Comprehensive Positive Phase 3 Data for Alexion's ALXN1210 in Patients with Paroxysmal Nocturnal Hemoglobinuria Presented at American Society of Hematology (ASH) Annual Meeting and Published in Blood

December 3, 2018

- First Conference Presentation of Results for ALXN1210 in Patients on Soliris® (Eculizumab) at ASH -

- Publications in Blood *of Results for ALXN1210 in Complement Inhibitor-Naïve Patients and Patients on Soliris® -*

- Presentation at ASH of New Results from Sensitivity Analyses in Inhibitor-Naïve Patients, and Analyses of C5 Inhibition and Breakthrough Hemolysis in Inhibitor-Naïve Patients and Patients on Soliris® -

SAN DIEGO--(BUSINESS WIRE)--Dec. 3, 2018-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today announced the presentation of comprehensive positive Phase 3 data for ALXN1210, the company's investigational long-acting C5 complement inhibitor, at the American Society of Hematology (ASH) Annual Meeting, taking place December 1-4, 2018. The presentations included both previously announced and new data from the two large Phase 3 studies in patients with paroxysmal nocturnal hemoglobinuria (PNH) who had either never been treated with a complement inhibitor before or who had been stable on Soliris® treatment. The conference presentations coincided with publications in *Blood* of the positive results on all primary and key secondary endpoints from these two studies.

"We are excited by the increasing body of data from our two active comparator-controlled Phase 3 studies, the largest PNH Phase 3 program ever conducted, on clinically meaningful endpoints in this devastating and potentially life-threatening disease. We are particularly pleased by the positive data in patients converting to ALXN1210 from Soliris®," said John Orloff, M.D., Executive Vice President and Head of Research & Development at Alexion. "Our ambition is to make ALXN1210 the new standard of care for patients with PNH."

Results from a Phase 3, Multicenter, Non-Inferiority Study of Ravulizumab (ALXN1210) Versus Eculizumab in Adult Patients with Paroxysmal Nocturnal Hemoglobinuria Currently Treated with Eculizumab – ASH, Session 101, Oral Presentation 625, Abstract ID# 119147, December 3, 2018[1]

The conference presentation of these previously announced data coincided with their peer-reviewed publication in *Blood*.[2]

A Phase 3 Study of Ravulizumab (ALXN1210) versus Eculizumab in Adults with Paroxysmal Nocturnal Hemoglobinuria Naïve to Complement Inhibitors: Results of a Subgroup Analysis with Patients Stratified by Baseline Hemolysis Level, Transfusion History, and Demographics – ASH, Session 101, Oral Presentation 627, Abstract ID# 110623, December 3, 2018[3]

These new results add to previously announced results on the co-primary and key secondary endpoints of this study, which have now also been published in *Blood*.[4]

A Prospective Analysis of Breakthrough Hemolysis in 2 Phase 3 Randomized Studies of Ravulizumab (ALXN1210) versus Eculizumab in Adults with Paroxysmal Nocturnal Hemoglobinuria – ASH, Oral and Poster Abstracts, Poster 2330, Abstract ID# 110874, December 2, 2018[5]

Ravulizumab (ALXN1210) versus Eculizumab in Adults with Paroxysmal Nocturnal Hemoglobinuria: Pharmacokinetics and Pharmacodynamics Observed in Two Phase 3 Randomized, Multicenter Studies – ASH Session 101, Oral Presentation 626, Abstract ID# 110858, December 3, 2018[6]

About Paroxysmal Nocturnal Hemoglobinuria (PNH)

