



Alexion Announces Upcoming Data Presentations at the American Society of Nephrology's Virtual Kidney Week 2020

October 9, 2020

- Four abstracts accepted for presentation, including new clinical trial analyses that provide further support for the safety and efficacy of ULTOMIRIS® (ravulizumab-cwvz) for the treatment of pediatric patients with atypical hemolytic uremic syndrome (aHUS) -

BOSTON--(BUSINESS WIRE)--Oct. 9, 2020-- [Alexion Pharmaceuticals, Inc.](#) (NASDAQ:ALXN) today announced that four abstracts have been accepted for presentation at the American Society of Nephrology's Kidney Week 2020, taking place virtually from October 22 to 25, 2020. New data will be presented from Alexion's pivotal Phase 3, single-arm trial evaluating the efficacy and safety of ULTOMIRIS® (ravulizumab-cwvz) to resolve thrombotic microangiopathy (TMA) in pediatric patients with atypical hemolytic uremic syndrome (aHUS). The data demonstrate that ULTOMIRIS improved hematologic and renal outcomes by 50 weeks in 94 percent of pediatric patients who had not previously received treatment with a complement inhibitor, without any unexpected safety concerns, when administered every four or eight weeks (depending on body weight). Additionally, new data will be presented that shows results from a switch study involving pediatric patients with aHUS from stable treatment with SOLIRIS® (eculizumab) to treatment either every four or eight weeks (depending on body weight) with ULTOMIRIS. The study demonstrated continued efficacy, presented no additional safety concerns and the benefit of reduced dosing frequency.

Alexion also plans to present on the comparative efficacy of ULTOMIRIS and SOLIRIS in the treatment of adults with aHUS. Data from Phase 3 clinical trials show there are no significant differences in outcomes between patients treated with SOLIRIS versus those treated with ULTOMIRIS at 26 weeks, after balancing patient characteristics between the study groups.

"ULTOMIRIS is quickly emerging as the new standard of care for patients living with atypical hemolytic uremic syndrome," said John Orloff, M.D., Executive Vice President and Head of Research and Development at Alexion. "The data we are presenting during the American Society of Nephrology's virtual meeting reinforce our commitment to advancing the understanding of the safety and efficacy of ULTOMIRIS among different patient populations—including pediatric patients—regardless of their previous treatment experience. We look forward to sharing these data at the upcoming Congress, which continues to provide the scientific community with valuable insights into ongoing research in nephrology, despite the meeting being held virtually this year."

The accepted abstracts are listed below and are now available on the [ASN website](#):

Oral Presentations

Characteristics and Outcomes of Pregnancy-Triggered Atypical Hemolytic-Uremic Syndrome: Global aHUS Registry Analysis. Abstract ID #SU-OR40 – oral presentation, October 25, 2020, 5:00 p.m. ET.

ePoster Presentations

Efficacy and Safety of Ravulizumab in Pediatric Patients with Atypical Hemolytic Uremic Syndrome Naïve to Complement Inhibitor Treatment: 26-week and 1-year Data. Abstract ID #PO2358 – poster presentation, October 22, 2020, 10:00 a.m. ET.

Efficacy and Safety of Ravulizumab in Pediatric Patients with Atypical Hemolytic Uremic Syndrome Previously Treated with Eculizumab: 26-week and 1-year Data. Abstract ID #PO2331 – poster presentation, October 22, 2020, 10:00 a.m. ET.

Comparative Efficacy of Ravulizumab and Eculizumab in the Treatment of Atypical Hemolytic Uremic Syndrome: An Indirect Comparison Using Clinical Trial Data. Abstract ID #PO1851 – poster presentation, October 22, 2020, 10:00 a.m. ET.

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. Atypical HUS occurs when the complement system—a part of the body's immune system—over-responds, leading the body to attack its own healthy cells. Atypical HUS 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. Atypical HUS 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). It is also approved in the U.S. and Japan for atypical hemolytic uremic syndrome (aHUS) to inhibit complement-mediated thrombotic microangiopathy (TMA) in adult and pediatric (one month of age and older) patients, as well as in the EU for the treatment of adults and children with a body weight of at least 10 kg with aHUS. 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)

INDICATIONS

What is ULTOMIRIS?

ULTOMIRIS is a prescription medicine used to treat:

- adults with a disease called Paroxysmal Nocturnal Hemoglobinuria (PNH).
 - 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

What is the most important information I should know about ULTOMIRIS?

