U.S. FDA Accepts Supplemental Biologics License Application (sBLA) for ULTOMIRIS® (ravulizumab-cwvz) under Priority Review for the Treatment of Atypical Hemolytic Uremic Syndrome (aHUS)

June 20, 2019

- FDA sets target action date of October 19, 2019-

BOSTON--(BUSINESS WIRE)--Jun. 20, 2019-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today announced that the U.S. Food and Drug Administration (FDA) has accepted for priority review the company's supplemental Biologics License Application (sBLA) for ULTOMIRIS ® (ravulizumab-cwvz), the company's long-acting C5 complement inhibitor, for the treatment of people with atypical hemolytic uremic syndrome (aHUS) in order to inhibit complement-mediated thrombotic microangiopathy (TMA). The FDA has set a target action date of October 19, 2019 under the Prescription Drug User Fee Act (PDUFA).

"This acceptance is an important step in our efforts to deliver a potential new standard of care to people living with this devastating disease," said John Orloff, M.D., Executive Vice President and Head of Research & Development at Alexion. "We look forward to working closely with the FDA to facilitate a rapid review of this application."

Atypical HUS, also known as complement-mediated TMA, is a severe and chronic ultra-rare disease that can cause progressive damage to vital organs, predominantly the kidneys, leading to kidney failure and premature death.

The sBLA is based on previously announced results, which were recently presented at the European Renal Association – European Dialysis and Transplant Association (ERA-EDTA) Congress, from the Phase 3 study of ULTOMIRIS in people with aHUS, which met the primary endpoint of complete TMA response, defined by hematologic normalization and improved kidney function.

Important ULTOMIRIS Safety Information

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.

ULTOMIRIS 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, are pregnant or plan to become pregnant, and are breastfeeding or plan 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.

Please see the full Prescribing Information and Medication Guide for ULTOMIRIS, including Boxed WARNING regarding serious and life-threatening

meningococcal infections/sepsis.

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.

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Forward-Looking Statement
This press release contains forward-looking statements, including statements related to: the belief that ULTOMIRIS has the potential to become the new standard of care for patients with aHUS in order to inhibit complement-mediated thrombotic microangiopathy; the Company will work closely with FDA in order to facilitate a rapid review of this sBLA; the Company's plans to make future regulatory filings for approval of certain products and product candidates and the timing of such filings; potential future regulatory approval for the marketing of ULTOMIRIS and other products and product candidates; and the potential benefits of current products and products under development and in clinical trials (including ULTOMIRIS as a treatment for patients with PNH). Forward-looking statements are subject to factors that may cause Alexion's results and plans to differ materially from those forward-looking statements, including for example: the FDA and other regulatory agencies do not approve the use of ULTOMIRIS as a therapy for patients with aHUS in order to inhibit complement-mediated thrombotic microangiopathy; the sBLA and other applications for approval of products are not deemed sufficient by the appropriate regulatory authorities and are not approved (or require additional information or data which may be time consuming to generate); 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; any potential post-approval restrictions that the FDA, European Commission, the EMA or any other regulatory agency may impose on ULTOMIRIS; ULTOMIRIS and other products and product candidates do not gain regulatory approval from the FDA, European Commission, MHLW, EMA or other regulatory authorities; our products, including ULTOMIRIS do not gain acceptance among patients and/or physicians and do not become the standard of care for certain indications; 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 principal product (SOLIRIS); our inability to facilitate the timely conversion of PNH patients (and patient with any future indications) from SOLIRIS to ULTOMIRIS; regulatory agencies do not accept the proposed indications for our product label (or approves a product with a for an indication that is for a limited patient population); payer, physician and patient acceptance of ULTOMIRIS as an alternative to SOLIRIS; appropriate pricing for ULTOMIRIS; future competition from biosimilars and novel products (and that this future competition causes, among other things, ULTOMIRIS not to be the standard of care for certain indications); 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, increased 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, European Commission, the EMA, MHLW and other regulatory agencies regarding products and product candidates; results in early stage clinical trials may not be indicative of full results or results from later stage or larger clinical trials (or in 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, discontinue sales of our products, delay or prevent us from making regulatory approval filings or result in denial of approval of our product candidates; 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 following commercialization); future product improvements may not be realized due to expense or feasibility or other factors; 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 partes 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; 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; 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 indications we are pursuing are inaccurate; the risks of changing foreign exchange rates; 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, 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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