December 10, 2014

Soliris® (eculizumab) Granted Orphan Drug Designation in Japan for the Treatment of Patients with Myasthenia Gravis

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today announced that Soliris® (eculizumab) has been granted orphan drug designation (ODD) by Japan's Ministry of Health, Labour and Welfare (MHLW) for the treatment of patients with myasthenia gravis (MG), a rare, debilitating neurologic disorder. In patients with MG, uncontrolled complement activation due to antibodies directed at the neuromuscular junction can ultimately lead to profound and debilitating weakness of various muscle groups throughout the body.[1,2] This significant muscle weakness can impair patients' ability to walk, speak clearly, swallow and, in some cases, to breathe.

"The orphan drug designation for eculizumab for MG highlights the significant need for an effective treatment option for patients in Japan who continue to suffer from the severe and debilitating symptoms of MG despite currently available therapies," said Martin Mackay, Ph.D., executive vice president and global head of R&D at Alexion. "By specifically inhibiting the terminal complement pathway, eculizumab has the potential to provide better treatment outcomes for patients with refractory generalized MG. We look forward to evaluating the clinical benefits of eculizumab in MG in our registration study, known as REGAIN, which is currently enrolling patients."

The MHLW, based on the opinion of the Pharmaceutical Affairs and Food Sanitation Council, grants orphan status to drugs and medical devices that treat serious diseases of high unmet medical need that affect fewer than 50,000 patients in Japan. ODD provides drug developers with certain benefits and incentives, including priority review for marketing authorization and a period of 10 years of market exclusivity if regulatory approval is received for the designated indication.

Alexion is enrolling patients in a multinational, placebo-controlled registration trial of eculizumab in patients with refractory generalized MG, known as the REGAIN (Eculizumab for **RE**fractory **G**ener**AI**zed Myasthe**N**ia Gravis) study. More information on this trial is available at www.clinicaltrials.gov under the identifier NCT01997229.

Soliris is a first-in-class terminal complement inhibitor and is currently approved in the United States, European Union, Japan and other countries for the treatment of patients with paroxysmal nocturnal hemoglobinuria (PNH) and atypical hemolytic uremic syndrome (aHUS), two debilitating, ultra-rare and life-threatening disorders caused by chronic uncontrolled complement activation. Soliris is not approved in any country for the treatment of MG. In 2014, Soliris was granted ODD in both the U.S. and EU for the treatment of MG.

About Myasthenia Gravis

Myasthenia gravis (MG) is a rare, debilitating neurologic disorder caused by auto-antibodies that recognize a specific target in the nerve-muscle junction, which results in life-long uncontrolled terminal complement activation causing tissue damage and interference with signaling between nerve and muscle fibers.[1,2] Patients with MG initially experience weakness in their ocular (eye) muscles, and the disease typically progresses to the more severe and generalized form to include weakness of head, trunk, limb and respiratory muscles. Symptoms can include drooping eyelid, weakness in the arms and legs, slurred speech, difficulty chewing or swallowing, and difficulty breathing, which could lead to a life-threatening myasthenic crisis.

About Soliris® (eculizumab)

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. (2007), European Union (2007), Japan (2010) and other countries as the first and only treatment for patients with paroxysmal nocturnal hemoglobinuria (PNH) to reduce hemolysis. PNH is 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 U.S. (2011), the European Union (2011), Japan (2013) and other countries as the first and only treatment for patients with atypical hemolytic uremic syndrome (aHUS) to inhibit complement-mediated thrombotic microangiopathy, or TMA (blood clots in small vessels). aHUS is a debilitating, ultra-rare and life-threatening genetic disorder characterized by complement-mediated TMA. Soliris is not indicated for the treatment of patients with Shiga-toxin *E. coli*-related hemolytic uremic syndrome (STEC-HUS). For the breakthrough medical innovation in complement inhibition, Alexion and Soliris have received some of the pharmaceutical industry's highest honors: the Prix Galien USA (2008, Best Biotechnology Product) and France (2009, Rare Disease Treatment).

More information including the full U.S. prescribing information on Soliris is available at www.soliris.net.

Important Safety Information

The U.S. 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 [see Warnings and Precautions (5.1)]. 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 two 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 Warnings and Precautions (5.1) for additional guidance on the management of the risk 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 [see Warnings and Precautions (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 headache, diarrhea, hypertension, upper respiratory infection, abdominal pain, vomiting, nasopharyngitis, anemia, cough, peripheral edema, nausea, urinary tract infections, pyrexia. Soliris is not indicated for the treatment of patients with Shiga-toxin *E. coli*-related hemolytic uremic syndrome (STEC-HUS). Please see full prescribing information for Soliris, including BOXED WARNING regarding risk of serious meningococcal infection.

About Alexion

Alexion is a biopharmaceutical company focused on serving patients with severe and 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 nearly 50 countries for the treatment of PNH, and in nearly 40 countries for the treatment of aHUS. Alexion is evaluating other potential indications for Soliris in additional severe and ultra-rare disorders beyond PNH and aHUS, and is developing other highly innovative biotechnology product candidates across multiple therapeutic areas. This press release and further information about Alexion can be found at www.alexionpharma.com.

[ALXN-G]

This news release contains forward-looking statements, including statements related to potential medical benefits of Soliris® (eculizumab) for the treatment of myasthenia gravis (MG). 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 MG, delays in arranging satisfactory manufacturing capabilities, the possibility that results of clinical trials are not predictive of safety and efficacy results of Soliris for MG in broader or different patient populations, decisions of regulatory authorities to require additional testing, the risk that estimates regarding the number of patients with MG and observations regarding the natural history of patients with MG are inaccurate, 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-Q for the period ended September 30, 2014. 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.

References

1. Conti-Fine BM, Milani M, Kaminski HJ. Myasthenia gravis: past, present, and future. J Clin Invest 2006;116(11):2843-54.

2. Tüzün E, Huda R, Christadoss P. Complement and cytokine based therapeutic strategies in myasthenia gravis. J Autoimmun 2011;37(2):136-43.

Alexion:
Media
Irving Adler, 203-271-8210
Executive Director, Corporate Communications
or
Kim Diamond, 203-439-9600
Senior Director, Corporate Communications

or
Investors
Elena Ridloff, CFA, 203-699-7722
Executive Director, Investor Relations

Source: Alexion Pharmaceuticals, Inc.