



European Commission Grants Orphan Drug Designation to Soliris® (eculizumab) for the Treatment of Patients with Myasthenia Gravis (MG)

August 1, 2014

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals (NASDAQ:ALXN) today announced that the European Commission has granted orphan drug designation (ODD) to Soliris® (eculizumab) for the treatment of patients with Myasthenia Gravis (MG), a rare, debilitating neurologic disorder caused by uncontrolled complement activation. 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.

"Patients with MG develop debilitating muscle weakness, impairing their ability to walk, speak clearly, swallow and, in some cases, to breathe normally, which could lead to a life-threatening myasthenic crisis," said Martin Mackay, Ph.D., Executive Vice President, Global Head of R&D at Alexion. "By specifically inhibiting the terminal complement pathway, which is believed to play a pivotal role in the pathophysiology of MG, we believe that eculizumab has the potential to help patients living with this devastating rare disorder."

Soliris is a first-in-class terminal complement inhibitor and is currently approved 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 to treat MG. Alexion is enrolling patients in a multinational, placebo-controlled registration trial of eculizumab in patients with refractory generalized MG. More information on this trial is available at www.clinicaltrials.gov under the identifier NCT01997229.

The European Commission grants orphan medicinal product status to provide incentives to develop medicinal products to treat, prevent or diagnose diseases or conditions that affect no more than five in 10,000 persons in the EU. The orphan medicinal product status designation would provide Alexion with certain benefits and incentives in the EU, including a period of market exclusivity if Soliris is ultimately approved for the designated indication.

About Myasthenia Gravis (MG)

Myasthenia Gravis 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 signalling 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

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), 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. (2011), the European Union (2011), Japan (2013) and other countries 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. Soliris is not indicated for the treatment of patients with Shiga-toxin *E. coli*-related hemolytic uremic syndrome (STEC-HUS). For the breakthrough innovation in complement inhibition, Alexion and Soliris have 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 U.S. prescribing information on Soliris is available at: http://soliris.net/sites/default/files/assets/soliris_pi.pdf.

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, including asfotase alfa, across multiple therapeutic areas. This press release and further information about Alexion 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 medical benefits of Soliris® (eculizumab) for the treatment of patients with 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 and establishing commercial infrastructure for Soliris for MG, 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, the risk that third party payors (including governmental agencies) will not reimburse for the use of Soliris for MG (if approved) at acceptable rates or at all, the risk that estimates regarding the number of patients with Soliris for MG and observations regarding the natural history of patients with Soliris for 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 June 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.

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