Positive Phase 3 Extension Data for ULTOMIRIS® (ravulizumab) in Patients with Paroxysmal Nocturnal Hemoglobinuria (PNH) Presented at European Hematology Association (EHA) Congress

June 14, 2019

- ULTOMIRIS demonstrated consistent efficacy and safety through 52 weeks, with no cases of breakthrough hemolysis (BTH) associated with incomplete C5 complement inhibition -

- Nearly all surveyed patients preferred ULTOMIRIS over SOLIRIS® (eculizumab) -

BOSTON--(BUSINESS WIRE)--Jun. 14, 2019-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today announced the presentation of new data demonstrating that ULTOMIRIS® (ravulizumab), the first and only long-acting C5 complement inhibitor administered every eight weeks, provided consistent efficacy and safety through 52 weeks in the extension[1] of the Phase 3 study of ULTOMIRIS and SOLIRIS® (eculizumab) in complement inhibitor-naïve, adult patients with paroxysmal nocturnal hemoglobinuria (PNH).[2] Sustained efficacy of ULTOMIRIS was observed on the co-primary endpoints of transfusion avoidance and normalization of lactate dehydrogenase (LDH) levels and the secondary endpoints of LDH level reduction and breakthrough hemolysis (BTH). In an additional sub-study, nearly all patients preferred ULTOMIRIS over SOLIRIS.[3] The data will be presented at the Annual Congress of the European Hematology Association (EHA), taking place June 13-19, 2019 in Amsterdam, Netherlands.

LDH level normalization and reduction are direct markers for reduced hemolysis in PNH, a severe, ultra-rare disease characterized by complement-mediated intravascular hemolysis. PNH can cause a wide range of debilitating symptoms and complications, including thrombosis, which can occur throughout the body and result in organ damage and premature death.[4,5,6,7,8,9,10,11] Incomplete inhibition of the C5 complement protein can increase the risk of BTH and related serious complications.[12,13,14,15]

“The confirmation of consistent efficacy and safety through 52 weeks with only six or seven infusions per year instead of 26 with SOLIRIS makes ULTOMIRIS a very compelling new therapy for patients with PNH,” said Professor Hubert Schrezenmeier, M.D., Medical Director of the Institute of Transfusion Medicine and the Institute for Clinical Transfusion Medicine and Immunogenetics, German Red Cross Blood Transfusion Service Baden-Württemberg-Hessen and University Hospital Ulm, Germany, study investigator and presenting author. “I am impressed with the continuing complete C5 inhibition in all patients receiving ULTOMIRIS and the absence of breakthrough hemolysis associated with incomplete C5 inhibition. This makes me hopeful that we can reduce the potentially devastating consequences of returning PNH symptoms.”

All patients in the initial ULTOMIRIS group of the extension study maintained complete C5 inhibition through 52 weeks, and no patient experienced BTH associated with incomplete C5 inhibition. All patients who had experienced incomplete C5 inhibition while receiving SOLIRIS in the first 26 weeks achieved complete C5 inhibition after the switch to ULTOMIRIS in the extension phase. No patient experienced BTH associated with incomplete C5 inhibition between weeks 27 and 52 after switching to ULTOMIRIS compared to six percent while receiving SOLIRIS in the first 26 weeks.[1]

“We continue to expand the body of clinical evidence supporting the potential of ULTOMIRIS to become the new standard of care for patients with PNH,” said John Orloff, M.D., Executive Vice President and Head of Research & Development at Alexion. “SOLIRIS, the first approved therapy for PNH, was a breakthrough for patients for whom only supportive care had been available before. With ULTOMIRIS, we want to enable patients to live their lives more freely thanks to maximal hemolysis control with established safety and reduced treatment burden.”

The most common adverse events occurred less frequently in the extension phase than during the initial treatment phase where the safety profile of ULTOMIRIS was consistent with that of SOLIRIS.[2] The most common treatment-emergent adverse events in the extension phase were upper respiratory tract infection (in the initial ULTOMIRIS arm) and nasopharyngitis (in the initial SOLIRIS arm). The most frequently observed serious adverse event was pyrexia. One patient in the initial SOLIRIS arm died from lung cancer (unrelated to SOLIRIS treatment). There was no case of meningococcal infection observed.[1,2]

ULTOMIRIS was studied in the largest-ever Phase 3 program in PNH. The entire clinical development program for ULTOMIRIS in PNH to date represents more than 800 patient years of experience.

