September 3, 2014

NICE Recommends Commissioning of Eculizumab (Soliris®) for All Patients with aHUS in England

- Draft Recommendation Confirms that Eculizumab is Very Effective and Only Treatment for aHUS -

- NICE Evaluation Committee Recommends Funding for Treatment of Patients with aHUS Subject to Conditions, Including Establishment of an Expert Center and a Robust System to Monitor Patients Receiving Treatment -

- Alexion Remains Committed to Working to Ensure Patients with aHUS in England Will Have Sustained Access to Eculizumab -

- Final Evaluation Decision Expected in Q4 2014 -

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) announced today that the National Institute for Health and Clinical Excellence (NICE) Highly Specialised Technologies Evaluation Committee (EC) has reaffirmed the very significant clinical value of eculizumab (Soliris®) for the treatment of atypical hemolytic uremic syndrome (aHUS) and the lack of other effective therapies, and has issued a recommendation that it be nationally commissioned for all patients suffering from this severe and life-threatening genetic disorder.

In the second Evaluation Consultation Document (ECD) released today, the NICE EC again confirmed the 2013 Advisory Group for National Specialised Services (AGNSS) positive assessment as well as the NICE assessment from earlier this year that eculizumab is a very effective treatment for aHUS patients and produces substantial quality-adjusted life year gains of a magnitude rarely seen for any new drug treatment. The Committee recommended the commissioning of eculizumab for aHUS patients subject to the following conditions:

- 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.

"We are pleased that NICE has once again confirmed that patients with aHUS are at constant risk of sudden, progressive and life-threatening damage to vital organs including the kidney and other organs, and that eculizumab is a significant breakthrough for patients with this devastating disorder," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "Alexion supports the use of eculizumab consistent with the EMA-approved label, which specifically directs that treatment is recommended to continue for the patient's lifetime, unless the discontinuation of eculizumab is clinically indicated. We believe that it is important that NICE work within its remit and that decisions regarding continuation of eculizumab should be made by the treating physician based on best clinical judgment. We will provide specific comments to NICE to address its conditions and look forward to working to ensure equity such that patients with aHUS in England, like patients with PNH in England, have equal and sustained access to this life-transforming therapy."

Currently, new and existing patients with aHUS in England are able to receive eculizumab through an interim policy commissioned by NHS England last year, and NICE confirmed today that this interim policy will remain in place pending the final outcome of NICE's appraisal. Alexion looks forward to confirmation by NICE of a final policy, which is expected to follow a public EC meeting scheduled on October 9, 2014.

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.[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.[1,3] Sixty-five percent of all patients with aHUS die, require kidney dialysis or have permanent kidney damage within the first year after diagnosis despite plasma exchange or

plasma infusion (PE/PI).[4,5] 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.[6]

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.[7]

About Soliris® (eculizumab)

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), a debilitating, ultra-rare and life-threatening blood disorder, characterized by complement-mediated hemolysis (destruction of red blood cells). Soliris is indicated to reduce hemolysis. 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), a debilitating, ultra-rare and life-threatening genetic disorder characterized by complement-mediated thrombotic microangiopathy, or TMA (blood clots in small vessels). Soliris is indicated to inhibit complement-mediated TMA. Soliris is not indicated for the treatment of patients with Shiga-toxin *E. coli*-related hemolytic uremic syndrome (STEC-HUS). For the breakthrough innovation in complement inhibition, Alexion and Soliris have 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 U.S. prescribing information on Soliris is available at: http://soliris.net/sites/default/files/assets/soliris_pi.pdf.

The full prescribing information on Soliris in Europe is available at: http://www.ema.europa.eu/docs/en_GB/document_library/EPAR_Product_Information/human/000791/WC500054208.pdf.

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, 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, whether eculizumab will be nationally commissioned for aHUS in England and the timing of such commissioning, 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 June 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:

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