October 2, 2015

Alexion Announces Presentations at ASN 2015, Including a Long-Term Follow-Up Study of Soliris® (eculizumab) for the Prevention of Thrombotic Microangiopathy (TMA) in Patients with Atypical Hemolytic Uremic Syndrome (aHUS)

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today announced that researchers will present data from a long-term follow-up study of the efficacy of Soliris® (eculizumab) in preventing thrombotic microangiopathy (TMA) events in patients with atypical hemolytic uremic syndrome (aHUS). Researchers will also present a post-hoc analysis of the safety of Soliris in pediatric patients with aHUS, as well as an update from the Global aHUS Registry. aHUS is a genetic, chronic, ultra-rare disease associated with vital organ failure and premature death. The data will be presented at the 2015 American Society of Nephrology (ASN) Annual Meeting being held November 3-8, 2015, in San Diego.

Soliris is approved in nearly 40 countries as a treatment for patients with aHUS and in nearly 50 countries as a 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). Both aHUS and PNH are caused by chronic uncontrolled complement activation.

Abstracts summarizing these presentations were published on the ASN website and can be accessed using the links below.

The following abstract will be presented in a poster session on Thursday, November 5, 2015, from 10:00 a.m. to 12:00 p.m., Pacific Standard Time (PST):

- Abstract TH-PO460: "Safety of Eculizumab in Pediatric Patients with Atypical Hemolytic Uremic Syndrome," Ariceta, et al.

  Accessible at: http://www.abstracts2view.com/asn_2015/view.php?nu=1003&type=abstract

The following abstracts will be presented in a poster session on Friday, November 6, 2015, from 10:00 a.m. to 12:00 p.m., Pacific Standard Time (PST):

- Abstract FR-PO446: "Eculizumab Prevents Thrombotic Microangiopathy in Atypical Hemolytic Uremic Syndrome Patients: Long-Term Follow-up," Menne, et al.

  Accessible at: http://www.abstracts2view.com/asn_2015/view.php?nu=416&type=abstract

- Abstract FR-PO483: "Characteristics of 681 Patients with Atypical Hemolytic Uremic Syndrome in the Global aHUS Registry," Licht, et al.

  Accessible at: http://www.abstracts2view.com/asn_2015/view.php?nu=1758&type=abstract

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] 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).[4] 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.[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.[4]

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) 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), 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 thrombotic microangiopathy, or TMA (blood clots in small vessels). aHUS is a debilitating, ultra-rare and life-threatening genetic disorder characterized by 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, 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 global biopharmaceutical company focused on developing and delivering life-transforming therapies for patients with devastating and rare disorders. Alexion developed and commercializes Soliris® (eculizumab), the first and only approved complement inhibitor to treat patients with paroxysmal nocturnal hemoglobinuria (PNH) and atypical hemolytic uremic syndrome (aHUS), two life-threatening ultra-rare disorders. Alexion is also establishing a premier global metabolic rare disease franchise, which includes Kanuma™ (sebelipase alfa) for patients with lysosomal acid lipase deficiency (LAD), and Strensiq™ (asfotas alfa) for patients with hypophosphatasia (HPP). In addition, Alexion is advancing the most robust rare disease pipeline in the biotech industry, with highly innovative product candidates in multiple therapeutic areas. As the global leader in complement inhibition, the Company is strengthening and broadening its portfolio of complement inhibitors across diverse platforms, including evaluating potential indications for Soliris in additional severe and ultra-rare disorders. This press release and further information about Alexion can be found at: www.alexion.com.

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References

1. Benz K, Amann K. Thrombotic microangiopathy: new insights. *Curr Opin Nephrol Hypertens.* 2010;19(3):242-7.

2. Ariceta G, Besbas N, Johnson S, et al. Guideline for the investigation and initial therapy of diarrhea-negative hemolytic uremic syndrome. *Pediatr Nephrol.* 2009;24:687-96.

3. Tsai HM. The molecular biology of thrombotic microangiopathy. *Kidney Int*. 2006;70(1):16-23.

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

5. Noris M, Caprioli J, Bresin E, et al. Relative role of genetic complement abnormalities in sporadic and familial aHUS and their impact on clinical phenotype. *Clin J Am Soc Nephrol.* 2010;5:1844-59.

6. Bresin E, Daina E, Noris M, et al. Outcome of renal transplantation in patients with non-Shiga toxin-associated hemolytic uremic syndrome: prognostic significance of genetic background. *Clin J Am Soc Nephrol.* 2006;1:88-99.

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