Alexion Announces Upcoming Data Presentations at the Virtual 25th Congress of the European Hematology Association

May 14, 2020

- Accepted abstracts include interim Phase 2 extension data demonstrating reduced duration of infusion with ULTOMIRIS® (ravulizumab-cwvz) 100 mg/mL formulation -

- Data from five abstracts demonstrate Alexion's continued commitment to advancing the understanding of PNH with ongoing real-world studies and emerging clinical data -

BOSTON--(BUSINESS WIRE)--May 14, 2020-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today announced that five abstracts have been accepted for presentation at the 25th Congress of the European Hematology Association (EHA), which will be held virtually from June 11 to 14, 2020. During the meeting, results will be presented from an interim analysis of the Phase 2 ULTOMIRIS® (ravulizumab-cwvz) 100 mg/mL formulation open-label study extension period. The 100 mg/mL formulation of ULTOMIRIS—a long-acting C5 inhibitor administered every eight weeks offering immediate, complete and sustained complement inhibition—was evaluated to determine if participants with paroxysmal nocturnal hemoglobinuria (PNH) could achieve greater convenience by reducing infusion times, thus lessening demands on the healthcare system.

"ULTOMIRIS is an important new standard of care for patients with PNH and atypical hemolytic uremic syndrome," said John Orloff, M.D., Executive Vice President and Head of Research and Development at Alexion. "An interim analysis of a PNH study evaluating the higher concentration formulation showed a 78-minute reduction in infusion time for adult patients in the 60-100kg cohort (representative of the majority of patients treated for PNH), and comparable safety, pharmacokinetics, and immunogenicity to the current formulation. This would reduce the time patients spend receiving their infusion—in clinic or in-home settings—lessening the overall burden on healthcare systems. We look forward to the opportunity to present these data during the virtually-held EHA meeting."

The U.S. Food and Drug Administration (FDA) is currently reviewing the company's supplemental biologics application for the ULTOMIRIS 100mg/mL formulation for the treatment of atypical hemolytic uremic syndrome (aHUS) and for adults with PNH. The agency has set a Prescription Drug User Fee Act (PDUFA) target action date of October 11, 2020. An application is also under review with the European Medicines Agency. The interim analysis that will be presented at EHA is from the extension period of the Phase 2 study evaluating the higher concentration of ULTOMIRIS in patients with PNH and is not inclusive of the aHUS patient population.

Additional data being presented at the meeting include analyses from the ongoing, observational real-world study of SOLIRIS® (eculizumab) as a treatment for PNH, along with a study evaluating the use of ALXN2040 (danicopan, formerly ACH-4471)—an investigational, oral, factor D inhibitor—used as an add-on therapy for the small, subgroup of PNH patients who are also diagnosed with extravascular hemolysis.

The accepted abstracts are listed below and are now available on the EHA website. All e-poster presentations will be available on the EHA website for the duration of the Congress.

e-Poster Presentations

An Interim Analysis of A Phase 2 Study Evaluating The Efficacy, Safety, and Pharmacokinetics of Intravenous Ravulizumab 100 mg/mL Formulation in Patients with Paroxysmal Nocturnal Hemoglobinuria. e-poster presentation, abstract ID#: EP862.

Development of A Composite Endpoint to Evaluate Treatment Benefit in Patients with Paroxysmal Nocturnal Hemoglobinuria. e-poster presentation, abstract ID#: EP845.

Effectiveness of Eculizumab Treatment in Patients Aged ≥65 Years with Paroxysmal Nocturnal Hemoglobinuria (PNH): Results from The International PNH Registry. e-poster presentation, abstract ID#: EP853.

Use of Prophylactic Antibiotics in Patients with Paroxysmal Nocturnal Hemoglobinuria Treated with Eculizumab. e-poster presentation, abstract ID#: EP866.

Effects of Oral, Factor D Inhibitor Danicopan on Transfusion Rates in Transfusion Dependent Paroxysmal Nocturnal Hemoglobinuria (PNH) Patients with A Sub-Optimal Response to Eculizumab: Phase 2 Study. E-poster presentation, abstract ID#: EP855.

