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NICE Issues Final Positive Recommendation for National Commissioning of Soliris® (eculizumab) for All Patients with aHUS in England

- Final Evaluation Determination Confirms that Soliris is a "Very Effective Treatment for aHUS with Significant Value to Patients" -

- Final Evaluation Determination Includes the Same Conditions Discussed in Draft Recommendation, Including Establishment of an Expert Center and a Robust System to Monitor Patients Receiving Treatment -

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) announced today that the National Institute for Health and Care Excellence (NICE) Highly Specialised Technologies Evaluation Committee (EC) has recommended that Soliris® (eculizumab) be commissioned for all patients in England suffering from atypical hemolytic uremic syndrome (aHUS), a severe and life-threatening ultra-rare disorder. With this final evaluation determination, NICE has recommended Soliris for use within the National Health Service (NHS) in England as the first and only treatment for patients with aHUS.

"Today's decision is an important victory for patients with aHUS and physicians who now have assurance that they will have access to the life-transforming efficacy of Soliris. We are pleased that Soliris will be made available on the NHS for patients with aHUS and we commend NHS England for its previous decision to provide interim funding to patients for the extended period it took for NICE to make this final determination," said Leonard Bell, M.D., Chairman and Chief Executive Officer of Alexion.

In the final evaluation determination published today, the NICE EC again confirmed that Soliris represents an important treatment option of significant value to patients with aHUS. The Committee recommended the commissioning of Soliris, within its marketing authorization, for aHUS subject to the conditions provided in the evaluation consultation document released in September. Soliris was approved in November 2011 by the European Commission for the treatment of patients with aHUS. The Clinical Particulars section of the EU label states that "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.[1] As noted by NICE in its final evaluation determination, after stopping Soliris, severe TMA complications were reported including graft failure needing hemodialysis, renal insufficiency, end-stage renal failure and respiratory distress needing intubation.

"Patients with aHUS are at constant risk of sudden, progressive and life-threatening damage to vital organs including the kidney and other organs," said Keith Woods, Vice President and General Manager of Alexion Pharma UK. "Alexion supports the use of Soliris consistent with the EMA-approved label and firmly believes that decisions regarding continuation of Soliris should be made by the treating physician based on best clinical judgment."

NICE recommended that the following arrangements be in place as conditions for the funding of Soliris for patients with aHUS:

- Coordination of the use of eculizumab through an expert center;
- Monitoring systems to record the number of people with a diagnosis of atypical hemolytic uremic syndrome, the number of people who receive eculizumab, and the dose and duration of treatment for these people;
- A national protocol for starting and stopping eculizumab for clinical reasons;
- And a research programme with robust methods to evaluate when stopping treatment or dose adjustment might occur.

About aHUS

aHUS is a chronic, ultra-rare, and life-threatening disease in which a life-long and permanent genetic deficiency in one or more complement regulatory genes causes chronic uncontrolled complement activation, resulting in complement-mediated thrombotic microangiopathy (TMA), the formation of blood clots in small blood vessels throughout the body.[2,3] 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.[2,4] Seventy-nine percent of all patients with aHUS die, require kidney dialysis or have permanent kidney damage within three years after diagnosis despite plasma exchange or plasma infusion (PE/PI).[5] Moreover, 33 to 40 percent of patients die or progress to end-stage renal disease with the first clinical manifestation of aHUS despite PE/PI.[5,6] The majority of patients with aHUS who receive a kidney transplant

commonly experience subsequent systemic TMA, resulting in a 90 percent transplant failure rate in these TMA patients.[7]

aHUS affects both children and adults. 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 percent of patients with a confirmed diagnosis of aHUS.[5]

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 U.S. (2007), European Union (2007), Japan (2010) and other countries as the first and only treatment for patients with paroxysmal nocturnal hemoglobinuria (PNH) to reduce hemolysis. PNH is 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 U.S. (2011), the European Union (2011), Japan (2013) and other countries as the first and only treatment for patients with atypical hemolytic uremic syndrome (aHUS) to inhibit complement-mediated TMA. aHUS is a debilitating, ultra-rare and life-threatening genetic disorder characterized by complement-mediated thrombotic microangiopathy, or TMA (blood clots in small vessels). Soliris is not indicated for the treatment of patients with Shiga-toxin *E. coli*-related hemolytic uremic syndrome (STEC-HUS). For the breakthrough medical innovation in complement inhibition, Alexion and Soliris have received some of the pharmaceutical industry's highest honors: the Prix Galien USA (2008, Best Biotechnology Product) and France (2009, Rare Disease Treatment).

More information including the full U.S. prescribing information on Soliris is available at www.soliris.net.

Important Safety Information

The U.S. product label for Soliris 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 [see Warnings and Precautions (5.1)]. 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 two 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 Warnings and Precautions (5.1) for additional guidance on the management of the risk 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 [see Warnings and Precautions (5.2)]. Enrollment in the Soliris REMS program and additional information are available by telephone: 1-888-SOLIRIS (1-888-765-4747)."

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 headache, diarrhea, hypertension, upper respiratory infection, abdominal pain, vomiting, nasopharyngitis, anemia, cough, peripheral edema, nausea, urinary tract infections, and pyrexia. Soliris is not indicated for the treatment of patients with Shiga-toxin *E. coli*-related hemolytic uremic syndrome (STEC-HUS). Please see full prescribing information for Soliris, including BOXED WARNING regarding risk of serious meningococcal infection.

About Alexion

Alexion is a biopharmaceutical company focused on serving patients with severe and 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 nearly 50 countries for the treatment of PNH and in nearly 40 countries for the treatment of aHUS. Alexion is evaluating other potential indications for Soliris in additional severe and ultra-rare disorders beyond PNH and aHUS, and is developing other highly innovative biotechnology product candidates, including asfotase alfa, across multiple therapeutic areas. This press release and further information about Alexion can be found at: www.alexionpharma.com.

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Safe Harbor Statement

This news release contains forward-looking statements, including statements related to potential health and medical benefits of Soliris® (eculizumab) for the treatment of patients with aHUS and PNH, pricing for Soliris in England, and the continuation of

existing programs in England that provide access to Soliris. 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 reimbursement of Soliris, 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 Annual Report on Form 10-Q for the period ended September 30, 2014. 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.

References:

[1] Soliris eMPC. Available at http://www.medicines.org.uk/emc/medicine/19966. Last downloaded on 20 Oct 2014.

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[5] Noris M, Remuzzi G. Atypical hemolytic-uremic syndrome. *N Engl J Med.* 2009;361:1676-87.

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Media:
Irving Adler, 203-271-8210
Executive Director, Corporate Communications
or
Kim Diamond, 203-439-9600
Senior Director, Corporate Communications
or
Investors:
Elena Ridloff, CFA, 203-699-7722
Executive Director, Investor Relations

Source: Alexion Pharmaceuticals, Inc.