Paroxysmal nocturnal hemoglobinuria (PNH) is a chronic, progressive, debilitating and life-threatening ultra-rare blood disorder characterized by hemolysis (destruction of red blood cells) that is mediated by an uncontrolled activation of the complement system, a component of the body's immune system.[7,8,9] PNH can strike men and women of all races, backgrounds and ages without warning, with an average age of onset in the early 30s.[7,10] PNH often goes unrecognized, with delays in diagnosis ranging from one to more than five years.[11] Patients with PNH may experience a wide range of signs and symptoms, such as fatigue, difficulty swallowing, shortness of breath, abdominal pain, erectile dysfunction, dark-colored urine and anemia.[9,12,13,14,15,16,17] The most devastating consequence of chronic hemolysis is thrombosis, which can occur in blood vessels throughout the body, damage vital organs and cause premature death.[18] The first thrombotic event can be fatal.[8,10,19] Despite historical supportive care, including transfusion and anticoagulation management, 20 to 35 percent of patients with PNH die within five to 10 years of diagnosis.[20,21] Patients with certain types of hemolytic anemia, bone marrow disorders and unexplained venous or arterial thrombosis are at increased risk of PNH.[9,22,23,24,25,26]

About ALXN1210

ALXN1210 is an innovative, investigational, long-acting C5 inhibitor discovered and developed by Alexion that works by inhibiting the C5 protein in the terminal complement cascade, a part of the body's immune system that, when activated in an uncontrolled manner, plays a role in severe ultra-rare disorders like paroxysmal nocturnal hemoglobinuria (PNH), atypical hemolytic uremic syndrome (aHUS), and anti-acetylcholine receptor (AchR) antibody-positive myasthenia gravis (MG). In Phase 3 clinical studies in complement inhibitor-naïve patients with PNH, and patients with PNH who had been stable on Soliris®, intravenous treatment with ALXN1210 every eight weeks demonstrated non-inferiority to intravenous treatment with Soliris® every two weeks, with numeric results for all primary and key secondary endpoints favoring ALXN1210. ALXN1210 is also currently being evaluated in

a Phase 3 clinical study in complement inhibitor-naïve patients with aHUS, administered intravenously every eight weeks. In addition, Alexion plans to initiate a Phase 3 clinical study of ALXN1210 delivered subcutaneously once per week as a potential treatment for patients with PNH and aHUS. Alexion is also planning to initiate the development of ALXN1210, intravenously administered every eight weeks, as a potential treatment for patients with generalized MG (gMG).

ALXN1210 has received Orphan Drug Designation (ODD) for the treatment of patients with PNH in the U.S., EU, and Japan, and for the subcutaneous treatment of patients with aHUS in the U.S.

About Soliris® (eculizumab)

Soliris® is a first-in-class complement inhibitor that works by inhibiting the C5 protein in the terminal part of the complement cascade, a part of the immune system that, when activated in an uncontrolled manner, plays a role in severe rare and ultra-rare disorders like paroxysmal nocturnal hemoglobinuria (PNH), atypical hemolytic uremic syndrome (aHUS), and anti-acetylcholine receptor (AchR) antibody-positive myasthenia gravis (MG). Soliris® is approved in the U.S., EU, Japan, and other countries as the first and only treatment for patients with PNH and aHUS, in the EU as the first and only treatment of refractory generalized MG (gMG) in adults who are anti-AchR antibody-positive, in the U.S. for the treatment of adult patients with gMG who are anti-AchR antibody-positive, and in Japan for the treatment of patients with gMG who are AChR antibody-positive and whose symptoms are difficult to control with high-dose intravenous immunoglobulin (IVIG) therapy or plasmapheresis (PLEX). Soliris® is not indicated for the treatment of patients with Shiga-toxin E. coli-related hemolytic uremic syndrome (STEC-HUS).

Soliris® has received Orphan Drug Designation (ODD) for the treatment of patients with PNH in the U.S., EU, Japan, and many other countries, for the treatment of patients with aHUS in the U.S., EU, and many other countries, for the treatment of patients with MG in the U.S. and EU, for the treatment of patients with refractory gMG in Japan, and for the treatment of patients with neuromyelitis optica spectrum disorder (NMOSD) in the U.S., EU, and Japan. 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. In patients with gMG who are anti-AchR antibody-positive, the most frequently reported adverse reaction observed with Soliris® treatment in the placebo-controlled clinical study (≥10%) was musculoskeletal pain.