Additional data to be presented at the EHA congress indicate a very strong patient preference for ULTOMIRIS over SOLIRIS.[3] According to results from a sub-study in the ULTOMIRIS Phase 3 extension in patients who had been stable on SOLIRIS before,[16] nearly all patients (93%) preferred ULTOMIRIS due to reduced infusion frequency, ability to plan activities, overall quality of life, convenience of treatment, and effectiveness of medication until the next infusion compared to SOLIRIS.[3]

New results from the International PNH Registry will also be presented at the EHA congress. These data suggest that a change in clone size does not change the increased risk of major adverse vascular events in PNH,[17] and that complement inhibition with SOLIRIS does not change the effectiveness of concomitant immunosuppressive therapy in patients with PNH and aplastic anemia (AA).[18]

The U.S. Food and Drug Administration (FDA) approved ULTOMIRIS for adult patients with PNH on December 21, 2018. The European Commission (EC) is reviewing the recommendation by the European Medicines Agency’s (EMA) Committee for Medicinal Products for Human Use (CHMP) from April 26, 2019 to approve ULTOMIRIS as a treatment for adult patients with PNH and typically delivers its final decision within two months. The Japanese Ministry of Health, Labour and Welfare (MHLW) is reviewing the recommendation by the Pharmaceuticals and Medical Devices Agency’s (PMDA) Drug Committee (BUKAI) to approve ULTOMIRIS as a treatment for patients with PNH and is anticipated to deliver a decision in late June.

One-Year Efficacy of Ravulizumab (ALXN1210) in Adult Patients with Paroxysmal Nocturnal Hemoglobinuria Naive to Complement Inhibitors, EHA

Congress, June 13-16, 2019Amsterdam, Netherlands, oral presentation, June 15, 2019, 12:00 p.m., abstract S863.[1]

Patient Preferences for the Treatment of Paroxysmal Nocturnal Hemoglobinuria: Results of a Patient Survey of Ravulizumab (ALXN1210) and Eculizumab, EHA Congress, June 13-16, 2019Amsterdam, Netherlands, poster presentation, June 14, 2019, 5:30 p.m., abstract PF734.[3]

Change in Clone Size Does Not Predict Risk of Major Adverse Vascular Events: Results from the International PNH Registry, EHA Congress, June 13-16, 2019Amsterdam, Netherlands, oral presentation, June 15, 2019, 12:30 p.m., abstract S865.[17]

No Change in the Effectiveness of Immunosuppressive Therapy in Patients with PNH and AA Receiving concomitant Eculizumab, EHA Congress, June 13-16, 2019Amsterdam, Netherlands, poster presentation, June 15, 2019, 5:30 p.m., abstract PS1117.[18]

About the extension[1] of the Phase 3 study in complement-naïve, adult patients with PNH[2]

At the end of the 26-week Phase 3 study,[2] all patients (246; 125 on ULTOMIRIS, 121 on SOLIRIS) had the option to receive ULTOMIRIS every eight weeks for up to two years. The aim of this extension study is to monitor the durability of efficacy in the initial ULTOMIRIS group, the efficacy of ULTOMIRIS in the initial SOLIRIS group after the switch to ULTOMIRIS and the safety of ULTOMIRIS in all patients. A total of 243 patients (124 from the initial ULTOMIRIS group, 119 from the initial SOLIRIS group) were followed. Results for the co-primary endpoints of transfusion avoidance and LDH level normalization and the secondary endpoints of percentage change from baseline in LDH levels and proportion of patients with BTH are provided descriptively. Complete C5 inhibition was defined as plasma levels of free C5 ≤0.5 µg/ml.[1]

About the patient preference study[3]