About Paroxysmal Nocturnal Hemoglobinuria (PNH)
Paroxysmal nocturnal hemoglobinuria (PNH) is a serious ultra-rare blood disorder with devastating consequences. It is characterized by the destruction of red blood cells, which is also referred to as hemolysis. PNH occurs when the complement system—a part of the body's immune system —over-responds, leading the body to attack its own red blood cells. PNH often goes unrecognized, with delays in diagnosis from one to more than five years. Patients with PNH may experience a range of symptoms, such as fatigue, difficulty swallowing, shortness of breath, abdominal pain, erectile dysfunction, dark-colored urine and anemia. The most devastating consequence of chronic hemolysis is the formation of blood clots, which can occur in blood vessels throughout the body, damage vital organs, and potentially lead to premature death. PNH can strike men and women of all races, backgrounds and ages without warning, with an average age of onset in the early 30s.

About Atypical Hemolytic Uremic Syndrome (aHUS)
Atypical HUS is an ultra-rare disease that can cause progressive injury to vital organs, primarily the kidneys, via damage to the walls of blood vessels and blood clots. aHUS occurs when the complement system—a part of the body's immune system—over-responds, leading the body to attack its own

healthy cells. aHUS can cause sudden organ failure or a slow loss of function over time—potentially resulting in the need for a transplant, and in some cases, death. aHUS affects both adults and children, and many patients present in critical condition, often requiring supportive care, including dialysis, in an intensive care unit. The prognosis of aHUS can be poor in many cases, so a timely and accurate diagnosis—in addition to treatment—is critical to improving patient outcomes. Available tests can help distinguish aHUS from other hemolytic diseases with similar symptoms.

About ULTOMIRIS® (ravulizumab-cwvz)

ULTOMIRIS® (ravulizumab-cwvz) is the first and only long-acting C5 complement inhibitor. The medication works by inhibiting the C5 protein in the terminal complement cascade, a part of the body's immune system. When activated in an uncontrolled manner, the complement cascade over-responds, leading the body to attack its own healthy cells. ULTOMIRIS is administered intravenously every eight weeks or every four weeks for pediatric patients less than 20 kg, following a loading dose. ULTOMIRIS is approved in the United States (U.S.), European Union (EU) and Japan as a treatment for adults with paroxysmal nocturnal hemoglobinuria (PNH) and in the U.S. for atypical hemolytic uremic syndrome (aHUS) to inhibit complement-mediated thrombotic microangiopathy (TMA) in adult and pediatric (one month of age and older) patients. To learn more about the regulatory status of ULTOMIRIS in the countries that we serve, please visit www.alexion.com.

About SOLIRIS® (eculizumab)

SOLIRIS® (eculizumab) is a first-in-class C5 complement inhibitor. The medication works by inhibiting the C5 protein in the terminal complement cascade, a part of the body's immune system. When activated in an uncontrolled manner, the terminal complement cascade over-responds, leading the body to attack its own healthy cells. SOLIRIS is administered intravenously every two weeks, following an introductory dosing period. In many countries around the world, SOLIRIS is approved to treat paroxysmal nocturnal hemoglobinuria (PNH), atypical hemolytic uremic syndrome (aHUS), adults with generalized myasthenia gravis (gMG) who are acetylcholine receptor (AchR) antibody positive and/or adults with neuromyelitis optica spectrum disorder (NMOSD) who are anti-aquaporin-4 (AQP4) antibody positive. SOLIRIS is not indicated for the treatment of patients with Shiga-toxin E. coli-related hemolytic uremic syndrome (STEC-HUS). To learn more about the regulatory status of SOLIRIS in the countries that we serve, please visit www.alexion.com.

INDICATIONS & IMPORTANT SAFETY INFORMATION FOR ULTOMIRIS® (ravulizumab-cwvz) AND SOLIRIS® (eculizumab)

INDICATIONS

ULTOMIRIS and SOLIRIS are prescription medicines called monoclonal antibodies. ULTOMIRIS and SOLIRIS are used to treat adults with a disease called Paroxysmal Nocturnal Hemoglobinuria (PNH). ULTOMIRIS and SOLIRIS are also used to treat adults and children with a disease called atypical Hemolytic Uremic Syndrome (aHUS). Neither ULTOMIRIS nor SOLIRIS is for use in treating people with Shiga toxin E. coli related hemolytic uremic syndrome (STEC-HUS).

In addition, SOLIRIS is used to treat adults with a disease called generalized myasthenia gravis (gMG) who are anti-acetylcholine receptor (AChR) antibody positive. SOLIRIS is also used to treat adults with a disease called neuromyelitis optica spectrum disorder (NMOSD) who are anti-aquaporin-4 (AQP4) antibody positive.

