Interim Analysis from Phase 3 Open-Label Extension Study Shows Sustained Benefits of Soliris® (Eculizumab) Treatment for Patients with Refractory Generalized Myasthenia Gravis

New Data Presented at the American Association of Neuromuscular & Electrodiagnostic Medicine Annual Meeting Further Substantiate and Extend Results from Pivotal REGAIN Study

NEW HAVEN, Conn.—September 13, 2017, -- Alexion Pharmaceuticals, Inc. (NASDAQ: ALXN) announced today results from an interim analysis of an ongoing Phase 3 open-label extension study of the pivotal, placebo-controlled REGAIN study of Soliris® (eculizumab) for the treatment of patients with refractory generalized myasthenia gravis (gMG) who are anti-acetylcholine receptor (AChR) antibody-positive. The new results show sustained treatment benefits across a range of MG-specific assessment scales through an additional 52 weeks for patients who continued to receive Soliris, and also demonstrate rapid, significant and sustained improvements through 52 weeks for patients who had crossed over from placebo in REGAIN to Soliris treatment in the extension study. The safety profile of Soliris was consistent with that observed in the REGAIN study. The results are presented at the annual meeting of the American Association of Neuromuscular & Electrodiagnostic Medicine (AANEM) in Phoenix, Arizona.

"There is an urgent need for a treatment for patients with refractory gMG who have attempted multiple therapies and continue to suffer from severe symptoms and complications," said Professor James F. Howard, MD, Department of Neurology at the University of North Carolina, Chapel Hill, USA, and lead investigator in REGAIN and its open-label extension study. "These new results build on the findings of the REGAIN study, and it is encouraging to see the rapid and sustained benefits of Soliris treatment with patients recovering functional ability to carry out activities of daily living, and quality of life."

Results presented show that the benefits for patients treated with Soliris in REGAIN through 26 weeks were maintained in the extension study across all four assessment scales for an additional 52 weeks (78 weeks in total). For patients who received placebo in REGAIN and then were treated with Soliris in the extension study, significant treatment benefits occurred within 1 to 4 weeks and were sustained through 52 weeks across all four assessment scales.[1]

Treatment benefits in the REGAIN open-label extension study[1]

Assessment scale	Soliris		Soliris/Soliris		Soliris/Soliris		Placebo		Placebo/Soliris		Placebo/Soliris	
	REGAIN – week 26 n=55-56		extension – week 26 n=47-49		extension – week 52 n=20		REGAIN – week 26 n=59		extension – week 26 n=55-56		extension – week 52 n=20	
MG-ADL	-4.4	[-5.6, -3.3]	-5.2	[-6.3, -4.2]	-4.4	[-6.0, -2.7]	-2.3	[-3.2, -1.5]	-4.9	[-5.8, -4.0]	-5.3	[-6.8 -3.7]
QMG	-5.0	[-6.4, -3.6]	-5.2	[-6.7, -3.6]	-4.5	[-6.7, -2.3]	-1.7	[-2.7, -0.6]	-4.8	[-6.4, -3.3]	-6.4	[-8.8 -4.0]
MGC	-8.4	[-10.7, -6.2]	-10.0	[-12.3, -7.8]	-8.8	[-11.9, -5.6]	-5.0	[-6.8, -3.2]	-10.0	[-12.0, -8.0]	-10.0	[-13.3,-6.7]
MG-QoL 15	-12.8	[-16.6, -9.0]	-15.2	[-19.0, -11.3]	-14.9	[-21.7, -8.1]	-5.4	[-7.8, -2.9]	-12.9	[-16.6, -9.1]	-16.2	[-21.3,-11.1]

Treatment effects measured in decreased mean scores (using repeated measures from baseline) at given time points compared to baseline at enrollment in the REGAIN study (summarized for patients who enrolled in the extension study) [95% confidence intervals]

MG-ADL – MG-Activities of Daily Living, a patient-reported assessment of functional ability to carry out daily activities
QMG – Quantitative MG, a clinical assessment of muscle strength by physicians
MGC – MG Composite, a patient- and physician-reported assessment of functional ability and signs and symptoms of MG
MG-QoL 15 – MG Quality of Life 15, a patient-reported assessment of MG-specific quality of life

"We are grateful to the patients and investigators who continue to participate in this ongoing extension study that further substantiates the rapid and sustained benefits of complement inhibition for this debilitating, chronic and progressive neurological disorder," said John Orloff, M.D., Executive Vice President and Head of Research & Development at Alexion.