At the end of the 26-week Phase 3 study in adult patients with PNH who had been clinically stable on SOLIRIS for at least 6 months,[16] all patients (195) were given the option to continue receiving ULTOMIRIS every eight weeks for up to two years. The patient preference study enrolled patients from the extension study who had received at least two doses of ULTOMIRIS. Patient treatment preference was evaluated at one time point using an 11-item PNH-specific Patient Preference Questionnaire (PNH-PPQ©). The 11 questions included one question on overall treatment preference, nine questions on preference based on treatment characteristics, one question on the most important treatment characteristic for the overall treatment preference, four questions on aspects of treatment with SOLIRIS and four matching questions for ULTOMIRIS. Of 98 patients enrolled, 95 patients from eight countries (European Union, North America, and Australia) completed PNH-PPQs per protocol.[3]

About Paroxysmal Nocturnal Hemoglobinuria (PNH)

Paroxysmal nocturnal hemoglobinuria (PNH) is a chronic, progressive, debilitating and life-threatening ultra-rare blood disorder characterized by intravascular hemolysis (destruction of red blood cells) that is mediated by an uncontrolled activation of the complement system, a part of the immune system.[4,5,19] PNH can strike men and women of all races, backgrounds and ages without warning, with an average age of onset in the early 30s.[4,20] PNH often goes unrecognized, with delays in diagnosis ranging from one to more than five years.[21] 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.[6,7,8,9,10,11,19] The most devastating consequence of chronic intravascular hemolysis is thrombosis, which can occur in blood vessels throughout the body, damage vital organs and cause premature death.[22] The first thrombotic event can be fatal.[4,20,23] 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.[24,25] Patients with certain types of hemolytic anemia, bone marrow disorders and unexplained venous or arterial thrombosis are at increased risk of PNH.[14,19,26,27,28,29]

About ULTOMIRIS®

ULTOMIRIS (ravulizumab-cwvz), the first and only long-acting C5 inhibitor administered every eight weeks, is approved in the U.S. as a treatment for adults with paroxysmal nocturnal hemoglobinuria (PNH). ULTOMIRIS works by inhibiting the C5 protein in the terminal complement cascade, a part of the body's immune system. The terminal complement cascade, when activated in an uncontrolled manner, plays a role in severe ultra-rare disorders like PNH, atypical hemolytic uremic syndrome (aHUS), anti-acetylcholine receptor (AchR) antibody-positive myasthenia gravis (MG) and anti-aquaporin-4 (AQP4) auto-antibody-positive neuromyelitis optica spectrum disorder (NMOSD). Regulatory authorities in the European Union (EU) and Japan are reviewing applications for the approval of ULTOMIRIS as a treatment for adult patients and patients with PNH, respectively. In Phase 3 clinical studies in complement inhibitor-naïve patients with PNH[2] and patients with PNH who had been stable on SOLIRIS® (eculizumab),[16] intravenous treatment with ULTOMIRIS every eight weeks demonstrated non-inferiority to intravenous treatment with SOLIRIS every two weeks on all endpoints.

The Phase 3 study of ULTOMIRIS, administered intravenously every eight weeks in adult patients with aHUS, met its primary objective. Alexion has submitted a supplemental Biologics License Application (sBLA) to the U.S. Food and Drug Administration (FDA) for approval of ULTOMIRIS as a treatment for patients with aHUS and plans to submit similar applications in the EU and Japan later in 2019. ULTOMIRIS is also currently being evaluated in a Phase 3 clinical study in children and adolescents with aHUS, administered intravenously every eight weeks. Alexion has initiated a Phase 3 study of ULTOMIRIS, intravenously administered every eight weeks, as a potential treatment for patients with generalized MG (gMG), and is planning to initiate a Phase 3 in patients with NMOSD. In addition, Alexion has initiated Phase 3 studies of ULTOMIRIS delivered subcutaneously once per week as a potential treatment for patients with PNH and aHUS.

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

U.S. Indication of ULTOMIRIS®

ULTOMIRIS (ravulizumab-cwvz) is a prescription medicine called a monoclonal antibody. ULTOMIRIS is used to treat adults with a disease called paroxysmal nocturnal hemoglobinuria (PNH). It is not known if ULTOMIRIS is safe and effective in children.