ULTOMIRIS is a medicine that affects your immune system and can lower the ability of your immune system to fight infections.

- ULTOMIRIS increases your chance of getting serious and life-threatening meningococcal infections that may quickly become life-threatening and cause death if not recognized and treated early.
1. You must receive meningococcal vaccines at least 2 weeks before your first dose of ULTOMIRIS if you are not vaccinated.
 2. If your doctor decided that urgent treatment with ULTOMIRIS is needed, you should receive meningococcal vaccination as soon as possible.
 3. If you have not been vaccinated and ULTOMIRIS therapy must be initiated immediately, you should also receive 2 weeks of antibiotics with your vaccinations.
 4. If you had a meningococcal vaccine in the past, you might need additional vaccination. Your doctor will decide if you need additional vaccination.
 5. Meningococcal vaccines reduce but do not prevent all meningococcal infections. Call your doctor or get emergency medical care right away if you get any of these signs and symptoms of a meningococcal infection: 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.

Your doctor will give you a Patient Safety Card about the risk of meningococcal infection. Carry it with you at all times during treatment and for 8 months after your last ULTOMIRIS dose. It is important to show this card to any doctor or nurse to help them diagnose and treat you quickly.

ULTOMIRIS is only available through a program called the ULTOMIRIS REMS. Before you can receive ULTOMIRIS, your doctor must: enroll in the ULTOMIRIS REMS program; counsel you about the risk of meningococcal infection; give you information and a **Patient Safety Card** about the symptoms and your risk of meningococcal infection (as discussed above); and make sure that you are vaccinated with a meningococcal vaccine.

ULTOMIRIS may also increase the risk of other types of serious infections. Call your doctor right away if you have any new signs or symptoms of infection.

Who should not receive ULTOMIRIS?

Do not receive ULTOMIRIS if you have a meningococcal infection or have not been vaccinated against meningococcal infection unless your doctor decides that urgent treatment with ULTOMIRIS is needed.

Before you receive ULTOMIRIS, tell your doctor about all of your medical conditions, including if you: 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 will harm your unborn baby or if it passes into your breast milk. You should not breastfeed during treatment and for 8 months after your final dose of ULTOMIRIS.

Tell your doctor about all the vaccines you receive and medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements which could affect your treatment.

If you have PNH and you stop receiving ULTOMIRIS, your doctor will need to monitor you closely for at least 16 weeks after you stop ULTOMIRIS. Stopping ULTOMIRIS may cause breakdown of your red blood cells due to PNH. Symptoms or problems that can happen due to

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

If you have aHUS, your doctor will need to monitor you closely for at least 12 months after stopping treatment for signs of worsening aHUS or problems related to a type of abnormal clotting and breakdown of your 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.

What are the possible side effects of ULTOMIRIS?

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 your arms or legs. Tell your doctor or nurse right away if you develop these symptoms, or any other symptoms during your ULTOMIRIS infusion that may mean you are having a serious infusion reaction, including: chest pain, trouble breathing or shortness of breath, swelling of your 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.

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. For more information, ask your doctor or pharmacist. Call your doctor right away if you miss an ULTOMIRIS infusion or for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

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)

INDICATIONS

What is SOLIRIS?

SOLIRIS is a prescription medicine 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).
- adults with a disease called generalized myasthenia gravis (gMG) who are anti-acetylcholine receptor (AChR) antibody positive.
- 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

What is the most important information I should know about SOLIRIS?

SOLIRIS is a medicine that affects your immune system and can lower the ability of your immune system to fight infections.

- **SOLIRIS increases your chance of getting serious and life-threatening meningococcal infections that may quickly become life-threatening and cause death if not recognized and treated early.**
1. You must receive meningococcal vaccines at least 2 weeks before your first dose of SOLIRIS if you are not vaccinated.
 2. If your doctor decided that urgent treatment with SOLIRIS is needed, you should receive meningococcal vaccination as soon as possible.
 3. If you have not been vaccinated and SOLIRIS therapy must be initiated immediately, you should also receive two weeks of antibiotics with your vaccinations.
 4. If you had a meningococcal vaccine in the past, you might need additional vaccination. Your doctor will decide if you need additional vaccination.
 5. Meningococcal vaccines reduce but do not prevent all meningococcal infections. Call your doctor or get emergency medical care right away if you get any of these signs and symptoms of a meningococcal infection: 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.

