving patients with severe and ultra-rare disorders through the innovation, development and commercialization of life-transforming therapeutic products. Alexion is the global leader in complement inhibition and has developed and markets Soliris® (eculizumab) as a treatment for patients with PNH and aHUS, two debilitating, ultra-rare and life-threatening disorders caused by chronic uncontrolled complement activation. Soliris is currently approved in more than 40 countries for the treatment of PNH, and in the United States and the European Union for the treatment of aHUS. Alexion is evaluating other potential indications for Soliris and is developing four other highly innovative biotechnology product candidates, which are being investigated across nine severe and ultra-rare disorders beyond PNH and aHUS. This press release and further information about Alexion Pharmaceuticals, Inc. can be found at: www.alexionpharma.com.

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