Alexion Announces Upcoming Data Presentations at 72nd Annual Meeting of the American Academy of Neurology

March 5, 2020

- Nine abstracts accepted, including long-term data and subgroup analyses from the Phase 3 PREVENT clinical trial evaluating SOLIRIS® (eculizumab) for the treatment of neuromyelitis optica spectrum disorder (NMOSD) -

- Breadth of data to be presented further support the significant and sustained relapse reduction demonstrated by SOLIRIS in the treatment of adults with NMOSD who are anti-aquaporin-4 (AQP4) antibody-positive -

BOSTON--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today announced that nine abstracts have been accepted for presentation at the 72nd annual meeting of the American Academy of Neurology (AAN) in Toronto, Ontario, Canada from April 25 through May 1, 2020. Long-term safety and efficacy results from the Phase 3 PREVENT study and open-label extension of SOLIRIS® (eculizumab) in adults with neuromyelitis optica spectrum disorder (NMOSD) who are anti-aquaporin-4 (AQP4) antibody-positive will be presented, demonstrating the percentage of SOLIRIS patients who were relapse-free remained high (94 percent) through 192 weeks. Additional PREVENT data to be presented highlight the efficacy of SOLIRIS and its impact on health outcomes, hospitalization rates and relapse treatment, disability worsening and quality of life, and the evidence of benefit of SOLIRIS across a broad range of patients with NMOSD.

The accepted abstracts are listed below and are now available on the AAN website:

Oral Presentations

Long-Term Safety and Efficacy of Eculizumab in Neuromyelitis Optica Spectrum Disorder. Oral presentation during session S34: Clinical Trials and Therapeutics in Autoimmune Neurology, April 28, 2020, 4:30 p.m. ET.

Efficacy and Safety of Eculizumab in Patients with Neuromyelitis Optica Spectrum Disorder Previously Treated with Rituximab: Findings from the Phase 3 PREVENT Study. Oral presentation during session S34: Clinical Trials and Therapeutics in Autoimmune Neurology, April 28, 2020, 4:18 p.m. ET.

Poster Presentations

Impact of Eculizumab on Health Outcomes in Patients with Aquaporin-4 Antibody-Positive Neuromyelitis Optica Spectrum Disorder: Findings from the PREVENT Study. Poster presentation during session P14: Autoimmune Neurology: NMOSD and MOG 2, April 30, 2020, 8:00 – 9:00 a.m. ET and session P17: Neuroinflammation, May 1, 2020, 11:30 a.m. – 1:00 p.m. ET.

Impact of Eculizumab on Hospitalization Rates and Relapse Treatment in Patients with Aquaporin-4 Antibody-Positive Neuromyelitis Optica Spectrum Disorder: the Phase 3 PREVENT Study. Poster presentation during session P14: Autoimmune Neurology: NMOSD and MOG 2, April 30, 2020, 8:00 – 9:00 a.m. ET and session P17: Neuroinflammation, May 1, 2020, 11:30 a.m. – 1:00 p.m. ET.

Impact of Eculizumab on Disability Worsening and Quality of Life in Patients with Aquaporin-4 Antibody-Positive Neuromyelitis Optica Spectrum Disorder: Results from the Phase 3 PREVENT Study. Poster presentation during session P14: Autoimmune Neurology: NMOSD and MOG 2, April 30, 2020, 8:00 – 9:00 a.m. ET and session P17: Neuroinflammation, May 1, 2020, 11:30 a.m. – 1:00 p.m. ET.

Infection Risk in Patients with Complement-Mediated Neurological Disorders Receiving Eculizumab: Findings from Two Phase 3 Studies and Their Extensions in Aquaporin-4 Antibody-Positive Neuromyelitis Optica Spectrum Disorder (AQP4+ NMOSD) and Acetylcholine-Receptor Antibody-Positive Refractory Generalized Myasthenia Gravis (AChR+ gMG). Poster presentation during session P14: Autoimmune Neurology: NMOSD and MOG 2, April 30, 2020, 8:00 – 9:00 a.m. ET.

The Impact of Relapses on Quality of Life in Patients with Neuromyelitis Optica Spectrum Disorder: Data from the Phase 3 PREVENT Study. Poster presentation during session P14: Autoimmune Neurology: NMOSD and MOG 2, April 30, 2020, 8:00 – 9:00 a.m. ET.

Benefit of Eculizumab for a Broad Range of Patients with Aquaporin-4 Antibody-Positive Neuromyelitis Optica Spectrum Disorder: Findings from the Phase 3 PREVENT Study. Poster presentation during session P14: Autoimmune Neurology: NMOSD and MOG 2, April 30, 2020, 8:00 – 9:00 a.m. ET and session P17: Neuroinflammation, May 1, 2020, 11:30 a.m. – 1:00 p.m. ET.

Neuromyelitis Optica Spectrum Disorder (NMOSD): Epidemiology, Treatments, and Outcomes in a Single Center. Poster presentation during session P14: Autoimmune Neurology: NMOSD and MOG 2, April 30, 2020, 8:00 – 9:00 a.m. ET.

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® (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 SOLIRIS® (eculizumab) 300 mg / 30 mL injection for intravenous use

U.S. 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.

U.S. 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 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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