# Alexion Receives CHMP Positive Opinion for Soliris® (eculizumab) in Patients with Atypical Hemolytic Uremic Syndrome (aHUS) in the European Union

***— CHMP recommends marketing authorization for Soliris to treat all patients with aHUS —***

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN) today announced that the European Committee for Medicinal Products for Human Use (CHMP) has adopted a positive opinion recommending that the therapeutic indication for Soliris® (eculizumab) be extended to include the treatment of pediatric and adult patients with atypical hemolytic uremic syndrome (aHUS) in Europe. Based on the CHMP's positive recommendation, a final decision from the European Commission is expected in approximately two months, after which the Company will then begin the country-by-country reimbursement processes.

There are no therapies approved for the treatment of aHUS in Europe. aHUS is an ultra-rare, life-threatening, genetic disease that progressively damages vital organs, leading to stroke, heart attack, kidney failure and death.[1] The morbidity and premature mortality in aHUS is caused by chronic uncontrolled activation of the complement system, resulting in the formation of blood clots in small blood vessels throughout the body, known as thrombotic microangiopathy or TMA.[2,3] Despite current 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 positive opinion adopted by the CHMP is a significant step towards making Soliris available to children and adults suffering with aHUS in Europe," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "Importantly, the adopted CHMP opinion recommends to extend marketing authorization for Soliris to treat all patients with aHUS. We look forward to efficiently working with authorities to make Soliris broadly available for European patients suffering with aHUS."

The CHMP based its opinion on clinical data from two prospective pivotal Phase 2 open-label clinical trials in adolescent and adult patients with aHUS, and a third retrospective study in children, adolescents, and adults with aHUS. A summary of the CHMP opinion can be accessed at http://www.emea.europa.eu.

Soliris is approved in the US (2007), European Union (2007), Japan (2010) and in other territories, for the treatment of patients with paroxysmal nocturnal hemoglobinuria (PNH), a debilitating, ultra-rare and life-threatening blood disorder. Alexion has also filed a supplemental Biologics License Application (sBLA) for Soliris as a treatment for patients with aHUS with the U.S. Food and Drug Administration (FDA).

**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] Patients with aHUS who receive a kidney transplant commonly experience subsequent systemic TMA, resulting in a 90% transplant failure rate.[5]

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.[6]

**About Soliris**

Soliris is a first-in-class terminal complement inhibitor developed from the laboratory through regulatory approval and commercialization by Alexion. Soliris has been approved in the U.S., European Union, Japan and other territories as the first treatment for patients with PNH, a debilitating, ultra-rare and life-threatening blood disorder defined by chronic uncontrolled complement activation which causes chronic red blood cell destruction (hemolysis), leading to blood clots, organ failure, and shortened survival. Prior to these approvals, there were no therapies specifically available for the treatment of patients with

PNH. Soliris (eculizumab) is not approved for the treatment of aHUS or any indication other than PNH. Alexion's breakthrough approach to complement inhibition has received some of 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 on Soliris is available at www.soliris.net

**Important Safety Information**

Soliris is generally well tolerated in patients with PNH. The most frequent adverse events observed in clinical studies of patients with PNH were headache, nasopharyngitis (runny nose), back pain and nausea. Treatment of patients with PNH with Soliris should not alter anticoagulant management because the effect of withdrawal of anticoagulant therapy during Soliris treatment has not been established.

In Europe, the product label for Soliris includes a special warning: "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 and 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 and Europe 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.

In the United States, please see full prescribing information for Soliris at www.soliris.net.

**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, a debilitating, ultra-rare and life-threatening blood disorder. Soliris is approved in more than 35 countries. 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 June 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.*

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**References**

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