# Alexion Announces Presentations at 2015 American Transplant Congress, Including Results from Studies Advancing the Understanding of Eculizumab (Soliris®) in Patients Undergoing Kidney Transplant

April 13, 2015

*--Presentations to Include Eculizumab Study in AMR, Burden of Disease AMR Study and Study Evaluating the Role of Complement in DGF --*

*--Study Underscoring the Benefit of Soliris in aHUS Patients With or Without History of Transplant also to be Presented--*

CHESHIRE, Conn.--(BUSINESS WIRE)--Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today announced that researchers will present results from four studies related to kidney transplant at the 2015 American Transplant Congress (ATC), being held May 2-6, 2015, in Philadelphia. The presentations will include data from two eculizumab (Soliris®) studies, one in the prevention of acute antibody-mediated rejection (AMR) in sensitized deceased-donor kidney transplant recipients, and another in adult patients with atypical hemolytic uremic syndrome (aHUS) with or without a history of kidney transplant. In addition, researchers will present an abstract describing the burden of AMR, and an abstract exploring the role of complement in delayed graft function (DGF). These abstracts have been published on the ATC website and can be accessed using the links below.

The following abstract will be presented in a late-breaking poster session on Saturday, May 2, 2015, from 5:30 p.m. to 7:30 p.m., Eastern Standard Time (EST):

- Abstract 3017: “Burden of Early Antibody-Mediated Rejection (AMR): Complications, Resource Utilization and Cost-Differential in Treatment of AMR,” Banga et al.

Accessible at: http://www.atcmeetingabstracts.com/abstract/burden-of-early-antibody-mediated-rejection-amr-complications-resource-utilization-and-cost-differential-in-treatment-of-amr/

The following abstract will be presented in an oral session on Monday, May 4, 2015, from 2:15 p.m. to 3:45 p.m., Eastern Standard Time (EST):

- Abstract 789: “Targeting Complement Pathways during Ischemia and Reperfusion: Implications for the Prevention of Delayed Graft Function,” Yu et al.

Accessible at: http://www.atcmeetingabstracts.com/abstract/targeting-complement-pathways-during-ischemia-and-reperfusion-implications-for-the-prevention-of-delayed-graft-function/

The following abstract will be presented in a late-breaking oral session on Tuesday, May 5, 2015, from 2:15 p.m. to 3:45 p.m., Eastern Standard Time (EST):

- Abstract 3039: “Eculizumab in Prevention of Acute Antibody-Mediated Rejection in Sensitized Deceased-Donor Kidney Transplant Recipients: 1-Year Outcomes,” Glotz et al.

Accessible at: http://www.atcmeetingabstracts.com/abstract/eculizumab-in-prevention-of-acute-antibody-mediated-rejection-in-sensitized-deceased-donor-kidney-transplant-recipients-1-year-outcomes/

The following abstract will be presented in an oral session on Tuesday, May 5, 2015, from 2:15 p.m. to 3:45 p.m., Eastern Standard Time (EST):

- Abstract 1243: “1-Year Safety and Efficacy of Eculizumab in Adult aHUS Patients, With or Without a History of Renal Transplant,” Legendre et al.

Accessible at: http://www.atcmeetingabstracts.com/abstract/1-year-safety-and-efficacy-of-eculizumab-in-adult-ahus-patients-with-or-without-a-history-of-renal-transplant/

Soliris® (eculizumab) is a first-in-class terminal complement inhibitor approved in nearly 50 countries as a treatment for patients with paroxysmal nocturnal hemoglobinuria (PNH) and in nearly 40 countries as a treatment for patients with aHUS. Both PNH and aHUS are life-threatening ultra-rare diseases caused by chronic uncontrolled complement activation. Soliris is not approved in any country for the prevention or treatment of AMR or DGF.

**About Acute Antibody-Mediated Rejection (AMR)**

Acute antibody-mediated rejection (AMR) is a severe and potentially life-threatening condition that can lead to severe allograft damage resulting in rapid loss of function and possible loss of the transplanted organ.[1] Patients who are sensitized (have high levels of donor-specific-antibodies [DSAs]) are at high risk for developing acute AMR.[1,2] The historical rate of acute AMR in high-risk living-donor kidney transplant recipients has been reported as high as 41%.[3] Acute AMR is believed to be primarily a result of uncontrolled complement activation caused by DSAs.[1,2] Currently, there are no approved therapies for the prevention or treatment of acute AMR.

**About Delayed Graft Function (DGF)**

DGF is an early and serious complication of organ transplantation that affects approximately 25 to 50 percent of deceased-donor kidney transplant cases and is characterized by the failure of a transplanted organ to function normally immediately following transplantation.[4,5] When DGF occurs in the setting of kidney transplantation, the patient requires dialysis after the transplant procedure.[6-8] Most often, DGF results from organ injury caused by severe inflammation and complement activation associated with the normal processes for removal, storage, and transplantation of the donor organ.[6-9] DGF has a substantial negative impact on graft function both in the short and long term, which can result in premature graft loss, prolonged hospitalization or patient death.[10,11] In addition, as donor organs are in short supply, reducing the risk of DGF for organs that are at higher risk to develop DGF may allow more donor organs to be transplanted. With specific regard to kidney transplantation, 15-20 percent of donor kidneys are reportedly never used and thus discarded each year in the U.S. and Europe due to the risk of poor outcomes associated with DGF[12,13], denying many patients the benefit of transplantation.

Currently, there are no approved therapies to prevent DGF after kidney transplantation.

**About Atypical Hemolytic Uremic Syndrome (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.[14,15] 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.[14,16] 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).[17] 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.[17,18] 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.[19]

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.[17]

**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 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, 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 paroxysmal nocturnal hemoglobinuria (PNH) and atypical hemolytic uremic syndrome (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.alexion.com.

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