Alexion Submits Application for Priority Review and Approval of ALXN1210 as a Treatment for Patients with Paroxysmal Nocturnal Hemoglobinuria (PNH) in the U.S.

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-- Use of Priority Review Voucher Provides Expedited Review of Eight Months --

-- Submission in the European Union on Track for Mid-Year and in Japan for the Second Half of the Year --

NEW HAVEN, Conn.--(BUSINESS WIRE)--Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today announced the submission of a Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA) for approval of ALXN1210, the Company's investigational long-acting C5 complement inhibitor, for the treatment of patients with paroxysmal nocturnal hemoglobinuria (PNH). The submission uses a rare disease priority review voucher, which designates the BLA for an expedited eight-month review by the FDA instead of the standard 12-month review.

"This first regulatory submission is an important step toward our goal of establishing ALXN1210 as the new standard of care for patients with PNH, building on 10 years of proven efficacy and safety with Soliris®, and 25 years of leadership in complement biology," said John Orloff, M.D., Executive Vice President and Head of Research & Development at Alexion. "We look forward to working with the FDA to facilitate a rapid review."

The application is supported by comprehensive data from two rigorous Phase 3 clinical trials in the largest population of patients with PNH ever studied: more than 440 patients, which included patients who had never received a complement inhibitor, and patients who were stable on Soliris® (eculizumab) and switched to ALXN1210. Weight-optimized treatment with ALXN1210 every eight weeks demonstrated non-inferiority to treatment every two weeks with Soliris® on all primary endpoints and key secondary endpoints in both studies. The numeric results for all these endpoints, including breakthrough hemolysis (one of the key secondary endpoints), favored ALXN1210 in both studies and are consistent with the immediate and complete C5 inhibition observed by the end of the first infusion of ALXN1210 and sustained throughout the entire 26-week treatment period. There were no notable differences in the safety profiles for ALXN1210 and Soliris®. Topline data of these Phase 3 studies were disclosed in press releases on March 15, 2018 and April 26, 2018, respectively.

In addition to the BLA in the U.S., Alexion is preparing submissions for the approval of ALXN1210 as a treatment for patients with PNH in the European Union (EU) by mid-year and in Japan in the second half of the year. ALXN1210 has received Orphan Drug Designation (ODD) for the treatment of patients with PNH in the U.S. and EU.

About Paroxysmal Nocturnal Hemoglobinuria (PNH)

Paroxysmal nocturnal hemoglobinuria (PNH) is a chronic, progressive, debilitating, and potentially life-threatening ultra-rare blood disorder that can strike men and women of all races, backgrounds, and ages without warning, with an average age of onset in the early 30s.[1,2,3] PNH often goes unrecognized, with delays in diagnosis ranging from one to more than 10 years.[2] In patients with PNH, chronic, uncontrolled activation of the complement system, a component of the body's immune system, results in hemolysis (the destruction of red blood cells) [4], which in turn can result in progressive anemia, fatigue, dark urine, and shortness of breath.[5,6,7] The most devastating consequence of chronic hemolysis is thrombosis (the formation of blood clots), which can damage vital organs and cause premature death.[8] Historically, it had been estimated that one in three patients with PNH did not survive more than five years from the time of diagnosis.[2] PNH is more common among patients with disorders of the bone marrow, including aplastic anemia (AA) and myelodysplastic syndromes (MDS).[9,10,11] In certain patients with thrombosis of unknown origin, PNH may be an underlying cause.[4]

About ALXN1210

ALXN1210 is an innovative, 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 also plans to initiate the development of ALXN1210 as a potential treatment for patients with generalized MG (gMG) and patients with immunoglobulin A nephropathy (IgAN).

ALXN1210 has received Orphan Drug Designation (ODD) for the treatment of patients with PNH in the U.S. and EU, 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, and for the treatment of patients with refractory gMG in 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 innovation, development and commercialization of life-changing therapies. 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 anti-acetylcholine receptor (AchR) antibody-positive 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 press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including statements related to the submission of regulatory applications for review and approval by authorities in the US, Japan and the European Union for ALXN1210, the timing of anticipated future submissions of regulatory applications for ALXN 1210 for review and approval by certain governmental authorities, the anticipated timing and the speed of the review of regulatory applications for ALXN 1210 by governmental authorities, making ALXN1210 the new standard of care for patients with PNH and the potential medical benefits of ALXN1210 for the treatment of PNH. 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, the inability to submit regulatory applications for ALXN 1210 for review and approval by certain governmental authorities in the timeframes expected due to delays or future product information, decisions of regulatory authorities regarding the adequacy of our research, marketing approval or material limitations on the marketing of our products (or the indications of such 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 our products are not sustained (or do not meet expected future rates), 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 (or proposed future products) at acceptable rates or at all, 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 other anticipated

regulatory filings are delayed, the risk that estimates regarding the number of patients with the diseases that our products treat are inaccurate, 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 March 31, 2018 and in Alexion's 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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