October 16, 2013

Researchers to Present New Data on Soliris® (eculizumab) as a Treatment for Patients with aHUS at ASN Annual Meeting

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN) today announced that researchers are scheduled to present data from clinical studies of Soliris® (eculizumab) in patients with atypical hemolytic uremic syndrome (aHUS), a genetic, life-long, ultra-rare disease associated with vital organ failure and premature death. Data will be presented at the annual meeting of the American Society of Nephrology (ASN), being held from November 5 —10, 2013 in Atlanta, Georgia. Presentations will include:

- Results from a prospective clinical trial of Soliris in pediatric patients with aHUS, following the published results of a retrospective study in pediatric patients with aHUS
- Results from an expanded adult population, representing the largest prospective trial of Soliris in adult patients with aHUS
- Three-year follow-up data from two pivotal Phase 2 extension studies that highlight the long-term benefits of Soliris therapy in patients with aHUS

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

Soliris is approved in the United States, European Union, Japan and other countries as a treatment for patients with aHUS. Soliris is also approved 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 life-threatening, ultra-rare diseases that are caused by chronic uncontrolled complement activation.

Soliris and aHUS

The following abstract will be presented in an oral session on Friday, November 8, 2013 from 4:30 — 6:30 p.m. Eastern Standard Time (EST):

Abstract [FR-OR057]: "Eculizumab Inhibits Thrombotic Microangiopathy (TMA) and Improves Renal Function in Adult Atypical Hemolytic Uremic Syndrome (aHUS) Patients," Fakhouri et al.

Accessible at: http://www.abstracts2view.com/asn_2013/view.php?nu=5593&terms=&type=abstract

The following abstracts will be presented in a poster session on Saturday, November 9, 2013 from 10:00 a.m. — 12:00 p.m., Eastern Standard Time (EST):

Abstract [SA-PO852]: "Eculizumab Maintains Efficacy in Atypical Hemolytic Uremic Syndrome (aHUS) Patients with Progressing Thrombotic Microangiopathy (TMA): 3-Year Update," Gaber, et al.

Accessible at: http://www.abstracts2view.com/asn_2013/view.php?nu=5311&terms=&type=abstract

Abstract [SA-PO850]: "Eculizumab in Atypical Hemolytic Uremic Syndrome (aHUS) Patients with Long Disease Duration and Chronic Kidney Disease (CKD): Sustained Efficacy at 3 Years," Delmas, et al.

Accessible at: http://www.abstracts2view.com/asn_2013/view.php?nu=5506&terms=&type=abstract

Abstract [SA-PO849]: "Eculizumab Inhibits Thrombotic Microangiopathy (TMA) and Improves Renal Function in Pediatric Atypical Hemolytic Uremic Syndrome (aHUS) Patients," Greenbaum, et al.

Accessible at: http://www.abstracts2view.com/asn_2013/view.php?nu=5579&terms=&type=abstract

Abstract [SA-PO853]: "An Observational, Non-Interventional, Multicenter, Multinational Registry of Patients with Atypical

Hemolytic Uremic Syndrome (aHUS): Initial Patient Characteristics," Licht, et al.

Accessible at: http://www.abstracts2view.com/asn_2013/view.php?nu=5184&terms=&type=abstract

About Soliris

Soliris is a first-in-class terminal complement inhibitor developed from the laboratory through regulatory approval and commercialization by Alexion. Soliris is also 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 US, the European Union, Japan 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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