# Alexion Announces Data Presentations at 61st American Society of Hematology Annual Meeting

November 6, 2019

*- Accepted abstracts include presentations of ULTOMIRIS® (ravulizumab-cwvz) Phase 3 PNH data through 52 weeks -*

*- ULTOMIRIS is the first and only long-acting C5 complement inhibitor, now available in the U.S. for the treatment of PNH and aHUS -*

BOSTON--(BUSINESS WIRE)--Nov. 6, 2019-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today announced that five scientific data presentations from the company's complement program will be showcased during the annual meeting of the American Society of Hematology (ASH) in Orlando, Fla., from Dec. 7 to 10, 2019. During the Congress, researchers will present new, long-term safety and efficacy findings through 52 weeks of treatment from the Phase 3 trial of ULTOMIRIS® (ravulizumab-cwvz) in adult patients with paroxysmal nocturnal hemoglobinuria (PNH) who had previously received eculizumab treatment, as well as data on breakthrough hemolysis in patients treated with ULTOMIRIS during the extension phase (weeks 27-52) of the two Phase 3 studies involving adult patients with PNH, which together comprise the largest PNH clinical program ever conducted. Collectively, the breadth of the data to be presented at ASH demonstrates the company's continued commitment to improving the care for people living with rare, complement-mediated diseases.

ULTOMIRIS is approved in the United States (U.S.), Canada, European Union (EU) and Japan for the treatment of adult patients with PNH. ULTOMIRIS was also recently approved by the U.S. Food and Drug Administration for the treatment of atypical hemolytic uremic syndrome (aHUS) to inhibit complement-mediated thrombotic microangiopathy (TMA) in adult and pediatric (one month of age and older) patients. ULTOMIRIS is the first and only long-acting complement inhibitor that provides immediate and complete C5 inhibition sustained with every eight-week dosing in adult patients.

The accepted abstracts are listed below and are now available on the ASH website.

**Paroxysmal Nocturnal Hemoglobinuria (PNH) Abstracts**
Breakthrough Hemolysis in Adult Patients with Paroxysmal Nocturnal Hemoglobinuria Treated with Ravulizumab: Results of a 52-Week Extension from Two Phase 3 Studies. Abstract ID#: 952 – Poster Presentation, Dec. 7, 2019, 5:30 – 7:30 p.m. EST, Hall B.

One-Year Efficacy and Safety from A Phase 3 Trial of Ravulizumab in Adult Patients with Paroxysmal Nocturnal Hemoglobinuria Receiving Prior Eculizumab Treatment. Abstract ID#: 2231 – Poster Presentation, Dec. 8, 2019, 6:00 – 8:00 p.m. EST, Hall B.

Comparison of Lost Productivity Due to Eculizumab and Ravulizumab Treatments for Paroxysmal Nocturnal Hemoglobinuria in France, Germany, Italy, Russia, Spain, the United Kingdom, and the United States. Available online.

Prophylactic Antibiotic Use and Risk of Meningococcal Infections in Patients with Paroxysmal Nocturnal Hemoglobinuria (PNH) Treated with Eculizumab Who Received Meningococcal Vaccination: Results from the International PNH Registry. Available online.

**Atypical Hemolytic Uremic Syndrome (aHUS) Abstracts**
Discordance between Free C5 and CH50 Complement Assays in Measuring Complement C5 Inhibition in Patients with aHUS Treated with Ravulizumab. Abstract ID#: 1099 – Poster Presentation, Dec. 7, 2019, 5:30 – 7:30 p.m. EST, Hall B.

**About Paroxysmal Nocturnal Hemoglobinuria (PNH)**
Paroxysmal nocturnal hemoglobinuria (PNH) is a serious ultra-rare blood disorder with potentially 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 healthy 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. Patients with certain types of hemolytic anemia, bone marrow disorders and unexplained venous or arterial thrombosis are at increased risk of PNH.

**About Atypical Hemolytic Uremic Syndrome (aHUS)**
Atypical hemolytic uremic syndrome (aHUS) is an ultra-rare disease that affects both children and adults and can lead to potentially irreversible damage to kidneys and other vital organs, sudden or progressive kidney failure (requiring dialysis or transplant) and premature death. aHUS is characterized by inflammation and the formation of blood clots in small blood vessels throughout the body (thrombotic microangiopathy [TMA]) mediated by chronic, uncontrolled activation of the complement system, which is part of the body's immune system. TMA consists of reduced platelet count (thrombocytopenia), hemolytic anemia (as a result of hemolysis [destruction of red blood cells]) and acute kidney injury (AKI). If left untreated, significant proportions of adults (46 percent) and children (16 percent) can progress to end-stage renal disease (ESRD) or die during first clinical manifestations of aHUS despite supportive care, including plasma exchange or plasma infusion (PE/PI). One year following clinical manifestations, 56 percent of adults and 29 percent of children can progress to ESRD or die, if left untreated. Early and careful diagnosis of aHUS is critical, as many coexisting diseases and events are known or suspected to activate the complement cascade, and as patients may not necessarily present with the classic TMA triad of thrombocytopenia, hemolytic anemia and renal impairment or may have less severe renal involvement. Available tests can help distinguish aHUS from other hemolytic diseases with similar symptoms such as HUS caused by Shiga toxin-producing Escherichia coli (STEC-HUS) and thrombotic thrombocytopenic purpura (TTP).

