Soliris® (eculizumab) Granted Marketing Authorization in Europe for Treatment of Patients with Atypical Hemolytic Uremic Syndrome (aHUS)

— First and Only Approved Treatment in both Europe and the United States for Patients with aHUS, an Ultra-Rare, Life-Threatening Disease —

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN) today announced that the European Commission (EC) has extended the therapeutic indication for Soliris® (eculizumab) to include the treatment of pediatric and adult patients with atypical hemolytic uremic syndrome (aHUS). Soliris is the first therapy approved in the European Union for the treatment of aHUS, an ultra-rare, life-threatening, chronic, genetic disease that progressively damages vital organs, leading to stroke, heart attack, kidney failure and death.[1]

The morbidity and premature mortality in aHUS are caused by chronic uncontrolled activation of the complement system, resulting in the formation of multiple blood clots in small blood vessels throughout the body, known as thrombotic microangiopathy or TMA.[2,3] Despite historical supportive care, more than half of all patients with aHUS die, require kidney dialysis or have permanent kidney damage within 1 year of diagnosis.[4]

"The approval of Soliris for aHUS in Europe is a major milestone for patients with aHUS, whose uncontrolled complement activation leads to progressive organ failure and a broad range of life-threatening outcomes," said Christophe Legendre, M.D., Professor of Nephrology at University Rene Descartes-Hôpital Necker in Paris.[5] "In clinical studies, chronic treatment with Soliris resulted in a rapid and sustained reduction in complement-mediated TMA. This drug alters the course of aHUS and can make a dramatic difference in patients' lives."

Soliris, a first-in-class terminal complement inhibitor, specifically targets uncontrolled complement activation. The EC has granted marketing authorization for Soliris to treat pediatric and adult patients with aHUS. The Clinical Particulars section of the EU label states, "Soliris treatment is recommended to continue for the patient's lifetime, unless the discontinuation of Soliris is clinically indicated," as described in the Special warnings and precautions for use subsection. Alexion will begin reimbursement discussions with healthcare authorities in major European countries, and expects to start serving patients with aHUS in initial major European countries in the first half of 2012, with additional major European countries commencing through mid-2013.

"The EC approval marks another milestone for Soliris and brings life-transforming hope another step closer to families in Europe living with this severe, devastating and life-threatening disease," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "We will work diligently with the healthcare authorities in individual countries to make Soliris available to children and adults with aHUS as quickly as possible."

Soliris was previously approved by the U.S. Food and Drug Administration (FDA) on September 23, 2011 for the treatment of patients with aHUS to inhibit complement-mediated TMA. Soliris is also approved in the United States, European Union, Japan and other territories for the treatment of patients with paroxysmal nocturnal hemoglobinuria (PNH), a debilitating, ultra-rare and life-threatening blood disorder.

Soliris in aHUS Clinical Data

The approval of Soliris for the treatment of aHUS is based on clinical data from two prospective, controlled, open-label studies in adolescent and adult patients with aHUS, and a third retrospective study in pediatric patients with aHUS, which together included a broad range of aHUS patients. All patients treated with Soliris when administered as recommended demonstrated rapid and sustained reduction in terminal complement activity, and chronic administration of Soliris resulted in rapid and sustained reduction in complement-mediated TMA.

In study C08-003 A/B, which included 20 patients with a long duration of aHUS and prior treatment before starting on Soliris,16 out of 20 Soliris-treated patients (80%) achieved TMA event-free status, defined as at least 12 consecutive weeks of stable platelet count, no plasma exchange/plasma infusion (PE/PI), and no new dialysis. Hematologic normalization was achieved in 18 of 20 Soliris-treated patients (90%). Renal function, as measured by eGFR, increased during Soliris therapy, and no patient required new dialysis.

In study C08-002 A/B, which included 17 patients with progressive clinical TMA complications despite intensive PE/PI, Soliris inhibited complement-mediated TMA as shown by a significant improvement in platelet count from baseline through week 26 of

73×10^9/L (p=0.0001). Hematologic normalization was observed in 13 of 17 Soliris-treated patients (76%), and TMA event-free status (stable platelet count, no PE/PI, and no new dialysis) was achieved by 15 of 17 Soliris-treated patients (88%). Renal function, as measured by eGFR, was significantly improved during Soliris therapy, and four out of five patients who required dialysis at study entry were able to discontinue dialysis for the duration of treatment. Patients also reported improved health-related quality of life.