Your doctor will give you a Patient Safety Card about the risk of meningococcal infection. Carry it with you at all times during treatment and for 3 months after your last SOLIRIS dose. It is important to show this card to any doctor or nurse to help them diagnose and treat you quickly.

SOLIRIS is only available through a program called the SOLIRIS REMS. Before you can receive SOLIRIS, your doctor must enroll in the SOLIRIS REMS program; counsel you about the risk of meningococcal infection; give you information and a **Patient Safety Card** about the symptoms and your risk of meningococcal infection (as discussed above); and make sure that you are vaccinated with the meningococcal vaccine and, if needed, get

revaccinated with the meningococcal vaccine. Ask your doctor if you are not sure if you need to be revaccinated.

SOLIRIS may also increase the risk of other types of serious infections. Make sure your child receives vaccinations against *Streptococcus pneumoniae* and *Haemophilus influenzae* type b (Hib) if treated with SOLIRIS. Certain people may be at risk of serious infections with gonorrhea. Certain fungal infections (*Aspergillus*) may occur if you take SOLIRIS and have a weak immune system or a low white blood cell count.

Who should not receive SOLIRIS?

Do not receive SOLIRIS if you have a meningococcal infection or have not been vaccinated against meningitis infection unless your doctor decides that urgent treatment with SOLIRIS is needed.

Before you receive SOLIRIS, tell your doctor about all of your medical conditions, including if you: have an infection or fever, are pregnant or plan to become pregnant, and are breastfeeding or plan to breastfeed. It is not known if SOLIRIS will harm your unborn baby or if it passes into your breast milk.

Tell your doctor about all the vaccines you receive and medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements which could affect your treatment. It is important that you have all recommended vaccinations before you start SOLIRIS, receive 2 weeks of antibiotics if you immediately start SOLIRIS, and stay up-to-date with all recommended vaccinations during treatment with SOLIRIS.

If you have PNH, your doctor will need to monitor you closely for at least 8 weeks after stopping SOLIRIS. Stopping treatment with SOLIRIS may cause breakdown of your red blood cells due to PNH. Symptoms or problems that can happen due to red blood cell breakdown include: drop in the number of your red blood cell count, drop in your platelet count, confusion, kidney problems, blood clots, difficulty breathing, and chest pain.

If you have aHUS, your doctor will need to monitor you 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, swelling in arms or legs, and a drop in your platelet count.

What are the possible side effects of SOLIRIS?

SOLIRIS can cause serious side effects including serious allergic reactions. Tell your doctor or nurse right away if you get any of these symptoms during your SOLIRIS infusion: chest pain, trouble breathing or shortness of breath, swelling of your face, tongue, or throat, and feel faint or pass out. If you have an allergic reaction to SOLIRIS, your 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 your 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 your 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 your 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 SOLIRIS. For more information, ask your doctor or pharmacist. Call your doctor for medical advice about side effects. You are encouraged to report negative side effects of prescription drugs to the FDA. Visit MedWatch, or call 1-800-FDA-1088.

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 and devastating conditions through the discovery, development and commercialization of life-changing medicines. As a leader in rare diseases for more than 25 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) as well as the first and only approved Factor Xa inhibitor reversal agent. 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 safety and efficacy of ULTOMIRIS for the treatment of pediatric patients with aHUS; the safety and efficacy of ULTOMIRIS to resolve TMA in pediatric patients with aHUS; ULTOMIRIS is quickly emerging as the new standard of care for patients living

with atypical hemolytic uremic syndrome; and a timely and accurate diagnosis of aHUS — in addition to treatment — is critical to improving patient outcomes. 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: anticipated safety profile and the benefits of ULTOMIRIS to resolve TMA in pediatric patients with aHUS may not be realized; results of clinical trials may not be sufficient to satisfy regulatory authorities (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 after the product is approved for use by regulatory agencies); the severity of the impact of the COVID-19 pandemic on Alexion's business, including on commercial 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 principal product (SOLIRIS); 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 failure of product candidates to obtain regulatory approval; 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 our products and product candidates; uncertainty of long-term success in developing, licensing or acquiring other product candidates or additional indications for existing products; 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); 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 or that tax liabilities exceed current expectations; 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 COVID-19; uncertainties surrounding legal proceedings, company investigations and government investigations; 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 restructuring; 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 June 30, 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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