Patients with refractory gMG represent an ultra-rare subset of patients[2-5] who have difficulties walking, talking, swallowing and breathing normally despite multiple therapies for MG. Exacerbations and crises of their disease may require hospitalization and intensive care and may be life-threatening.[6-8] 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 the disease.[9-11]

Soliris is the first and only complement inhibitor, and the only complement-based therapy approved in the European Union (EU) for the treatment of patients with refractory gMG who are anti-AChR antibody-positive. Alexion's supplemental Biologics License Application (sBLA) in the U.S. and a supplemental new drug application in Japan for Soliris for similar indications 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 the Open-Label Extension Study (MG-302)
94% (117/125) of patients who completed the REGAIN study enrolled in the open-label extension, of which 56 continued to receive Soliris (Soliris/Soliris group) and 61 were switched from placebo to Soliris (placebo/Soliris group) within two weeks of completing the REGAIN study. Patients were not informed of prior treatment assignment in REGAIN through a four-week blinded induction phase, after which all patients received ongoing open-label treatment with Soliris (1,200 mg/dose) every two weeks. For this interim analysis, 49 patients in the Soliris/Soliris group and 56 patients in the placebo/Soliris group completed week 26 assessments; 20 patients in each group completed week 52 assessments. Patient numbers for scores at week 26 may differ slightly because a total score could not be computed when an assessment was missed or data were not complete. Mean scores over time were calculated using repeated measures from baseline. The study is ongoing and planned to continue until January 2019.

About REGAIN (MG-301)
This was a randomized, double-blind, placebo-controlled, multicenter Phase 3 clinical study that evaluated the efficacy and safety of Soliris over 26 weeks in 125 patients with refractory gMG who had a confirmed diagnosis of MG with positive serologic test for antibodies against AChR. Patients initially received 900 mg of Soliris or placebo weekly for 4 weeks followed by 1,200 mg of Soliris or placebo 1 week later, and then 1,200 mg of Soliris or placebo every 2 weeks. The primary efficacy endpoint of change from baseline in MG-ADL total score at week 26, as well as the three secondary endpoints — changes from baseline in QMG, MGC, and MG-QoL 15—were assessed using a worst-rank analysis. The study narrowly missed statistical significance on the primary endpoint (p=0.0698). However, 18 out of 22 pre-specified endpoints and analyses showed results with p-values <0.05 across the four assessment scales, supporting early, sustained and substantial responses. The safety profile was consistent with what has been reported for Soliris during the past 10 years in patients with paroxysmal nocturnal hemoglobinuria (PNH) and atypical hemolytic uremic syndrome (aHUS).

The REGAIN study (MG-301) and its open-label extension study (MG-302) are sponsored by Alexion.

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[2-4] who continue to suffer from severe disease symptoms and complications despite therapies currently used for MG.[2,3,12]

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.[6,7,13,14] It typically begins with weakness in the muscles that control the movements of the eyes 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.[14]

While most patients with gMG can be managed with therapies for MG, 10% to 15% of patients are considered refractory—meaning they fail to respond adequately to or cannot tolerate multiple therapies for MG and continue to suffer profound muscle weakness, and severe disease symptoms that limit function.[2,4,12] Patients with refractory gMG can suffer from slurred speech; choking; 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.[6-8]

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.[6,7] 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 muscle membrane at the NMJ. As a result, the communication between nerve and muscle is impaired, which in turn leads to a loss of normal muscle function.[9-11,15]

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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