Study C009-001r included 15 pediatric patients (ages 2 months to <12 years) who received Soliris outside of prospective clinical trials and with or without prior PE/PI. Platelet count was normalized in 14 of 15 Soliris-treated patients (93%). All patients treated with Soliris also achieved a reduction in the TMA intervention rate. The efficacy results for these pediatric patients appeared consistent with results for patients enrolled in the prospective aHUS studies. No pediatric patient required new dialysis during treatment with Soliris.

Soliris was well tolerated in these clinical studies. The most frequently reported adverse events were hypertension, upper respiratory tract infection, and diarrhea.

About aHUS

aHUS is a chronic, ultra-rare, and life-threatening disease in which a genetic deficiency in one or more complement regulatory genes causes life-long uncontrolled complement activation, resulting in complement-mediated thrombotic microangiopathy (TMA), the formation of blood clots in small blood vessels throughout the body.[1,2] Permanent, uncontrolled complement activation in aHUS causes a life-long risk for TMA, which leads to sudden, catastrophic, and life-threatening damage to the kidney, brain, heart, and other vital organs, and premature death. [2,3] More than half of all patients with aHUS die, require kidney dialysis or have permanent kidney damage within 1 year of diagnosis.[4] The majority of patients with aHUS who receive a kidney transplant commonly experience subsequent systemic TMA, resulting in a 90% transplant failure rate.[6]

aHUS affects both children and adults. In a large group of aHUS patients, 60% were first diagnosed at younger than 18 years of age.[6] Complement-mediated TMA also causes reduction in platelet count (thrombocytopenia) and red blood cell destruction (hemolysis). While mutations have been identified in at least ten different complement regulatory genes, mutations are not identified in 30-50% of patients with a confirmed diagnosis of aHUS.[7]

About Soliris

Soliris is a first-in-class terminal complement inhibitor developed from the laboratory through regulatory approval and commercialization by Alexion. Soliris is approved in the US, European Union, Japan and other countries as the first and only treatment for patients with paroxysmal nocturnal hemoglobinuria (PNH), a debilitating, ultra-rare and life-threatening blood disorder, characterized by complement-mediated hemolysis (destruction of red blood cells). Soliris is also approved in the US as the first and only treatment for patients with atypical Hemolytic Uremic Syndrome (aHUS) to inhibit complement-mediated thrombotic microangiopathy, a debilitating, ultra-rare and life-threatening genetic disorder characterized by complement-mediated thrombotic microangiopathy (blood clots in small vessels). The effectiveness of Soliris in aHUS is based on the effects on thrombotic microangiopathy (TMA) and renal function. Prospective clinical trials in additional patients are ongoing to confirm the benefit of Soliris in patients with aHUS. Soliris is not indicated for the treatment of patients with Shiga toxin E. coli related hemolytic uremic syndrome (STEC-HUS). Alexion's breakthrough approach in complement inhibition has received the pharmaceutical industry's highest honors: the 2008 Prix Galien USA Award for Best Biotechnology Product with broad implications for future biomedical research and the 2009 Prix Galien France Award in the category of Drugs for Rare Diseases. More information including the full prescribing information on Soliris is available at www.soliris.net.

Important Safety Information

Soliris is generally well tolerated in patients with PNH and aHUS. In patients with PNH, the most frequently reported adverse events observed with Soliris treatment in clinical studies were headache, nasopharyngitis (runny nose), back pain and nausea. Soliris treatment of patients with PNH should not alter anticoagulant management because the effect of withdrawal of anticoagulant therapy during Soliris treatment has not been established. In patients with aHUS, the most frequently reported adverse events observed with Soliris treatment in clinical studies were hypertension, upper respiratory tract infection, diarrhea, headache, anemia, vomiting, nausea, urinary tract infection, and leukopenia.

