November 6, 2014

Researchers to Present Data on Improving the Understanding of PNH and aHUS and Underscoring the Effectiveness of Soliris® (eculizumab) Treatment at ASH 2014 Annual Meeting

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today announced that researchers will present data from clinical studies of Soliris® (eculizumab) in patients with paroxysmal nocturnal hemoglobinuria (PNH) and atypical hemolytic uremic syndrome (aHUS), two life-threatening and ultra-rare diseases caused by chronic uncontrolled complement activation, as well as data from the Global aHUS Registry, at the 56th Annual Meeting of the American Society of Hematology (ASH). Soliris is the first and only approved treatment for PNH and aHUS. Abstracts summarizing these presentations are published on the ASH website and can be accessed using the links below. The ASH annual meeting will be held December 6-9, 2014, in San Francisco.

Soliris, a first-in-class terminal complement inhibitor, 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 and ultra-rare disease associated with vital organ failure and premature death.

Soliris and PNH

The following abstract will be presented in a poster session on Saturday, December 6, 2014, from 5:30 to 7:30 p.m., Pacific Standard Time (PST):

Abstract 1595: "The Interim Analysis of the OPTIMA (Observation of GPI-anchored Protein-Deficient [PNH-type] Cells in Japanese Patients with Bone Marrow Failure Syndrome and in those Suspected of Having PNH) Study," Noji, et al.

Accessible at: https://ash.confex.com/ash/2014/webprogram/Paper74188.html

Soliris and aHUS

The following abstract will be presented in a poster session on Sunday, December 7, 2014, from 6:00 to 8:00 p.m., Pacific Standard Time (PST):

Abstract 2789: "Eculizumab is an Effective Treatment for Atypical Hemolytic Uremic Syndrome in Pediatric and Adult Patients with or without Identified Genetic Complement Mutations or Complement Factor H Autoantibodies," Cataland, et al.

Accessible at: https://ash.confex.com/ash/2014/webprogram/Paper73345.html

The following abstract will be presented in a poster session on Monday, December 8, 2014, from 6:00 to 8:00 p.m., Pacific Standard Time (PST):

Abstract 4204: "Baseline Demographics and Characteristics of 466 Patients with Atypical Hemolytic Uremic Syndrome in the Global aHUS Registry," Licht, et al.

Accessible at: https://ash.confex.com/ash/2014/webprogram/Paper69197.html

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.alexionpharma.com.

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