

# 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 are scheduled to present data on Soliris<sup>®</sup> (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, at the 53rd Annual Meeting of the American Society of Hematology (ASH). Abstracts summarizing these presentations were published today on the ASH web site and can be accessed using the links below. The ASH annual meeting will be held December 10-13, 2011, at the San Diego Convention Center in San Diego.

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 US 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 abstract will be presented in a poster session on Saturday, December 10, 2011 from 5:30 — 7:30 p.m., Pacific Standard Time (PST):

"Incidence of PNH Clones by Diagnostic Code Utilizing High Sensitivity Flow Cytometry," Movalia, et al.

Abstract 1033: http://ash.confex.com/ash/2011/webprogram/Paper44143.html

The following abstract will be presented in a poster session on Saturday, December 10, 2011 from 5:30 — 7:30 p.m. PST:

"Efficacy and Safety of Eculizumab in Children and Adolescents with Paroxysmal Nocturnal Hemoglobinuria," Reiss, et al.

Abstract 1034: http://ash.confex.com/ash/2011/webprogram/Paper44198.html

The following abstract will be presented in a poster presentation on Sunday, December 11, 2011 from 6:00 — 8:00 p.m. PST:

"Clinical Characteristics of Classic Paroxysmal Nocturnal Hemoglobinuria (PNH) in Pediatric Patients: A Comparison with Classic PNH in Adults. An International PNH Registry Study," Urbano-Ispizua, et al.

Abstract 2102: http://ash.confex.com/ash/2011/webprogram/Paper43502.html

The following abstract will be presented in a poster session on Monday, December 12, 2011 from 6:00 - 8:00 p.m. PST:

"Uncontrolled Complement Activation and the Resulting Chronic Hemolysis As Measured by LDH Serum Level At Diagnosis As Predictor of Thrombotic Complications and Mortality in a Large Cohort of Patients with Paroxysmal Nocturnal Hemoglobinuria (PNH)," Lee, et al.

Abstract 3166: http://ash.confex.com/ash/2011/webprogram/Paper44782.html

#### Soliris and aHUS

The following abstract will be presented in an oral session on Monday, December 12, 2011, at 7:00 a.m. PST:

"Eculizumab Is An Effective Long-Term Treatment In Patients with Atypical Hemolytic Uremic Syndrome (aHUS) Resistant to Plasma Exchange/Infusion (PE/PI): Results of An Extension Study," Greenbaum, et al.

Abstract 193: <u>http://ash.confex.com/ash/2011/webprogram/Paper44113.html</u>

The following abstract will be presented in a poster session on Monday, December 12, 2011, from 6:00 to 8:00 p.m. PST:

"Eculizumab Is An Effective Long-Term Treatment In Patients with Atypical Hemolytic Uremic Syndrome (aHUS) Previously Receiving Chronic Plasma Exchange/Infusion (PE/PI): Extension Study Results," Licht, et al.

Abstract 3303: http://ash.confex.com/ash/2011/webprogram/Paper44479.html

### Additional Abstracts to be Published in the ASH Abstract Book

"Eculizumab Treatment of Paroxysmal Nocturnal Hemoglobinuria Relapsing After Bone Marrow Transplant and Subsequent Clonal: Case Report," Brodsky, et al.

"Long-Term Outcome in a Pediatric Patient with Atypical Hemolytic Uremic Syndrome (aHUS) with Sustained Eculizumab (ECU) Treatment," Gruppo, et al.

"Use of Eculizumab in the treatment of idiopathic TTP," Laurence, et al.

"Pain is associated with increased TE and Mortality," Lee, et al.

"Eculizumab (ECU) Significantly Improves Health-Related Quality of Life (HRQoL in Patients with Atypical Hemolytic Uremic Syndrome (aHUS)," Muus, et al.

## 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 also approved in the US 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. More information including the full prescribing information on Soliris is available at <u>www.soliris.net</u>.

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#### **Important Safety Information**

Soliris is generally well tolerated in patients with PNH and aHUS. 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.

The U.S. product label for Soliris also 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)."

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<sup>®</sup> (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 35 countries for the treatment of PNH, and in the United States for the treatment of aHUS. Alexion is evaluating other potential indications for Soliris and is pursuing development of other innovative biotechnology product candidates in early stages of development. This press release and further information about Alexion Pharmaceuticals, Inc. can be found at: <a href="http://www.alexionpharma.com">www.alexionpharma.com</a>.

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