About Alexion

Alexion is a global biopharmaceutical company focused on serving patients and families affected by rare diseases through the discovery, development and commercialization of life-changing therapies. As the global leader in complement biology and inhibition for more than 20 years, Alexion 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 anti-acetylcholine receptor (AchR) antibody-positive generalized myasthenia gravis (gMG), and is also developing it for patients with neuromyelitis optica spectrum disorder (NMOSD). Alexion also 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). In addition, the company is developing several mid-to-late-stage therapies, including a second complement inhibitor, a copper-binding agent for Wilson disease and an anti-neonatal Fc receptor (FcRn) antibody for rare Immunoglobulin G (IgG)-mediated diseases. 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. Alexion has been named to the Forbes list of the World's Most Innovative Companies seven years in a row and is headquartered in Boston, Massachusetts' Innovation District. The company also has offices around the globe and serves patients in more than 50 countries. This press release and further information about Alexion can be found at: www.alexion.com.

[ALXN-G]

Forward-Looking Statement

This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 that involve risks and uncertainties relating to future events and the future performance of Alexion, including statements related to: the Company's ambition to make ALXN1210 the new standard of care for patients with PNH; Alexion plans to initiate a Phase 3 clinical study of ALXN1210 delivered subcutaneously once per week as a potential treatment for patients with PNH and aHUS; Alexion is planning to initiate the development of ALXN1210, intravenously administered every eight weeks, as a potential treatment for patients with generalized MG (gMG); the Company is developing a complement inhibitor

for patients with neuromyelitis optica spectrum disorder (NMOSD); future plans to initiate a clinical studies of ALXN1210 delivered subcutaneously once per week as a potential treatment for patients with PNH and for studies of ALXN1210 for other indications; and the potential medical benefits of ALXN1210 for the treatment of PNH and other diseases. Forward-looking statements are subject to factors that may cause Alexion's results and plans to differ materially from those expected by these forward looking statements, including for example: our dependence on sales from our principal product (Soliris®); future competition from biosimilars and other products; decisions of regulatory authorities regarding the adequacy of our research, marketing approval or material limitations on the marketing of our products; delays or failure of product candidates to obtain regulatory approval; delays or the inability to launch product candidates due to regulatory restrictions, anticipated expense or other matters; 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; results in early stage clinical trials may not be indicative of full results or results from later stage or larger clinical trials (or broader patient populations) and do not ensure regulatory approval; the possibility that results of clinical trials are not predictive of safety and efficacy and potency of our products (or we fail to adequately operate or manage our clinical trials) which could cause us to halt trials, delay or prevent us from making regulatory approval filings or result in denial of approval of our product candidates; unexpected delays in clinical trials; future product improvements may not be realized due to expense or feasibility; uncertainty of long-term success in developing, licensing or acquiring other product candidates or additional indications for existing products; inability to complete planned acquisitions due to failure of regulatory approval or material changes in the target or otherwise; inability to complete acquisitions and investments due to increased competition for technology; the possibility that current rates of adoption of Soliris® in PNH, aHUS, gMG or other diseases are not sustained; the adequacy of our pharmacovigilance and drug safety reporting processes; failure to protect and enforce our data, intellectual property and proprietary rights and the risks and uncertainties relating to intellectual property claims and challenges against us; 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; failure to realize the benefits and potential of investments, collaborations, licenses and acquisitions; the possibility that expected tax benefits will not be realized; assessment of impact of recent accounting pronouncements; potential declines in sovereign credit ratings or sovereign defaults in countries where we sell our products; delay of collection or reduction in reimbursement due to adverse economic conditions or changes in government and private insurer regulations and approaches to reimbursement; uncertainties surrounding legal proceedings, company investigations and government investigations, including investigations of Alexion by the U.S. Securities and Exchange Commission (SEC) and U.S. Department of Justice; the risk that estimates regarding the number of patients with PNH, aHUS, gMG, HPP and LAL-D and other future indications we are pursuing are inaccurate; the risks of changing foreign exchange rates; risks relating to the potential effects of the Company's restructuring; risks related to the acquisition of Syntimmune and other companies and co-development efforts; and a variety of other risks set forth from time to time in Alexion's filings with the SEC, including but not limited to the risks discussed in Alexion's Quarterly Report on Form 10-Q for the period ended September 30, 2018 and in our other filings with the SEC. Alexion disclaims any obligation 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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