



Alexion Initiates Clinical Trial of Eculizumab as a Potential Treatment for Patients with STEC-HUS in Expanded Response to EHEC Crisis in Germany

Study Aims to Include All Patients with STEC-HUS Who Are Receiving Eculizumab at the Request of Their Physicians and Treatment Centers

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN) and Alexion Pharma International Sàrl (APIS), announced today that the Paul-Ehrlich-Institut (PEI), Germany's healthcare regulatory body for biological products, has authorized initiation of an open-label clinical trial to investigate eculizumab (Soliris®) as a treatment for patients with Shiga-toxin producing E. coli hemolytic uremic syndrome (STEC-HUS), which has resulted from infections by Enterohemorrhagic Escherichia coli (EHEC) in an unusually wide outbreak in Germany that began in May.

EHEC infections are very uncommon, and the STEC-HUS complication of EHEC, which affects only a subset of these patients, is ultra-rare. STEC-HUS, a devastating and life-threatening disease, is caused by uncontrolled complement activation.

As announced on May 30, 2011, in response to requests of German physicians and hospitals who are treating patients with STEC-HUS, Alexion has initiated an eculizumab access program and has been providing eculizumab free of charge throughout the crisis. As the number of cases of STEC-HUS has increased to unprecedented levels, PEI, leading physicians, and Alexion have agreed that implementation of a clinical trial is the best environment to ensure that the investigational therapy is provided to patients in a controlled manner to support safety and potential efficacy in this severe clinical setting. The study aims to include all patients who are receiving eculizumab in the current STEC-HUS outbreak.

"We have an unprecedented number of patients with STEC-HUS who are severely ill with a broad range of clinical manifestations. As we continue to react quickly to this public health crisis, we believe that the best way to evaluate the safety and efficacy of eculizumab in treating STEC-HUS patients is through a controlled study," said Rolf Stahl, M.D., Chairman, Department of Nephrology, University Hospital Hamburg-Eppendorf UKE and lead investigator of the clinical trial. "The purpose of this study is to provide a framework for a coordinated and orderly collection of data to both educate physicians in the current crisis and to learn more about the potential of eculizumab to treat patients with STEC-HUS in the future."

According to the Robert Koch Institute, more than 3,400 people in Germany have been affected by the EHEC outbreak, with over 35 deaths. Approximately 800 confirmed cases of STEC-HUS have been reported in what is likely one of history's worst outbreaks of the disease. (1)

"From the start of the current crisis Alexion moved quickly to respond to physician requests for immediate access to eculizumab. We are now increasing our commitment by working with leading German physicians to implement this clinical trial under the authorization of the Paul-Ehrlich-Institut," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "Alexion is eager to work with the medical community in Germany during this crisis, and to share our two decades of experience in researching and treating complement-based diseases. Our chief objective is to work with German physicians and PEI to learn more about the potential role of terminal complement inhibition with eculizumab as a treatment option for the subset of patients who develop STEC-HUS. Our experience with ultra-rare disorders is especially relevant here considering the extremely small numbers of patients who will ever develop STEC-HUS on a global basis."

About STEC-HUS

STEC-HUS is an ultra-rare and life-threatening disease due to uncontrolled complement activation which causes platelet activation, thrombosis (blood clots), hemolysis (red blood cell destruction), and inflammation in small blood vessels throughout the body, a process known as systemic thrombotic microangiopathy, or systemic TMA. Due to systemic TMA, STEC-HUS patients are at risk of progressive damage to multiple vital organs including the brain, heart, lungs, kidneys and organs of the gastrointestinal system. This severe organ damage also causes significant and early mortality in affected patients.

Separately, and as part of Alexion's expanding research and development initiatives, eculizumab is under regulatory review by the FDA and EMA as a treatment for patients with atypical hemolytic uremic syndrome (aHUS). Although similar in its life-threatening TMA clinical manifestations, aHUS and STEC-HUS are different diseases: aHUS is a life-long genetic disease with uncontrolled complement activation while STEC-HUS is not genetic and uncontrolled complement activation follows an isolated episode of infection.

Eculizumab is approved in the US, European Union, Japan and other countries for the treatment of patients with paroxysmal nocturnal hemoglobinuria (PNH), an ultra-rare, life-threatening blood disorder caused by chronic uncontrolled complement

activation, and is not approved in any country as a treatment for any other condition. There have been no controlled studies of eculizumab for STEC-HUS, though recent case studies published in the *New England Journal of Medicine* (2) describe encouraging results from the drug's use in several patients with STEC-HUS.

About Eculizumab (Soliris®)

Eculizumab is a first-in-class terminal complement inhibitor developed from the laboratory through regulatory approval and commercialization by Alexion. Under the trade name Soliris®, eculizumab has been approved in the US, European Union, Japan and other territories as the first treatment for patients with PNH, an ultra-rare, debilitating and life-threatening blood disorder defined by chronic uncontrolled complement activation which causes chronic red blood cell destruction (hemolysis), leading to blood clots, organ failure, and shortened survival. Prior to these approvals, there were no therapies specifically available for the treatment of patients with PNH. Eculizumab is not approved for the treatment of STEC-HUS or other indications other than PNH. Alexion's breakthrough approach to complement inhibition has received some of 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 on Soliris is available at www.soliris.net.

Important Safety Information

Soliris is generally well tolerated in patients with PNH. The most frequent adverse events observed in clinical studies of patients with PNH were headache, nasopharyngitis (runny nose), back pain and nausea. Treatment with Soliris should not alter anticoagulant management because the effect of withdrawal of anticoagulant therapy during Soliris treatment has not been established.

The U.S. product label for Soliris also includes a boxed warning: "Soliris increases the risk of meningococcal infections. Meningococcal infection may become rapidly life-threatening or fatal if not recognized and treated early. Vaccinate patients with a meningococcal vaccine at least two weeks prior to receiving the first dose of Soliris; revaccinate according to current medical guidelines for vaccine use. Monitor patients for early signs of meningococcal infections, evaluate immediately if infection is suspected, and treat with antibiotics if necessary." During PNH clinical studies, two out of 196 vaccinated PNH patients treated with Soliris experienced a serious meningococcal infection. Prior to beginning Soliris therapy, all patients and their prescribing physicians are encouraged to enroll in the PNH Registry, which is part of a special risk-management program that involves initial and continuing education and long-term monitoring for detection of new safety findings.

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. Further information about Alexion Pharmaceuticals, Inc. can be found at: www.alexionpharma.com.

Safe Harbor Statement

This news release contains forward-looking statements, including statements related to anticipated clinical development and potential health and medical benefits of eculizumab (Soliris®) for the potential treatment of patients with STEC-HUS. Forward-looking statements are subject to factors that may cause Alexion's results and plans to differ from those expected, including for example, decisions of regulatory authorities regarding marketing approval or material limitations on the marketing of Soliris for its current or potential new indications, and a variety of other risks set forth from time to time in Alexion's filings with the Securities and Exchange Commission, including but not limited to the risks discussed in Alexion's Quarterly Report on Form 10-Q for the period ended March 31, 2011, and in Alexion's other filings with the Securities and Exchange Commission. Alexion does not intend to update any of these forward-looking statements to reflect events or circumstances after the date hereof, except when a duty arises under law.

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References

1. Robert Koch Institut. Available at http://www.rki.de/EN/Home/homepage_node.html. Accessed June 19, 2011.
2. Lapeyraque A-L, Malina M, Fremeaux-Bacchi V, et al. Complement blockade in severe Shigatoxin—associated HUS. *N Engl J Med* 2011. DOI: 10.1056/NEJMc1100859.

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