**About ULTOMIRIS® (ravulizumab-cwvz)**

ULTOMIRIS (ravulizumab-cwvz) is the first and only long-acting C5 complement inhibitor. It is administered intravenously every eight weeks or every four weeks for pediatric patients less than 20 kg, following a loading dose. 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. ULTOMIRIS is approved in the U.S., Japan, and the EU as a treatment for adults with PNH and in the U.S. for aHUS to inhibit complement-mediated thrombotic microangiopathy (TMA) in adult and pediatric (one month of age and older) patients.

You can read more about the study results for this clinical program on www.alexion.com.

**About SOLIRIS® (eculizumab)**

SOLIRIS® (eculizumab) 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. The terminal complement cascade, when activated in an uncontrolled manner, plays a role in severe rare and ultra-rare disorders. SOLIRIS, an intravenously administered therapy, is approved in the U.S., EU, Japan and other countries as a treatment for adult patients with PNH and for adults and children with aHUS. SOLIRIS is not indicated for the treatment of patients with Shiga-toxin E. coli-related hemolytic uremic syndrome (STEC-HUS). In the U.S., SOLIRIS is also approved for the treatment of generalized MG (gMG) in adult patients who are anti-AchR antibody-positive and for the treatment of neuromyelitis optica spectrum disorder (NMOSD) in adult patients who are anti-AQP4 antibody-positive, in the EU as the first and only treatment for refractory gMG in adults who are anti-AchR antibody-positive and for the treatment of NMOSD in adult patients who are anti-AQP4 antibody-positive with a relapsing course of the disease, and in Japan for the treatment of patients with gMG who are anti-AChR antibody-positive and whose symptoms are difficult to control with high-dose intravenous immunoglobulin (IVIG) therapy or plasmapheresis (PLEX).

**INDICATIONS & IMPORTANT SAFETY INFORMATION FOR ULTOMIRIS® (ravulizumab-cwvz) 300 mg / 30 mL injection for intravenous use**

**INDICATIONS**

ULTOMIRIS is a prescription medicine called a monoclonal antibody. ULTOMIRIS is used to treat adults with a disease called Paroxysmal Nocturnal Hemoglobinuria (PNH). ULTOMIRIS is used to treat adults and children 1 month of age and older with a disease called atypical Hemolytic Uremic Syndrome (aHUS). ULTOMIRIS is not used in treating people with Shiga toxin E. coli related hemolytic uremic syndrome (STEC-HUS). It is not known if ULTOMIRIS is safe and effective in children with PNH. It is not known if ULTOMIRIS is safe and effective in children younger than 1 month of age.

**IMPORTANT SAFETY INFORMATION**

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. One's doctor will decide if additional meningococcal vaccination is needed. Meningococcal vaccines reduce the risk of meningococcal infection but do not prevent all meningococcal infections. 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. One's doctor will give a Patient Safety Card about the risk of meningococcal infection. Carry the card at all times during treatment and for 8 months after the last ULTOMIRIS dose.

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 doctor. Call the doctor right away if one has any new signs or symptoms of infection.

Do not receive ULTOMIRIS if one has a meningococcal infection, or has not been vaccinated against meningococcal infection unless the doctor decides that urgent treatment with ULTOMIRIS is needed.

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 breastfeed 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 medicines 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 and 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 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. If one has aHUS, the doctor will need to monitor closely for at least 12 months after stopping treatment for signs of worsening aHUS symptoms or problems related to a type of abnormal clotting and breakdown of the 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. If one misses an ULTOMIRIS infusion, call the doctor right away.

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, feeling faint or discomfort in the arms or legs. 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 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 infections, diarrhea, nausea, vomiting, headache, high blood pressure, and fever.

**Please see the accompanying full Prescribing Information and Medication Guide for ULTOMIRIS, including Boxed WARNING regarding serious and life-threatening meningococcal infections/sepsis.**

**INDICATIONS & IMPORTANT SAFETY INFORMATION FOR SOLIRIS® (eculizumab) 300 mg / 30 mL injection for intravenous use**

**INDICATIONS**
**What is SOLIRIS?**
SOLIRIS is a prescription medicine called a monoclonal antibody. SOLIRIS is used to treat patients with a disease called Paroxysmal Nocturnal Hemoglobinuria (PNH). SOLIRIS is used to treat 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). SOLIRIS is used to treat adults with a disease called generalized myasthenia gravis (gMG) who are anti-acetylcholine receptor (AchR) antibody positive. SOLIRIS is 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 SOLIRIS is safe and effective in children with PNH, gMG, or NMOSD.

**IMPORTANT SAFETY INFORMATION**
SOLIRIS 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 two 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, two 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. 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. 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. One's doctor will provide a Patient Safety Card about the risk of meningococcal infection. Carry the card at all times during treatment and for 3 months after the last SOLIRIS dose.

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.

Do not receive SOLIRIS if one has a meningococcal infection, or has not been vaccinated against meningitis infection unless one's doctor decides that urgent treatment with SOLIRIS is needed.

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 or 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 during and for at least 12 weeks after stopping treatment 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 the 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. 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).

**Please see the accompanying full Prescribing Information and Medication Guide for SOLIRIS, including Boxed WARNING regarding serious**

**and life-threatening meningococcal infections.**

**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) 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 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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