Alexion Joins EURORDIS, NORD and Patient Organizations Worldwide in Celebrating Rare Disease Day 2012

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 groups, families, governments, medical communities and industry in celebrating Rare Disease Day, a global effort to raise awareness of rare diseases and their profound impact on patients' lives.

Many rare and ultra-rare diseases are chronic, progressive and marked by continuing pain, severe disability and high mortality rates. The severity of rare diseases is often compounded by a lack of scientific knowledge, research and medical innovation, leading to delayed diagnoses, misdiagnoses and sub-optimal treatment.

"Too often, patients and families coping with a rare disease feel isolated and lack information, support programs, access to medical experts and effective therapies," said Deborah Sittig, Founder of Soft Bones, the U.S. Hypophosphatasia Foundation. "As the mother of a child with a severe, ultra-rare metabolic disorder and the founder of a patient organization, I have experienced the benefits of joining together with families, physicians and industry to improve the care of these often underserved patients."

"Rare Disease Day brings much-needed attention to rare diseases as an important public health issue with unique challenges," said Yann Le Cam, Chief Executive Officer, EURORDIS. "This year's Rare Disease Day celebration calls for solidarity in meeting the challenges faced by many patients with rare diseases, including difficulty obtaining an accurate diagnosis, limited scientific information, and limited treatment options."

Helping More Patients With Ultra-Rare Disorders

The goals of Rare Disease Day align with Alexion's mission to develop innovative, life-transforming therapies for patients with ultra-rare and severe disorders for which there are few, if any, treatment options. 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, and the first treatment for atypical hemolytic uremic syndrome (aHUS), an ultra-rare, debilitating, and life-threatening genetic disorder.

Alexion is committed to serving an expanding number of patients with PNH, aHUS and other ultra-rare disorders worldwide. The Company is evaluating the benefits of Soliris in several other ultra-rare and life-threatening disorders, and is simultaneously developing other highly innovative drug candidates for the treatment of additional ultra-rare disorders. These include hypophosphatasia (HPP), a life-threatening, genetic metabolic disorder that leads to progressive damage to multiple vital organs, and molybdenum cofactor deficiency (MoCD) Type A, a devastating disorder that leads to severe brain damage and rapid death in newborns. There are no approved or effective therapies for HPP or MoCD Type A.

"We applaud EURORDIS, NORD and other patient advocacy groups for their continued collaboration to serve patients and families affected by severe and ultra-rare disorders," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "The employees of Alexion share this steadfast commitment to bringing hope to these patients and families, by developing and delivering innovative and effective new therapies."

To learn more about Alexion's research and development programs, and our commitment to patients with ultra-rare disorders, please 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.

Rare and Ultra-Rare Disorders

In the United States a rare disease is designated as rare if it affects fewer than approximately 650 patients per million of the population. In the European Union, a disease is designated as rare if it affects fewer than 500 patients per million of the population. An even rarer set of diseases are known as ultra-rare, typically designated when there are fewer than 20 patients per million of the population. Thus in the United States, while a very prevalent disease may affect millions of people, a rare disease will affect no more than 200,000 Americans and an ultra-rare disease will affect approximately 6,000 at most — and often far fewer.

About Paroxysmal Nocturnal Hemoglobinuria (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.[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] 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.[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.

About Atypical Hemolytic Uremic Syndrome (aHUS)

aHUS is a chronic, ultra-rare, and life-threatening disease in which a genetic deficiency in one or more complement regulatory genes causes life-long uncontrolled complement activation, resulting in complement-mediated thrombotic microangiopathy (TMA), the formation of blood clots in small blood vessels throughout the body.[7,8] 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. [8,9] More than half of all patients with aHUS die, require kidney dialysis or have permanent kidney damage within 1 year of diagnosis.[10] The majority of patients with aHUS who receive a kidney transplant commonly experience subsequent systemic TMA, resulting in a 90% transplant failure rate.[11]

aHUS affects both children and adults. In a large group of aHUS patients, 60% were first diagnosed at younger than 18 years of age.[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.[12]

About Hypophosphatasia (HPP)

HPP is an ultra-rare, genetic, and life-threatening metabolic disease characterized by defective bone mineralization and impaired phosphate and calcium regulation leading to progressive damage to multiple vital organs including destruction and deformity of bones, profound muscle weakness, seizures, impaired renal function, and respiratory failure.[13,14,15,16] The severe manifestations of the genetic deficiency in HPP affect people of all ages, and approximately 50 percent of infants with the disease do not survive past one year of age.[13]

HPP is caused by a genetic deficiency of an enzyme known as tissue non-specific alkaline phosphatase (TNSALP), which causes life-long abnormalities in metabolism of the two vital minerals calcium and phosphate, leading directly to the debilitating morbidities and premature mortality of the disease.[13] There are currently no therapies approved for HPP.[13]

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 US, 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 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, 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

Soliris is generally well tolerated in patients with PNH and aHUS. 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.

The U.S. product label for Soliris also 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)."

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 35 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. This press release and further information about Alexion Pharmaceuticals, Inc. can be found at: www.alexionpharma.com.

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