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Alexion Submits U.S. and EU Applications Seeking Approval of Soliris® (Eculizumab) as a Treatment for Patients with Refractory Generalized Myasthenia Gravis (gMG)

NEW HAVEN, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today announced that the Company has submitted marketing applications to the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) to extend the indication for Soliris® (eculizumab) as a treatment for patients with refractory generalized myasthenia gravis (gMG) who are anti-acetylcholine receptor (AChR) antibody-positive. The European submission has been validated by the European Medicines Agency (EMA), marking the beginning of the review process in Europe for this potential new indication for Soliris. Both the U.S. and EU submissions are supported by the comprehensive data from the Phase 3 REGAIN study.

If approved, Soliris would address a significant unmet need for patients with refractory gMG who have largely exhausted conventional therapy. Refractory gMG patients who are anti-AChR antibody-positive are an ultra-rare segment of MG, a debilitating, complement-mediated neuromuscular disease in which patients suffer profound muscle weakness throughout the body, resulting in slurred speech, impaired swallowing and choking, double vision, disabling fatigue, shortness of breath due to respiratory muscle weakness, frequent hospital and ICU visits with prolonged stays, and episodes of respiratory failure.[1-6]

"Today there is an urgent need among patients suffering with refractory gMG, as there are no effective therapies for this ultra-rare and devastating disease population, causing patients to face disabling limitations in their daily lives, including difficulty walking, talking, swallowing, and breathing normally," said Martin Mackay, Ph.D., Executive Vice President and Global Head of R&D at Alexion. "The U.S. and EU regulatory submissions put us one step closer to accomplishing our goal of transforming the lives of patients suffering with refractory gMG with anti-AChR antibodies. We look forward to working with regulatory authorities as they review our applications."

Soliris has received Orphan Drug Designation (ODD) for the treatment of patients with MG in the U.S. and EU. Soliris is not approved in any country for the treatment of patients with refractory gMG.

About Refractory Generalized Myasthenia Gravis

Refractory generalized myasthenia gravis (gMG) patients who are anti-acetylcholine receptor (AChR) antibody-positive represent an ultra-rare segment of patients with MG—a debilitating, complement-mediated neuromuscular disease—who experience severe morbidities despite currently available MG therapies.[1,2,3]

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.[7] While most symptoms in gMG patients 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, resulting in slurred speech, impaired swallowing and choking, double vision, disabling fatigue, shortness of breath due to respiratory muscle weakness, frequent hospital and ICU visits with prolonged stays, and episodes of respiratory failure.[4,5,6,8]

Today, there are no therapies that are effective in this ultra-rare population of patients suffering from refractory 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).

More information on Soliris, including the full U.S. prescribing information, 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) or at www.solirisrems.com."

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, and 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 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 commercializes 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 the most robust rare disease pipeline in the biotech industry 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 Statements

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 materially differ from those expected, including for example, decisions of regulatory authorities regarding 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, progress in establishing and developing commercial infrastructure, 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 in the disease studied or other diseases, the risk that strategic transactions will not result in short-term or long-term benefits, the possibility that current results of commercialization are not predictive of future rates of adoption of Soliris in PNH, aHUS or other diseases, the possibility that clinical trials of our product candidates could be delayed or that additional research and testing is required by regulatory agencies, 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 Quarterly Report on Form 10-Q for the period ended September 30, 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.

References

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