Alexion Announces Upcoming Data Presentations at MSVirtual2020, the 8th Joint ACTRIMS – ECTRIMS Meeting

September 9, 2020

- Accepted abstracts include data assessing the long-term efficacy and safety of SOLIRIS® (eculizumab) in neuromyelitis optica spectrum disorder (NMOSD) -

- Data from 11 abstracts demonstrate Alexion's commitment to continuing to advance the understanding of the burden of NMOSD for patients and the efficacy and safety of SOLIRIS in real-world settings -

BOSTON--(BUSINESS WIRE)--Sep. 9, 2020-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today announced that 11 abstracts will be presented at MSVirtual2020, the 8th joint Americas Committee for Treatment and Research in Multiple Sclerosis and European Committee for Treatment and Research in Multiple Sclerosis (ACTRIMS-ECTRIMS) meeting, taking place virtually from September 11 to 13, 2020. The data being presented include two oral presentations of additional findings from the Phase 3 PREVENT study of SOLIRIS® (eculizumab) in anti-aquaporin-4 (AQP4) antibody positive neuromyelitis optica spectrum disorder (NMOSD) – one on long-term safety and efficacy data through 192 weeks of treatment with SOLIRIS as monotherapy, and another demonstrating the risk of adjudicated relapse was significantly lower with SOLIRIS than placebo in patients who had previously received rituximab. An additional analysis reinforces the safety of SOLIRIS as a treatment for patients with AQP4 antibody positive NMOSD and for adult patients with generalized Myasthenia Gravis (gMG) who are anti-acetylcholine receptor (AchR) antibody positive, based on data from the Phase 3 PREVENT and REGAIN studies and their extensions.

The totality of data being presented at MSVirtual2020 reinforces Alexion's commitment to conducting research that helps increase awareness and understanding of the burden of disease. Additionally, the data further strengthen the evidence supporting the substantial efficacy and durability of SOLIRIS in reducing relapse risk in patients with AQP4 antibody positive NMOSD and illustrate the benefits of SOLIRIS in decreasing healthcare resource utilization and use of concomitant immunosuppressive therapies (ISTs).

All accepted abstracts are listed below and are now available on the MSVirtual2020 website:

Long-term efficacy and safety of eculizumab monotherapy in AQP4+ neuromyelitis optica spectrum disorder.
Oral: FC01.01 – Sunday, September 13, 2020, 2:00 PM-2:12 PM ET; Free Communications 1

Efficacy and safety of eculizumab in patients with neuromyelitis optica spectrum disorder previously treated with rituximab: findings from PREVENT.
Oral: FC01.02 – Sunday, September 13, 2020, 2:12 PM-2:24 PM ET; Free Communications 1

Safety of eculizumab in NMOSD and MG – analysis of the phase 3 studies PREVENT and REGAIN and their extensions
Poster: P0752 – e-Poster presentation

Long-term efficacy and safety of eculizumab in AQP4+ neuromyelitis optica spectrum disorder
Poster: P0727– e-Poster presentation

Benefit of eculizumab for a broad range of patients with aquaporin-4 antibody-positive neuromyelitis optica spectrum disorder: findings from PREVENT
Poster: P0692 – e-Poster presentation

Burden of disease in patients with neuromyelitis optica spectrum disorder: insights from the CIRCLES study cohort
Poster: P0695 – e-Poster presentation

Relapse-associated visual impairment and disability in patients with neuromyelitis optica spectrum disorder
Poster: P0748 – e-Poster presentation

Patients with neuromyelitis spectrum disorder who experience relapses take more chronic pain medication
Poster: P0743 – e-Poster presentation

Cost of neuromyelitis optica spectrum disorder in US clinical practice.
Poster: P0706 – e-Poster presentation

Relapses and associated healthcare utilization among patients with neuromyelitis optica spectrum disorder in US clinical practice.
Poster: P0749 – e-Poster presentation

Impact of relapse on disability and quality of life in patients with neuromyelitis optica spectrum disorder: findings from the phase 3 PREVENT study.
Poster: P0718 – e-Poster presentation

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 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) injection for intravenous use, 300 mg/30 mL vial

INDICATION(S)
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, join 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 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), 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: Alexion’s commitment to continue to advance the understanding of the burden of NMOSD for patients and the efficacy and safety of SOLIRIS in real-world settings; the presented data will demonstrate the risk of adjudicated relapse was significantly lower with SOLIRIS than placebo in patients who had previously received rituximab; and that data being presented at MSVirtual2020 reinforces Alexion’s commitment to conducting research that helps increase awareness and understanding of the burden of disease; the data further strengthen the evidence supporting the substantial efficacy and durability of SOLIRIS in reducing relapse risk in patients with AQP4 antibody positive NMOSD and illustrate the benefits of SOLIRIS in decreasing healthcare resource utilization and use of concomitant ISTs. 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: the anticipated safety profile and the benefits of SOLIRIS for adult patients with AQP4 antibody-positive NMOSD may not be realized (and the results of the clinical trials may not be indicative of future results); results of clinical trials may not be sufficient to satisfy regulatory authorities; results in clinical trials may not be indicative of results from later stage or larger clinical trials (or in broader patient populations);; 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 discontinue sales of the product (or halt trials, delay or prevent us from making regulatory approval filings or result in denial of approval of our product candidates); 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; 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; risks related to the acquisitions of Portola Pharmaceuticals, Achillion and other companies and co-development efforts; 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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