November 5, 2015

Researchers to Present Data on Enhancing the Understanding of PNH and aHUS and Underscoring the Effectiveness of Soliris® (eculizumab) Treatment at ASH 2015 Annual Meeting

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today announced that researchers will present data from the International Paroxysmal Nocturnal Hemoglobinuria (PNH) Registry that enhance the understanding of PNH and provide important information for the medical community on the long-term management of the disease, including the continued benefits of ongoing Soliris® (eculizumab) treatment regardless of transfusion history. Researchers will also present data from a long-term follow-up study of the effectiveness of Soliris in preventing thrombotic microangiopathy (TMA) events in patients with atypical hemolytic uremic syndrome (aHUS). These findings will be presented at the 57th Annual Meeting of the American Society of Hematology (ASH), which will be held December 5-8, 2015, in Orlando, Florida.

Soliris is approved in nearly 50 countries as a treatment for patients with PNH, a debilitating, ultra-rare and life-threatening blood disorder characterized by complement-mediated hemolysis (destruction of red blood cells), and in nearly 40 countries as a treatment for patients with aHUS, a genetic, chronic, ultra-rare disease associated with vital organ failure and premature death. Both PNH and aHUS are caused by chronic uncontrolled complement activation.

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

Soliris and PNH

The following abstracts will be presented in a poster session on Monday, December 7, 2015, from 6:00 p.m. to 8:00 p.m., Eastern Standard Time (EST):

- Abstract 3340: "Clinical Benefit of Eculizumab in Patients with No Transfusion History in the International Paroxysmal Nocturnal Hemoglobinuria Registry," Almeida, et al.

  Accessible at: https://ash.confex.com/ash/2015/webprogram/Paper81505.html

- Abstract 3339: "Patients with Paroxysmal Nocturnal Hemoglobinuria and Hemolysis Demonstrate More Frequent Disease-Related Features Than Those without Hemolysis, but Similar Proportions Experience Thromboembolism," Yeneral, et al.

  Accessible at: https://ash.confex.com/ash/2015/webprogram/Paper79977.html

- Abstract 3341: "Different Clinical Characteristics of Paroxysmal Nocturnal Hemoglobinuria in Pediatric and Adult Patients," Urbano-Ispizua, et al.

  Accessible at: https://ash.confex.com/ash/2015/webprogram/Paper79136.html

Soliris and aHUS

The following abstract will be presented in a poster session on Sunday, December 6, 2015, from 6:00 p.m. to 8:00 p.m., Eastern Standard Time (EST):

- Abstract 2252: "Eculizumab Prevents Thrombotic Microangiopathy: Long-term Follow-up Study of Patients with Atypical Hemolytic Uremic Syndrome," Greenbaum, et al.

  Accessible at: https://ash.confex.com/ash/2015/webprogram/Paper78892.html

About PNH

PNH is an ultra-rare blood disorder in which chronic, uncontrolled activation of complement, a component of the normal immune system, results in hemolysis (destruction of the patient's red blood cells). PNH strikes people of all ages, with an average age of onset in the early 30s.[1] Approximately 10 percent of all patients first develop symptoms at 21 years of age or younger.[2] PNH develops without warning and can occur in men and women of all races, backgrounds and ages. PNH often goes unrecognized, with delays in diagnosis ranging from one to more than 10 years.[3] In the period of time before Soliris was available, it had been

estimated that approximately one-third of patients with PNH did not survive more than five years from the time of diagnosis.[1] PNH has been identified more commonly among patients with disorders of the bone marrow, including aplastic anemia (AA) and myelodysplastic syndromes (MDS).[4,5,6] In patients with thrombosis of unknown origin, PNH may be an underlying cause.[1]

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.[7,8] 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.[7,9] 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).[10] 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.[10,11] 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 aHUS patients.[12]

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 clinical diagnosis of aHUS.[10]

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. As the global leader in complement inhibition, Alexion is strengthening and broadening its portfolio of complement inhibitors, including evaluating potential indications for eculizumab in additional severe and ultra-rare disorders. Alexion's metabolic franchise includes two highly innovative enzyme replacement therapies for patients with life-threatening and ultra-rare disorders, Strensiq™ (asfotase alfa) to treat patients with hypophosphatasia (HPP and Kanuma™ (sebelipase alfa) to treat patients with lysosomal acid lipase deficiency (LAD). In addition, Alexion is advancing the most robust rare disease pipeline in the biotech industry, with highly innovative product candidates in multiple therapeutic areas. This press release and further information about Alexion can be found at: www.alexion.com.

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