U.S. Important Safety Information for ULTOMIRIS®

ULTOMIRIS (ravulizumab-cwvz) is a medicine that affects the immune system. ULTOMIRIS can lower the ability of the immune system to fight infections. ULTOMIRIS increases the chance of getting serious and life-threatening meningococcal infections. Meningococcal infections may quickly become life-threatening and cause death if not recognized and treated early.

Meningococcal vaccines must be received at least 2 weeks before the first dose of ULTOMIRIS if one has not already had this vaccine. If one's doctor decided that urgent treatment with ULTOMIRIS is needed, meningococcal vaccination should be administered as soon as possible. If one has not been vaccinated and ULTOMIRIS therapy must be initiated immediately, 2 weeks of antibiotics should also be administered with the vaccinations. If one had a meningococcal vaccine in the past, additional vaccination might be needed before starting ULTOMIRIS. Call one's doctor or get emergency medical care right away if any of these signs and symptoms of a meningococcal infection occur: headache with nausea or vomiting, headache with a stiff neck or stiff back, fever and a rash, muscle aches with flu-like symptoms, headache and fever, fever, confusion, and eyes sensitive to light.

ULTOMIRIS is only available through a program called the ULTOMIRIS REMS.

ULTOMIRIS may also increase the risk of other types of serious infections. People who take ULTOMIRIS may have an increased risk of getting infections caused by *Streptococcus pneumoniae* and *Haemophilus influenzae*. Certain people may also have an increased risk of gonorrhea infection. To find out if one is at risk for gonorrhea infection, about gonorrhea prevention, and regular testing, talk to the healthcare provider. Call the healthcare provider right away if one has any new signs or symptoms of infection.

Before one receives ULTOMIRIS, tell the doctor about all of the medical conditions, including if one: has an infection or fever, is pregnant or plans to become pregnant, and is breastfeeding or plans to breastfeed. It is not known if ULTOMIRIS will harm an unborn baby. It is not known if ULTOMIRIS passes into the breast milk. One should not breast feed during treatment and for 8 months after one's final dose of ULTOMIRIS.

Tell the doctor about all the medicines one takes, including prescription and over-the-counter medicines, vitamins, and herbal supplements. ULTOMIRIS and other medicines can affect each other causing side effects. Know the medications one takes and the vaccines one receives. Keep a list of them to show the doctor and pharmacist when one gets a new medicine.

If one stops receiving ULTOMIRIS, the doctor will need to monitor closely for at least 16 weeks after one stops ULTOMIRIS. Stopping ULTOMIRIS may cause breakdown of the red blood cells due to PNH. Symptoms or problems that can happen due to red blood cell breakdown include: drop in the number of the red blood cell count, tiredness, blood in the urine, stomach-area (abdomen) pain, blood clots, shortness of breath, trouble swallowing, and erectile dysfunction (ED) in males.

ULTOMIRIS can cause serious side effects including infusion reactions. Infusion reactions may happen during one's ULTOMIRIS infusion. Symptoms of an infusion reaction with ULTOMIRIS may include lower back pain, pain with the infusion, or feeling faint. Tell the doctor or nurse right away if these symptoms develop, or any other symptoms during the ULTOMIRIS infusion that may mean one is having a serious infusion reaction, including: chest pain, trouble breathing or shortness of breath, swelling of the face, tongue, or throat, and feel faint or pass out. One's doctor will treat the symptoms as needed. The most common side effects of ULTOMIRIS are upper respiratory infection and headache.

For more information, please see the full U.S. Prescribing Information and Medication Guide for ULTOMIRIS, including Boxed WARNING regarding serious and life-threatening meningococcal infections/sepsis, also available at: www.ultomiris.com.

U.S. Indication for SOLIRIS®
SOLIRIS (eculizumab) is a prescription medicine called a monoclonal antibody. SOLIRIS is used to treat patients with a disease called Paroxysmal Nocturnal Hemoglobinuria (PNH), adults and children with a disease called atypical Hemolytic Uremic Syndrome (aHUS) (SOLIRIS is not for use in treating people with Shiga toxin E. coli related hemolytic uremic syndrome [STEC-HUS]), and adults with a disease called generalized Myasthenia Gravis (gMG) who are anti-acetylcholine receptor (AchR) antibody positive. It is not known if SOLIRIS is safe and effective in children with PNH or gMG.

