



March 22, 2017

Alexion Submits Application in Japan for Soliris® (Eculizumab) as a Potential Treatment for Patients with Refractory Generalized Myasthenia Gravis (gMG)

NEW HAVEN, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ: ALXN) announced today that it has submitted an application to Japan's Ministry of Labour and Welfare (MHLW) to extend the indication for Soliris® (eculizumab) as a potential treatment for patients with refractory generalized myasthenia gravis (gMG) who are anti-acetylcholine receptor (AChR) antibody-positive. The submission is supported by comprehensive data from the Phase 3 REGAIN study.

"We look forward to working with the Japanese health authorities to bring this potentially life-transforming treatment to patients in Japan who are suffering and in urgent need of an effective therapy," said Martin Mackay, Ph.D., Executive Vice President and Global Head of R&D at Alexion. "If approved, Soliris could be a critical step toward improving the lives of patients with refractory gMG."

Patients with AChR-positive refractory gMG represent an ultra-rare population. Despite existing treatment options for gMG, these patients continue to face severe complications, including difficulty walking, talking, swallowing, and breathing normally. Exacerbations of their disease may be life-threatening and require hospitalization and intensive care.

If approved, Soliris would be the first and only complement inhibitor for patients with refractory AChR-positive gMG in Japan. Alexion has also submitted marketing applications in the U.S. and EU to extend the indication for Soliris as a potential treatment for patients with refractory gMG who are AChR-positive, which have been accepted by the U.S. Food and Drug Administration (FDA) and validated by the European Medicines Agency (EMA). Soliris has received Orphan Drug Designation (ODD) for the treatment of patients with MG in the U.S. and EU, and for treatment of patients with refractory gMG in Japan. Soliris is not approved in any country for the treatment of patients with refractory AChR-positive gMG.

About Refractory Generalized Myasthenia Gravis¹⁻⁸

Refractory generalized myasthenia gravis (gMG) patients who are anti-acetylcholine receptor (AChR) antibody-positive represent an ultra-rare segment of patients with MG—a chronic, debilitating and progressive autoimmune neuromuscular disease where the complement system mediates a progressive, destructive inflammatory effect on the neuromuscular junction. Patients with refractory AChR-positive gMG experience severe morbidities and life-threatening complications despite currently available MG therapies.

MG typically begins with weakness in the ocular muscles and often progresses to the more severe and generalized form, known as gMG, to include weakness of the head, neck, trunk, limb and respiratory muscles. While most symptoms in patients with gMG are managed with conventional therapies, 10% to 15% of patients are considered refractory—meaning they do not respond to multiple conventional therapies and continue to suffer profound muscle weakness throughout the body that can result in slurred speech, impaired swallowing and choking, double vision, disabling fatigue, shortness of breath due to respiratory muscle weakness, immobility requiring assistance, frequent hospital and intensive care unit admissions with prolonged stays, and periods of respiratory failure.

In patients with AChR-positive MG, the body's own immune system turns on itself to produce antibodies against AChR, a receptor located on muscle cells in the neuromuscular junction (NMJ) and used by nerve cells to communicate with the muscles these nerves control. The binding of these antibodies to AChR activates the complement cascade which leads to the destruction of the NMJ. As a result, the communication between nerve and muscle is disrupted, which leads to a loss of normal muscle function.

Today, there are no approved therapies for the ultra-rare population of patients suffering from refractory AChR-positive gMG.

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), 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).

For more information on Soliris, please see full prescribing information for Soliris, including BOXED WARNING regarding risk of serious meningococcal infection, available at www.soliris.net.

Important Safety Information

The U.S. prescribing information for Soliris includes the following warnings and precautions: 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 Centers for Disease Control (CDC)'s Advisory Committee on Immunization Practices (ACIP) recommendations for meningococcal vaccination in patients with complement deficiencies. Immunize patients with meningococcal vaccines 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. 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. Enrollment in the Soliris REMS program and additional information are available by telephone: 1-888-SOLIRIS (1-888-765-4747) or at www.solirisrems.com.

Patients may have increased susceptibility to infections, especially with encapsulated bacteria. Aspergillus infections have occurred in immunocompromised and neutropenic patients. Children treated with Soliris may be at increased risk of developing serious infections due to *Streptococcus pneumoniae* and *Haemophilus influenzae* type b (Hib). 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. Administration of Soliris may result in infusion reactions, including anaphylaxis or other hypersensitivity reactions.

In patients with PNH, the most frequently reported adverse events observed with Soliris treatment in clinical studies were headache, nasopharyngitis, back pain and nausea. 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, and pyrexia.

About Alexion

Alexion is a global biopharmaceutical company focused on developing and delivering life-transforming therapies for patients with devastating and rare disorders. Alexion is the global leader in complement inhibition and has developed and commercialized the first and only approved complement inhibitor to treat patients with paroxysmal nocturnal hemoglobinuria (PNH) and atypical hemolytic uremic syndrome (aHUS), two life-threatening ultra-rare disorders. In addition, Alexion's metabolic franchise includes two highly innovative enzyme replacement therapies for patients with life-threatening and ultra-rare disorders, hypophosphatasia (HPP) and lysosomal acid lipase deficiency (LAL-D). Alexion is advancing its rare disease pipeline with highly innovative product candidates in multiple therapeutic areas. This press release and further information about Alexion can be found at: www.alexion.com.

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Forward-Looking Statement

This news release contains forward-looking statements, including statements related to the potential medical benefits of Soliris® (eculizumab) for the treatment of myasthenia gravis, and Alexion's future clinical, regulatory and commercial plans for Soliris for the treatment of myasthenia gravis. 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 the adequacy of our research, marketing approval or material limitations on the marketing of our products, delays, interruptions or failures in the manufacture and supply of our products and our product candidates, failure to satisfactorily address matters raised by the FDA and other regulatory agencies, the possibility that results of clinical trials are not predictive of safety and efficacy results of our products in broader patient populations, the possibility that current rates of adoption of Soliris in PNH, aHUS or other diseases are not sustained, the possibility that clinical trials of our product candidates could be delayed, the adequacy of our pharmacovigilance and drug safety reporting processes, the risk that third party payors (including governmental agencies) will not reimburse or continue to reimburse for the use of our products at acceptable rates or at all, risks regarding government investigations, including investigations of Alexion by the SEC and

DOJ, the risk that anticipated regulatory filings are delayed, the risk that estimates regarding the number of patients with PNH, aHUS, HPP and LAL-D are inaccurate, the risks of shifting foreign exchange rates, and a variety of other risks set forth from time to time in Alexion's filings with the U.S. Securities and Exchange Commission, including but not limited to the risks discussed in Alexion's Annual Report on Form 10-K for the period ended December 31, 2016 and in our other filings with the U.S. 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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