It is not known if ULTOMIRIS is safe and effective in children with PNH or in children younger than one month of age in aHUS. It is also not known if SOLIRIS is safe and effective in children with PNH, gMG, or NMOSD.

IMPORTANT SAFETY INFORMATION

ULTOMIRIS and SOLIRIS are medicines that affect the immune system. These medicines can lower the ability of the immune system to fight infections. Both medicines increase 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 two weeks before the first dose of ULTOMIRIS or SOLIRIS if the patient has not already had this vaccine. If the patient's doctor decided that urgent treatment is needed, meningococcal vaccination should be administered as soon as possible. If the patient has not been vaccinated and therapy must be initiated immediately, two weeks of antibiotics should also be administered with the vaccinations. If the patient had a meningococcal vaccine in the past, additional vaccination might be needed before starting ULTOMIRIS or SOLIRIS. Patients should ask their doctor if an additional meningococcal vaccination is needed. Meningococcal vaccines reduce the risk of meningococcal infection but do not prevent all meningococcal infections. Patients should be instructed to call their 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. The doctor will provide a Patient Safety Card about the risk of meningococcal infection. This card must be carried at all times during treatment and for 8 months after the last ULTOMIRIS dose or 3 months after the last SOLIRIS dose.

Before a patient can receive ULTOMIRIS or SOLIRIS, their doctor must: enroll in the corresponding ULTOMIRIS REMS or SOLIRIS REMS program; counsel the patient about the risk of meningococcal infection; give the patient information and a Patient Safety Card about the symptoms and risk of meningococcal infection (as discussed above); and make sure that the patient is vaccinated with a meningococcal vaccine.

ULTOMIRIS and SOLIRIS may also increase the risk of other types of serious infections. Patients should talk to their doctor right away if they have any new signs or symptoms of infection.

Patients must not receive ULTOMIRIS or SOLIRIS if they have a meningococcal infection, or if they have not been vaccinated against meningococcal infection, unless their doctor decides that urgent treatment is needed.

Before a patient receives ULTOMIRIS or SOLIRIS, they should tell their doctor about all of their medical conditions, including if they: have an infection or fever, are pregnant or plan to become pregnant, and are breastfeeding or plan to breastfeed. It is not known if ULTOMIRIS or SOLIRIS will harm an unborn baby or if these medicines pass into the breast milk.

Patients should tell their doctor about all the medicines they take, including prescription and over-the-counter medicines, vitamins, and herbal supplements. ULTOMIRIS and SOLIRIS can affect how other medicines work, causing side effects.

For patients with PNH, the doctor will need to monitor the patient closely for at least 16 weeks after stopping ULTOMIRIS or 8 weeks after stopping SOLIRIS. Stopping treatment with these medicines may cause breakdown of the red blood cells due to PNH. Symptoms or problems that can happen

due to red blood cell breakdown include: a drop in red blood cell count, tiredness, blood in the urine, stomach-area (abdomen) pain, shortness of breath, blood clots, trouble swallowing, and erectile dysfunction (ED) in males.

For patients with aHUS, the doctor will need to monitor closely during and for at least 12 months after stopping ULTOMIRIS, or 12 weeks after stopping SOLIRIS, for signs of worsening aHUS symptoms or problems related to abnormal clotting and breakdown of red blood cells called thrombotic microangiopathy (TMA). Symptoms or problems that can happen with TMA may include: confusion or loss of consciousness, seizures, chest pain (angina), difficulty breathing and blood clots or stroke.

ULTOMIRIS can cause serious side effects including infusion reactions. Symptoms of an infusion reaction with ULTOMIRIS may include lower back pain, pain with the infusion, feeling faint or discomfort in the arms or legs. Patients should tell their doctor or nurse right away if they develop these symptoms, or any other symptoms during their ULTOMIRIS infusion that may mean they are 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.

The most common side effects of ULTOMIRIS in people treated for PNH are upper respiratory infection and headache. The most common side effects of ULTOMIRIS in people with aHUS are upper respiratory infection, diarrhea, nausea, vomiting, headache, high blood pressure and fever.