U.S. Important Safety Information for SOLIRIS®
SOLIRIS (eculizumab) is a medicine that affects the immune system. SOLIRIS can lower the ability of the immune system to fight infections. SOLIRIS increases the chance of getting serious and life-threatening meningococcal infections. Meningococcal infections may quickly become life-threatening and cause death if not recognized and treated early.

Meningococcal vaccines must be received at least 2 weeks before the first dose of SOLIRIS if one has not already had this vaccine. If one's doctor decided that urgent treatment with SOLIRIS is needed, meningococcal vaccination should be administered as soon as possible. If one has not been vaccinated and SOLIRIS therapy must be initiated immediately, 2 weeks of antibiotics should also be administered with the vaccinations. If one had a meningococcal vaccine in the past, additional vaccination might be needed before starting SOLIRIS. Call one's doctor or get emergency medical care right away if any of these signs and symptoms of a meningococcal infection occur: headache with nausea or vomiting, headache and fever, headache with a stiff neck or stiff back, fever, fever and a rash, confusion, muscle aches with flu-like symptoms, and eyes sensitive to light.

SOLIRIS is only available through a program called the SOLIRIS REMS.

SOLIRIS may also increase the risk of other types of serious infections. If one's child is treated with SOLIRIS, make sure that the child receives vaccinations against *Streptococcus pneumoniae* and *Haemophilus influenzae* type b (Hib). Certain people may be at risk of serious infections with gonorrhea. Talk to the doctor about whether one is at risk for gonorrhea infection, about gonorrhea prevention, and regular testing. Certain fungal infections (Aspergillus) may also happen if one takes SOLIRIS and has a weak immune system or a low white blood cell count.

Before one receives SOLIRIS, tell the doctor about all of the medical conditions, including if one: has an infection or fever, is pregnant or plans to become pregnant, and is breastfeeding or plans to breastfeed. It is not known if SOLIRIS will harm an unborn baby. It is not known if SOLIRIS passes into the breast milk.

Tell the doctor about all the medicines one takes, including prescription and over-the-counter medicines, vitamins, and herbal supplements. SOLIRIS and other medicines can affect each other causing side effects.

It is important that one: has all recommended vaccinations before starting SOLIRIS, receives 2 weeks of antibiotics if one immediately starts SOLIRIS, and stays up-to-date with all recommended vaccinations during treatment with SOLIRIS. Know the medications one takes and the vaccines one receives. Keep a list of them to show the doctor and pharmacist when one gets a new medicine.

If one has PNH, the doctor will need to monitor closely for at least 8 weeks after stopping SOLIRIS. Stopping treatment with SOLIRIS may cause breakdown of the red blood cells due to PNH. Symptoms or problems that can happen due to red blood cell breakdown include: drop in the number of

the red blood cell count, drop in the platelet counts, confusion, kidney problems, blood clots, difficulty breathing, and chest pain.

If one has aHUS, the doctor will need to monitor closely for at least 12 weeks after stopping SOLIRIS for signs of worsening aHUS symptoms or problems related to abnormal clotting (thrombotic microangiopathy). Symptoms or problems that can happen with abnormal clotting may include: stroke, confusion, seizure, chest pain (angina), difficulty breathing, kidney problems, swellings in arms or legs and a drop in platelet count.

