Alexion Announces Upcoming Data Presentations at the 73rd Annual Meeting of the American Academy of Neurology

March 4, 2021

– Data highlight long-term and real-world efficacy of SOLIRIS® (eculizumab) in patients living with rare neurologic complement-mediated disorders, including neuromyelitis optica spectrum disorder (NMOSD) and generalized myasthenia gravis (gMG) –

BOSTON--(BUSINESS WIRE)--Mar. 4, 2021-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today announced that three abstracts have been accepted for presentation at the 73rd annual meeting of the American Academy of Neurology (AAN), taking place virtually from April 17 through April 22, 2021. New real-world data will be presented evaluating SOLIRIS® (eculizumab) for the treatment of generalized myasthenia gravis (gMG) in the United States, suggesting substantial reductions in myasthenic crises, exacerbations and related hospitalizations, consistent with results from the Phase 3 REGAIN clinical trial and the open-label extension. Additionally, long-term data on SOLIRIS for the treatment of neuromyelitis optica spectrum disorder (NMOSD) will be presented, including a disease model assessing the benefits of treatment and Phase 3 PREVENT clinical trial results evaluating SOLIRIS as a monotherapy.

The accepted abstracts are listed below and are now available on the AAN website. Posters will be available throughout the duration of the Congress.

Neuromyelitis Optica Spectrum Disorder (NMOSD)
Long-Term Efficacy and Safety of Eculizumab Monotherapy in AQP4+ Neuromyelitis Optica Spectrum Disorder. Oral presentation, Program Number S29.004, Session S29: Autoimmune Neurology: Clinical Trials, Treatment, and Diagnosis of CNS and PNS Autoimmune Neurologic Disorders, April 21, 2021, 4:00 p.m. – 5:00 p.m. Eastern Time.

The Potential Impact of Long-Term Relapse Reduction: A Disease Model of Eculizumab in Neuromyelitis Optica Spectrum Disorder. ePoster presentation, Program Number P15.055, Session P15: MS Clinical Practice and Decision Making.

Generalized Myasthenia Gravis (gMG)
Real-World Use of Eculizumab in Generalized Myasthenia Gravis in the United States: Results from a Pilot Retrospective Chart-Review Study. ePoster presentation, Program Number P2.062, Session P2: Autoimmune Neurology: Inflammatory Neuropathies and Stiff Person Syndrome.

About Neuromyelitis Optica Spectrum Disorder (NMOSD)
Neuromyelitis Optica Spectrum Disorder (NMOSD) is a rare autoimmune disease of the central nervous system (CNS). Approximately three-quarters of NMOSD patients have anti-AQP4 antibody-positive NMOSD. In patients with these antibodies, NMOSD occurs when the complement system—a part of the body's immune system—over-responds—leading the body to primarily attack the optic nerves and/or spinal cord in the CNS. People living with NMOSD often experience unpredictable attacks, also referred to as relapses, which tend to be severe and recurrent and may result in permanent disability. The most common symptoms of NMOSD are optic neuritis, which can cause visual problems including blindness, and transverse myelitis, which can cause mobility problems including paralysis. The disease primarily affects women, with an average age of onset of 39 years. NMOSD is more common and more severe in non-Caucasian populations worldwide.

About Generalized Myasthenia Gravis (gMG)
Myasthenia gravis (MG) is a rare, progressive, autoimmune neuromuscular disease. In patients with anti-acetylcholine receptor (AchR) antibody-positive MG, the body's own immune system over-responds, leading the body to attack its own healthy cells and produce antibodies to fight against AchR, a receptor located on muscle cells at the neuromuscular junction. As a result, communication between the nerves and muscles is impaired, leading to a loss of normal muscle function. MG typically begins with weakness in the muscles that control the movements of the eyes and eyelids and often progresses to the more severe and generalized form, known as generalized myasthenia gravis (gMG). People with gMG can suffer from slurred speech, choking, difficulty swallowing, drooping of the eyelids, double or blurred vision, disabling fatigue, immobility requiring assistance, shortness of breath and episodes of respiratory failure that can be life-threatening. Complications, exacerbations and myasthenic crises can require hospital and intensive care unit admissions with prolonged stays. gMG can occur at any age but most commonly begins before the age of 40 in women and after the age of 60 in men.

About SOLIRIS®
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 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 infusion-related 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 infusion-related 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 hematology, nephrology, neurology, metabolic disorders, cardiology, ophthalmology and acute care. 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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