SOLIRIS can cause serious side effects including serious allergic reactions. Serious allergic reactions can happen during the SOLIRIS infusion. Patients should tell their doctor or nurse right away if they get 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 a patient 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. The most common side effects in people with NMOSD treated with SOLIRIS include: common cold (upper respiratory infection); pain or swelling of the nose or throat (nasopharyngitis); diarrhea; back pain; dizziness; flu like symptoms (influenza) including fever, headache, tiredness, cough, sore throat, and body aches; joint pain (arthralgia); throat irritation (pharyngitis), and bruising (contusion).

Tell your doctor about any side effect that bothers you or that does not go away. These are not all the possible side effects of ULTOMIRIS or SOLIRIS. For more information, ask your doctor or pharmacist. Call your doctor right away if you miss an ULTOMIRIS or SOLIRIS infusion or for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

Please refer to the full U.S. Prescribing Information and Medication Guide for ULTOMIRIS and SOLIRIS available via the links below, including the BOXED WARNING regarding serious and life-threatening meningococcal infections for both medicines.

ULTOMIRIS Full Prescribing Information and Medication Guide

SOLIRIS Full Prescribing Information and Medication Guide

About Alexion
Alexion is a global biopharmaceutical company focused on serving patients and families affected by rare and devastating diseases through the discovery, development and commercialization of life-changing medicines. 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) and atypical hemolytic uremic syndrome (aHUS), as well as the first and only approved complement inhibitor to treat anti-acetylcholine receptor (AchR) antibody-positive generalized myasthenia gravis (gMG) and 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 copper-binding agent for Wilson disease, an anti-neonatal Fc receptor (FcRn) antibody for rare Immunoglobulin G (IgG)-mediated diseases and an oral Factor D inhibitor as well as several early-stage therapies, including one for light chain (AL) amyloidosis, a second oral Factor D inhibitor and a third complement inhibitor. 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, metabolic disorders and cardiology. Headquartered in Boston, Massachusetts, Alexion 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.

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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 anticipated benefits of the ULTOMIRIS 100 mg/mL formulation (including to the patients and the healthcare system); the Company's commitment to advancing the understanding of PNH with ongoing real-world studies and emerging clinical data; and the anticipated timing of the review and decision of regulatory agencies with respect to the potential approval of certain of our product candidates. 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: ULTOMIRIS 100 mg/mL formulation may not generate the expected benefits to patients or the healthcare system that are anticipated; anticipated regulatory approvals may be delayed or refused; results of clinical trials may not be sufficient to satisfy regulatory authorities to approve ULTOMIRIS 100 mg/mL formulation as a treatment for PNH and/or aHUS (or they may request additional trials or additional information); results in clinical trials may not be indicative of results from later stage or larger clinical trials (or in broader patient populations once the product is approved for use by regulatory agencies); 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 discontinue sales of the product (or halt trials, delay or prevent us from making regulatory approval filings or result in denial of approval of our product candidates); the severity of the impact of the COVID-19 pandemic on Alexion's business, including on commercial and clinical trial and clinical development programs; unexpected delays in clinical trials; unexpected concerns regarding products and product candidates that may arise from additional data or analysis obtained during clinical trials or obtained once used by patients following product approval; future product improvements may not be realized due to expense or feasibility or other factors; 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; 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) and complete future clinical trials due to safety issues, IRB decisions, CMC-related issues, expense or unfavorable results from earlier trials (among other reasons); our dependence on sales from our complement inhibitors; future competition from biosimilars and novel 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 regulatory agencies regarding products and product candidates; uncertainty of long-term success in developing, licensing or acquiring other product candidates or additional indications for existing products; proposed acquisition of Portola by Alexion may not be completed; inability to complete acquisitions or grow the product pipeline through acquisitions (including due to failure to obtain antitrust approvals); the possibility that current rates of adoption of our products 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, lawsuits and challenges against us (including intellectual property lawsuits relating to ULTOMIRIS brought by third parties and inter parties review petitions submitted by third parties); the risk that third party payors (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; 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; adverse impacts on our supply chain, clinical trials, manufacturing operations, financial results, liquidity, hospitals, pharmacies and health care systems from natural disasters and global pandemics, including the coronavirus; 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, NMOSD, HPP and LAL-D and other indications we are pursuing are inaccurate; the risks of changing foreign exchange rates; risks relating to the potential effects of the Company's restructurings; 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 quarter ended March 31, 2020 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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