



Alexion Announces Upcoming Data Presentations at 35th Congress of the European Committee for Treatment and Research in Multiple Sclerosis

August 28, 2019

- Five abstracts accepted, including new long-term data from PREVENT trial of SOLIRIS® (eculizumab) in adults with anti-AQP4 antibody-positive NMOSD -

BOSTON--(BUSINESS WIRE)--Aug. 28, 2019-- [Alexion Pharmaceuticals, Inc.](#) (NASDAQ:ALXN) today announced that five abstracts from the Phase 3 PREVENT study of SOLIRIS® (eculizumab) in neuromyelitis optica spectrum disorder (NMOSD) have been accepted for presentation at the 35th Congress of the European Committee for Treatment and Research in Multiple Sclerosis (ECTRIMS) in Stockholm, September 11 to 13, 2019. Key data will include new long-term safety and efficacy findings through 192 weeks of SOLIRIS treatment, as well as data on the impact of SOLIRIS on relapse-related hospitalizations, quality of life (QoL) and disability. Collectively, the breadth of the data to be presented at ECTRIMS support the robust treatment effect of SOLIRIS on relapse prevention demonstrated in the PREVENT study and help to further characterize the benefits of SOLIRIS for adult patients with anti-aquaporin-4 (AQP4) antibody-positive NMOSD.

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

[Impact of eculizumab on hospitalization rates and relapse treatment in patients with aquaporin-4 antibody-positive neuromyelitis optica spectrum disorder: findings from the phase 3 PREVENT study.](#) Abstract ID#: P604 – Poster Presentation 1, September 11, 2019, 17:15-19:15 CET, Poster Exhibition

[Subgroup analyses from the Phase 3 PREVENT study in patients with aquaporin-4 antibody-positive neuromyelitis optica spectrum disorder.](#) Abstract ID#: P605 – Poster Presentation 1, September 11, 2019, 17:15-19:15 CET, Poster Exhibition

[Impact of eculizumab on reported quality of life in patients with aquaporin-4 antibody-positive neuromyelitis optica spectrum disorder: findings from the PREVENT study.](#) Abstract ID#: P612 – Poster Presentation 1, September 11, 2019, 17:15-19:15 CET, Poster Exhibition

[Long-term safety and effectiveness of eculizumab in neuromyelitis optica spectrum disorder.](#) Abstract ID#: 142 – Free Communication, September 12, 2019, 09:06-09:18 CET, Hall A

[Impact of eculizumab on disability measures in patients with aquaporin-4 antibody-positive neuromyelitis optica spectrum disorder: phase 3 PREVENT study.](#) Abstract ID#: P1343 – Poster Presentation 3, September 13, 2019, 12:15-14:15 CET, Poster Exhibition

SOLIRIS is approved in the EU for the treatment of NMOSD in adult patients who are anti-AQP4 antibody positive with a relapsing course of the disease. In the U.S., SOLIRIS is approved for the treatment of adults with NMOSD who are anti-AQP4 antibody positive. A supplemental New Drug Application currently is under review by regulatory authorities in Japan. SOLIRIS received Orphan Drug Designation (ODD) for the treatment of NMOSD in the U.S., EU and Japan.

About NMOSD

NMOSD is a rare and severe, autoimmune, inflammatory disorder that attacks the central nervous system (CNS), in which complement activation due to anti-aquaporin-4 (AQP4) antibodies plays a significant role in the disease process. Patients with NMOSD experience unpredictable attacks, also referred to as relapses, which can cause irreversible damage predominantly to the optic nerve and spinal cord and can lead to long-term disability. The most common symptoms of NMOSD are optic neuritis and transverse myelitis. Optic neuritis can cause visual problems including blindness; transverse myelitis can cause mobility problems including paralysis.

The disease primarily affects women, often in the prime of their lives, with an average age of onset of 39 years. The prevalence of NMOSD may be more common and more severe in non-Caucasian populations worldwide.

Complement activation by anti-AQP4 antibodies can cause destruction of vital cells in the CNS, leading to demyelination and to the death of neurons, predominantly in the spinal cord and optic nerve. Approximately three quarters (73%) of all patients with NMOSD have AQP4 antibodies. In patients with anti-AQP4 antibody-positive NMOSD, the body's own immune system can turn against itself to produce antibodies against AQP4, a protein on certain cells in the optic nerves, brain and spinal cord that are critical for the survival of nerve cells. The binding of these anti-AQP4 antibodies activates the complement cascade, another part of the immune system.

About SOLIRIS

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 of 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 SOLIRIS® (eculizumab)

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 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) as well as the first and only approved complement inhibitor to treat atypical hemolytic uremic syndrome (aHUS), 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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For patient or advocacy inquiries please contact patientadvocacy@alexion.com.

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 data to be presented at ECTRIMS support the robust treatment effect of SOLIRIS on relapse prevention and help to further characterize the benefits of SOLIRIS for adult patients with anti-aquaporin-4 (AQP4) antibody-positive neuromyelitis optica spectrum disorder (NMOSD), the potential approval of SOLIRIS as a treatment for NMOSD in Japan; SOLIRIS can provide benefits for patients with NMOSD; and the anticipated timing of the review and decision of regulatory agencies with respect to the potential approval of SOLIRIS as a treatment for NMOSD. 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 benefits of SOLIRIS for NMOSD patients may not be realized (and the results of the clinical trials may not be indicative of the results once approved for use in the European Union); results of clinical trials may not be sufficient to satisfy the European Commission or any other regulatory authority in order to approve SOLIRIS as a treatment for NMOSD (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 once the product is approved for use by regulatory agencies); 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); 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 the European Commission and other regulatory agencies regarding 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 and inter partes review petitions submitted 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; uncertainties surrounding legal proceedings, company investigations and government investigations, including investigations of Alexion by the U.S. Securities and Exchange Commission (SEC) and U.S. Department of Justice; 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 acquisition of Syntimmune 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 quarter ended June 30, 2019 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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