European Commission Grants New Indication for Soliris® (Eculizumab) for the Treatment of Patients with Refractory Generalized Myasthenia Gravis (gMG)

- First and Only Complement-based Therapy Approved for an Ultra-rare Subset of gMG -

NEW HAVEN, Conn.—August 21, 2017, -- Alexion Pharmaceuticals, Inc. (NASDAQ: ALXN) announced today that the European Commission (EC) approved the extension of the indication for Soliris® (eculizumab) to include the treatment of refractory generalized myasthenia gravis (gMG) in adults who are anti-acetylcholine receptor (AChR) antibody-positive. Soliris is the first and only complement-based therapy approved in the European Union (EU) for this ultra-rare subset of patients.[1-4] Patients with refractory gMG can have difficulties walking, talking, swallowing and breathing normally despite therapies currently used for MG. Exacerbations and crises of their disease may require hospitalization and intensive care and may be life-threatening.[5-7] Soliris will be launched for this new indication initially in Germany, and Alexion is evaluating launches in additional EU countries.

"Patients with refractory gMG have exhausted multiple therapies and continue to suffer from severe symptoms and complications that markedly impact their daily lives," said Renato Mantegazza, MD, from the Department of Neuroimmunology and Neuromuscular Diseases, at the Istituto Neurologico Carlo Besta in Milan, Italy, and an investigator in the Phase 3 REGAIN study. "There is an urgent need for therapy for these patients, and it's exciting to have a product such as Soliris available that has demonstrated in clinical studies that it improves patients' symptoms and their ability to undertake daily activities."

Chronic uncontrolled activation of the complement cascade, a part of the immune system, can play a major role in the debilitating symptoms and potentially life-threatening complications of refractory gMG.[8-10] Soliris is a first-in-class complement inhibitor that specifically and effectively inhibits the terminal part of the complement cascade.

"Our deep understanding of complement-mediated diseases enabled us to develop Soliris for the treatment of patients with refractory gMG," said John Orloff, M.D., Executive Vice President and Head of Research & Development at Alexion. "We are grateful to the investigators and patients who participated in our clinical program, and we are excited about the opportunity to bring Soliris to patients who continue to suffer from this debilitating disease despite current therapies."

The EC based its approval of the extended indication for Soliris on comprehensive clinical data from the Phase 3 REGAIN study (MG-301) and its long-term open-label extension study (MG-302).

Alexion's supplemental Biologics License Application (sBLA) in the U.S. and a supplemental new drug application in Japan for Soliris as a treatment for patients with anti-AChR antibody-positive refractory gMG have been accepted for review by the U.S. Food and Drug Administration (FDA) and the Japanese Ministry of Health, Labour and Welfare (MHLW), respectively. Soliris has received Orphan Drug

Designation (ODD) for the treatment of patients with MG in the U.S. and EU, and for the treatment of patients with refractory gMG in Japan.

About Refractory Generalized Myasthenia Gravis

Patients with refractory generalized myasthenia gravis (gMG) who are anti-acetylcholine receptor (AChR) antibody-positive represent an ultra-rare subset of MG patients[1-4] who continue to suffer from severe disease symptoms and complications despite therapies currently used for MG.[1-2,11]

MG is a debilitating, chronic and progressive autoimmune neuromuscular disease that can occur at any age but most commonly begins for women before the age of 40 and men after the age of 60.[5,6,12,13] It typically begins with weakness in the muscles that control the movements of the eyeballs and eyelids, and often progresses to the more severe and generalized form, known as gMG with weakness of the head, neck, trunk, limb and respiratory muscles.[13]

While most symptoms in patients with gMG are managed with therapies for MG, 10% to 15% of patients are considered refractory—meaning they do not respond to multiple therapies for MG and continue to suffer profound muscle weakness, and severe disease symptoms that limit function.[1-2,11] Patients with refractory gMG can suffer from slurred speech; impaired swallowing; double or blurred vision; disabling fatigue; immobility requiring assistance; shortness of breath, and episodes of respiratory failure. Complications, exacerbations and myasthenic crises can require hospital and intensive care unit admissions with prolonged stays and can be life-threatening.[5-7]

In patients with anti-AChR antibody-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.[5,6] The binding of these antibodies to AChR activates the complement cascade, another part of the immune system, which leads to a localized destruction of the NMJ. As a result, the communication between nerve and muscle is impaired, which in turn leads to a loss of normal muscle function.[8-10,14]

About Soliris® (eculizumab)

Soliris® is a first-in-class complement inhibitor that works by inhibiting the terminal part of the complement cascade, a part of the immune system that, when activated in an uncontrolled manner, plays a role in serious ultra-rare disorders like paroxysmal nocturnal hemoglobinuria (PNH), atypical hemolytic uremic syndrome (aHUS) and anti-acetylcholine receptor (AChR) antibody-positive refractory generalized myasthenia gravis (gMG).

Soliris is approved in the U.S., EU, Japan and other countries as the first and only treatment for patients with PNH and aHUS, and in the EU as the first and only treatment for refractory gMG in patients who are anti-AChR antibody-positive. Soliris is not indicated for the treatment of patients with Shiga-toxin E. coli-related hemolytic uremic syndrome (STEC-HUS). Alexion and Soliris have received some of the pharmaceutical industry's highest honors for the medical innovation in complement inhibition: 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 Soliris 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 influenza* 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 commercializes the first and only approved complement inhibitor to treat patients with paroxysmal nocturnal hemoglobinuria (PNH), atypical hemolytic uremic syndrome (aHUS), and refractory generalized myasthenia gravis (gMG). In addition, Alexion has two highly innovative enzyme replacement therapies for patients with life-threatening and ultra-rare metabolic disorders, hypophosphatasia (HPP) and lysosomal acid lipase deficiency (LAL-D). As the leader in complement biology for over 20 years, Alexion focuses its research efforts on novel molecules and targets in the complement cascade, and its development efforts on the core therapeutic areas of hematology, nephrology, neurology, and metabolic disorders. 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 generalized myasthenia gravis (gMG), 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, the risks and uncertainties of drug development,

decisions of regulatory authorities regarding the adequacy of our research, marketing approval or material limitations on the marketing of eculizumab for the treatment of gMG, 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 clinical trials of our product candidates could be delayed, the adequacy of our pharmacovigilance and drug safety reporting processes, the risk that third party payers (including governmental agencies) will not reimburse or continue to reimburse for the use of our products at acceptable rates or at all, the outcome of challenges and opposition proceedings to our intellectual property, assertion or potential assertion by third parties that the manufacture, use or sale of our products infringes their intellectual property, 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 gMG are inaccurate, 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 June 30, 2017 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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