SOLIRIS can cause serious side effects including serious allergic reactions. Serious allergic reactions can happen during one's SOLIRIS infusion. Tell the doctor or nurse right away if one gets any of these symptoms during the SOLIRIS infusion: chest pain, trouble breathing or shortness of breath, swelling of the face, tongue, or throat, and feeling faint or pass out. If one has an allergic reaction to SOLIRIS, the doctor may need to infuse SOLIRIS more slowly, or stop SOLIRIS. The most common side effects in people with PNH treated with SOLIRIS include: headache, pain or swelling of the nose or throat (nasopharyngitis), back pain, and nausea. The most common side effects in people with aHUS treated with SOLIRIS include: headache, diarrhea, high blood pressure (hypertension), common cold (upper respiratory infection), stomach-area (abdominal pain), vomiting, pain or swelling of the nose or throat (nasopharyngitis), low red blood cell count (anemia), cough, swelling of legs or feet (peripheral edema), nausea, urinary tract infections, and fever. The most common side effects in people with gMG treated with SOLIRIS include: muscle and joint (musculoskeletal) pain.

Please see the accompanying full U.S. Prescribing Information and Medication Guide for SOLIRIS, including Boxed WARNING regarding serious and life-threatening meningococcal infections, also available at: www.soliris.net.

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 two approved complement inhibitors to treat patients with paroxysmal nocturnal hemoglobinuria (PNH) as well as the first and only approved complement inhibitor to treat 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 as well as several early-stage therapies, including one for light chain (AL) amyloidosis and a second anti-FcRn therapy. 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 that involve risks and uncertainties relating to future events and the future performance of Alexion, including statements related to: the efficacy and safety of ULTOMIRIS as a treatment of PNH; that the Phase 3 extension study data confirm consistent efficacy and safety of ULTOMIRIS; that six or seven infusions per year instead of 26 with SOLIRIS make ULTOMIRIS a very compelling new therapy for patients with PNH; that patients prefer ULTOMIRIS over SOLIRIS and that ULTOMIRIS results in a reduced treatment burden for patients as compared to SOLIRIS; ULTOMIRIS has the potential to become the new standard of care for patients with PNH; the expected timing of the final decision of the EC with respect to pharmaceutical product approval including ULTOMIRIS for PNH; Alexion's future plans for submitting supplemental Biologics License Application and similar applications to the applicable regulatory authorities for ULTOMIRIS as a therapy for certain indications; ULTOMIRIS is a potential treatment for patients with gMG and NMOSD; the company is planning to initiate a Phase 3 clinical trial in patients with NMOSD; the anticipated timing of the review and decision of regulatory agencies with respect to the potential approval of ULTOMIRIS as a treatment for PNH in certain jurisdictions; future plans for the evaluation and clinical trials of ULTOMIRIS in additional indications and patient populations, including subcutaneous administration; and the potential medical benefits of ULTOMIRIS 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 inability to facilitate the timely conversion of patients with PNH (and any future indications) from SOLIRIS to ULTOMIRIS; payer, physician and patient acceptance of ULTOMIRIS as an alternative to SOLIRIS; ULTOMIRIS does not gain market acceptance and/or does not become the standard of care for patients with PNH and/or is not recognized by patients and physicians as the standard of care for patients with PNH; the benefits (including safety and efficacy) of ULTOMIRIS evidenced in clinical trials are not witnessed in a broader patient population; any potential post-approval restrictions that the FDA or any other regulatory agency may impose on ULTOMIRIS; ULTOMIRIS does not gain regulatory approval from the EC or Japanese MHLW as a treatment for PNH; ULTOMIRIS does not gain approval from regulatory agencies as a treatment for indications beyond PNH, including aHUS; delays (expected or unexpected) in the time it takes regulatory agencies to review and make determinations on applications for the marketing approval of our products (including ULTOMIRIS as a treatment for PNH); inability to timely submit (or failure to submit) future applications for regulatory approval for our products and product candidates; inability to timely initiate (or failure to initiate) future clinical trials due to safety issues, Institutional Review Board (IRB) decisions, Chemistry, Manufacturing, and Controls (CMC)-related issues, expense or unfavorable results from earlier trials (among others); 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 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; 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; the possibility that current rates of adoption of SOLIRIS in PNH, aHUS, gMG or other diseases (and ULTOMIRIS in PNH in the U.S.) 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 (including intellectual property lawsuits relating to ULTOMIRIS brought by third parties against Alexion and inter partes review petitions submitted by third parties); 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; 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 (including intellectual property suits initiated against Alexion and our products), 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, NMOSD, 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 March 31, 2019 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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