November 7, 2013

Researchers to Present Clinical Trial Data With Soliris® (eculizumab) Treatment in Both Approved Indications — PNH and aHUS — at ASH Annual Meeting

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (Nasdaq:ALXN) today announced that researchers will present data from clinical studies of Soliris® (eculizumab) as a treatment for 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, at the 55th Annual Meeting of the American Society of Hematology (ASH). Soliris is the first and only approved treatment for PNH and aHUS. Abstracts summarizing these data are published on the ASH website and can be accessed using the links below. The ASH annual meeting will be held December 7-10, 2013, at the Ernest N. Morial Convention Center in New Orleans.

Soliris was first approved in 2007 and is now 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). Soliris is also approved in the United States (2011), European Union (2011), Japan (2013) and other 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 7, 2013 from 5:30 — 7:30 p.m., Central Standard Time (CST):

Abstract 1241: "Baseline Assessment of GPI-anchored Protein Deficient Blood Cells in Patients with Bone Marrow Failure (The OPTIMA study)," Obara, et al.

Accessible at: https://ash.confex.com/ash/2013/webprogram/Paper63472.html

The following abstracts will be presented in a poster session on Monday, December 9, 2013 from 6:00 — 8:00 p.m., Central Standard Time (CST):

Abstract 3715: "Periodic Evaluation of the Clone Size is Mandatory in PNH: A Study of the Spanish Cohort of the International PNH Registry," Villegas, et al.

Accessible at: https://ash.confex.com/ash/2013/webprogram/Paper60116.html

Abstract 3720: "Clinical Signs and Symptoms in Non-transfused Patients with Paroxysmal Nocturnal Hemoglobinuria from a Korean Prospective PNH Registry," Lee, et al.

Accessible at: https://ash.confex.com/ash/2013/webprogram/Paper61567.html

Soliris and aHUS

The following abstracts will be presented in a poster session on Sunday, December 8, 2013 from 6:30 - 8:30 p.m., Central Standard Time (CST):

Abstract 2184: "Biomarkers of Complement and Endothelial Activation, Inflammation, Thrombosis, and Renal Injury in Patients with Atypical Hemolytic Uremic Syndrome (aHUS) Treated with Eculizumab," Cofiell, et al.

Accessible at: https://ash.confex.com/ash/2013/webprogram/Paper63810.html

Abstract 2191: "Eculizumab Inhibits Thrombotic Microangiopathy (TMA) and Improves Renal Function in Pediatric Patients with Atypical Hemolytic Uremic Syndrome (aHUS)," Greenbaum, et al.

Accessible at: https://ash.confex.com/ash/2013/webprogram/Paper61643.html

Abstract 2179: "Eculizumab Inhibits Thrombotic Microangiopathy (TMA) and Improves Renal Function in Adult Patients with Atypical Hemolytic Uremic Syndrome (aHUS)," Fakhouri, et al.

Accessible at: https://ash.confex.com/ash/2013/webprogram/Paper61803.html

Abstract 2186: "Time to Hematologic and Renal Improvements in Atypical Hemolytic Uremic Syndrome Patients with Long Disease Duration and Chronic Kidney Disease (CKD) Treated with Eculizumab," Licht, et al.

Accessible at: https://ash.confex.com/ash/2013/webprogram/Paper62059.html

The following abstract will be presented in a poster session on Sunday, December 9, 2013 from 6:00 - 8:00 p.m., Central Standard Time (CST):

Abstract 3426: "Time to Hematologic and Renal Improvements in aHUS Patients with Progressing Thrombotic Microangiopathy Treated with Eculizumab Over Two Years," Muus, et al.

Accessible at: https://ash.confex.com/ash/2013/webprogram/Paper60646.html

About Soliris

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 US (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 also approved in the US (2011), the 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, a debilitating, ultra-rare and life-threatening genetic disorder characterized by complement-mediated thrombotic microangiopathy (blood clots in small vessels). The effectiveness of Soliris in aHUS is based on the effects on thrombotic microangiopathy (TMA) and renal function. Prospective clinical trials in additional patients are ongoing to confirm the benefit of Soliris in patients with aHUS. Soliris is not indicated for the treatment of patients with Shiga-toxin *E. coli* related hemolytic uremic syndrome (STEC-HUS).

Alexion's breakthrough approach in complement inhibition has 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.

Important Safety Information

The US 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. 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 2 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 Serious Meningococcal Infections (5.1) for additional guidance on the management 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. 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 hypertension, upper respiratory tract infection, diarrhea, headache, anemia, vomiting, nausea, urinary tract infection, and leukopenia. Please see full prescribing information for Soliris, including boxed WARNING regarding risk of serious meningococcal infection.

About Alexion

Alexion Pharmaceuticals, Inc. is a biopharmaceutical company focused on serving patients with severe and ultra-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 the United States, Europe, Japan and other countries for the treatment of aHUS. Alexion is evaluating other potential indications for Soliris and is pursuing development of four other innovative biotechnology product candidates which are being investigated across additional severe and ultra-rare disorders beyond PNH and aHUS. This press release and further information about Alexion Pharmaceuticals, Inc. can be found at www.alexionpharma.com.

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