In Europe, the Summary of Product Characteristics (SmPC) for Soliris includes a special warning and precaution for use: "Due to its mechanism of action, the use of Soliris increases the patient's susceptibility to meningococcal infection (*Neisseria meningitidis*). These patients might be at risk of disease by uncommon serogroups (particularly Y, W135 and X), although meningococcal disease due to any serogroup may occur. To reduce the risk of infection, all patients must be vaccinated at least 2 weeks prior to receiving Soliris. Patients less than 2 years of age and those who are treated with Soliris less than 2 weeks after receiving a meningococcal vaccine must receive treatment with appropriate prophylactic antibiotics until 2 weeks after vaccination. Patients must be re-vaccinated according to current medical guidelines for vaccination use. Tetravalent vaccines

against serotypes A, C, Y and W135 are strongly recommended, preferably conjugated ones. Vaccination may not be sufficient to prevent meningococcal infection. Consideration should be given to official guidance on the appropriate use of antibacterial agents. Cases of serious or fatal meningococcal infections have been reported in Soliris treated patients. All patients should be monitored for early signs of meningococcal infection, evaluated immediately if infection is suspected, and treated with antibiotics if necessary. Patients should be informed of these signs and symptoms and steps taken to seek medical care immediately."

The U.S. product label for Soliris also includes a boxed warning: "Life-threatening and fatal meningococcal infections have occurred in patients treated with Soliris. Meningococcal infection may become rapidly life-threatening or fatal if not recognized and treated early. Comply with the most current Advisory Committee on Immunization Practices (ACIP) recommendations for meningococcal vaccination in patients with complement deficiencies. Immunize patients with a meningococcal vaccine at least 2 weeks prior to administering the first dose of Soliris, unless the risks of delaying Soliris therapy outweigh the risk of developing a meningococcal infection. (See Serious Meningococcal Infections (5.1) for additional guidance on the management of meningococcal infection.) Monitor patients for early signs of meningococcal infections and evaluate immediately if infection is suspected. Soliris is available only through a restricted program under a Risk Evaluation and Mitigation Strategy (REMS). Under the Soliris REMS, prescribers must enroll in the program (5.2). Enrollment in the Soliris REMS program and additional information are available by telephone: 1-888-soliris (1-888-765-4747)."

Prior to beginning Soliris therapy, all patients and their prescribing physicians in the United States will be enrolled in the Soliris Safety Registry which is part of a special risk management program that involves initial and continuing education and long-term monitoring for detection of new safety findings.

Please see full prescribing information for Soliris, including boxed WARNING regarding risk of serious meningococcal infection.

About Alexion

Alexion Pharmaceuticals, Inc. is a biopharmaceutical company focused on serving patients with severe and ultra-rare disorders through the innovation, development and commercialization of life-transforming therapeutic products. Alexion is the global leader in complement inhibition, and has developed and markets Soliris® (eculizumab) as a treatment for patients with PNH and aHUS, two debilitating, ultra-rare and life-threatening disorders caused by chronic uncontrolled complement activation. Soliris is currently approved in more than 35 countries for the treatment of PNH, and in the United States and the European Union for the treatment of aHUS. Alexion is evaluating other potential indications for Soliris and is pursuing development of other innovative biotechnology product candidates in early stages of development. This press release and further information about Alexion Pharmaceuticals, Inc. can be found at: www.alexionpharma.com.

Safe Harbor Statement

This news release contains forward-looking statements, including statements related to anticipated clinical development, regulatory and commercial milestones and potential health and medical benefits of Soliris® (eculizumab) for the potential treatment of patients with aHUS. Forward-looking statements are subject to factors that may cause Alexion's results and plans to differ from those expected, including for example, decisions of regulatory authorities regarding marketing approval or material limitations on the marketing of Soliris for its current or potential new indications, and a variety of other risks set forth from time to time in Alexion's filings with the Securities and Exchange Commission, including but not limited to the risks discussed in Alexion's Quarterly Report on Form 10-Q for the period ended September 30, 2011, and in Alexion's other filings with the Securities and Exchange Commission. Alexion does not intend to update any of these forward-looking statements to reflect events or circumstances after the date hereof, except when a duty arises under law.

[ALXN-G]

References and Footnotes

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5. Dr. Christophe Legendre receives research support from Alexion Pharmaceuticals, Inc. and is a consultant to the company.

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