November 7, 2012

Researchers to Present Additional Data on Soliris® (eculizumab) as a Treatment for Patients with 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 debilitating, ultra-rare and life-threatening disorders, at the 54th Annual Meeting of the American Society of Hematology (ASH). 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 8-11, 2012, at the Georgia World Congress Center in Atlanta.

Soliris is approved in the US, European Union, Japan 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 United States and European Union as the first and only treatment for patients with atypical hemolytic uremic syndrome (aHUS), a debilitating, ultra-rare, life-threatening and chronic genetic disorder characterized by complement-mediated thrombotic microangiopathy (blood clots in small vessels).

Soliris and PNH

The following abstracts will be presented in a poster session on Saturday, December 8, 2012 from 5:30 -7:30 p.m., Eastern Standard Time (EST):

Abstract 1260: "Long-Term Safety of Sustained Eculizumab Treatment in Patients with Paroxysmal Nocturnal Hemoglobinuria," Szer, et al.

Accessible at: https://ash.confex.com/ash/2012/webprogram/Paper49026.html

Abstract 1271: "Distribution of PNH Clone Sizes within High Risk Diagnostic Categories Among 481 PNH Positive Patients Identified by High Sensitivity Flow Cytometry," Movalia and Illingworth.

Accessible at: https://ash.confex.com/ash/2012/webprogram/Paper54330.html

Abstract 1273: "Risk of Thromboembolism in Patients with Paroxysmal Nocturnal Hemoglobinuria Presenting with Both Clinical Symptoms and Elevated Hemolysis," Lee, et al.

Accessible at: https://ash.confex.com/ash/2012/webprogram/Paper51478.html

The following abstracts will be presented in a poster session on Monday, December 10, 2012 from 6:00 — 8:00 p.m., EST:

Abstract 3197: "A Rare Genetic Polymorphism in C5 Confers Poor Response to the Anti-C5 Monoclonal Antibody Eculizumab by Nine Japanese Patients with PNH," Nishimura, et al.

Accessible at: https://ash.confex.com/ash/2012/webprogram/Paper52847.html

Abstract 3472: "Eculizumab in Paroxysmal Nocturnal Hemoglobinuria (PNH): A Report of All 153 Patients Treated in the UK," Hill, et al.

Accessible at: https://ash.confex.com/ash/2012/webprogram/Paper48352.html

Abstract 3480: "Eculizumab Protects Against TE and Prolongs Survival in Patients with Paroxysmal Nocturnal Hemoglobinuria: An International PNH Registry Study," Socié, et al.

Accessible at: https://ash.confex.com/ash/2012/webprogram/Paper51570.html

Soliris and aHUS

The following abstract will be presented in a poster session on Saturday, December 8, 2012 from 5:30 - 7:30 p.m., Eastern Standard Time (EST):

Abstract 985: "Eculizumab (ECU) Safety and Efficacy in Atypical Hemolytic Uremic Syndrome (aHUS) Patients with Long Disease Duration and Chronic Kidney Disease (CKD): 2-Year Results," Licht, et al.

Accessible at: https://ash.confex.com/ash/2012/webprogram/Paper48647.html

The following abstracts will be presented in a poster session on Sunday, December 9, 2012 from 6:00 -8:00 p.m., EST:

Abstract 2084: "Eculizumab (ECU) in Atypical Hemolytic Uremic Syndrome (aHUS) Patients with Progressing Thrombotic Microangiopathy (TMA): 2-Year Data," Greenbaum, et al.

Accessible at: https://ash.confex.com/ash/2012/webprogram/Paper48657.html

Abstract 2085: "Eculizumab Is an Effective Treatment for Atypical Hemolytic Uremic Syndrome in Patients with or without Identified Genetic Complement Mutations or Complement Factor H Auto-Antibodies," Al-Akash, et al.

Accessible at: https://ash.confex.com/ash/2012/webprogram/Paper48870.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, European Union, Japan 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 US and the European Union 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. The effectiveness of Soliris in aHUS is based on the effects on 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. More information, including the full 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. 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 (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 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 more than 40 countries for the treatment of PNH, and in the United States and the European Union for the treatment of aHUS. Alexion is evaluating other potential indications for Soliris and is developing four other highly innovative biotechnology product candidates, which are being investigated across eight 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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