Alexion Announces Interim Results from Phase 2 Trials of Eculizumab (Soliris(R)) in Patients with atypical Hemolytic Uremic Syndrome (aHUS)

Data Accepted for Presentation at American Society of Nephrology Annual Meeting in November

CHESHIRE, Conn., Oct 20, 2010 (BUSINESS WIRE) -- Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN) today announced that its two open-label Phase 2 studies investigating eculizumab (Soliris(R)) as a treatment for patients with atypical Hemolytic Uremic Syndrome (aHUS) have met the primary and key secondary endpoints with high clinical and statistical significance, in interim analyses. aHUS is an ultra-rare, chronic and life-threatening disease in which uncontrolled complement activation causes blood clots in small blood vessels (thrombotic microangiopathy, or TMA) throughout the body leading to stroke, heart attack, kidney failure and death.[1,2] Approximately 60 percent of patients with aHUS require dialysis, a kidney transplant or die within a year of diagnosis.[2] Abstracts summarizing these interim data have been posted on the web site of the American Society of Nephrology (ASN) at http://www.abstracts2view.com/asn. These two trials are currently ongoing and data will be presented at the ASN annual meeting held November 18 - 21 in Denver, Colorado.

Patients Resistant to Plasma Therapy

Abstract 1338, "Safety and Efficacy of Eculizumab in aHUS Patients Resistant to Plasma Therapy: Interim Analysis from a Phase 2 Trial," summarized an interim analysis of 17 adolescent and adult patients with aHUS who were resistant to plasma therapy and were treated with eculizumab for up to 26 weeks.[3] The primary endpoint of the study is the change in platelet count, a measure of TMA. In this interim analysis, researchers observed a significant 80 ± 64 x 10^3/uL ($p<0.0001$) increase in platelet count with eculizumab treatment compared to baseline. Key secondary clinical endpoints were also positive. Updated data from this study will be presented at the ASN annual meeting on Saturday, November 20 at 5:30 p.m. Mountain Standard Time (MST).

Patients on Chronic Plasma Therapy

Abstract #157, "Safety and Efficacy of Eculizumab in aHUS Patients on Chronic Plasma Therapy: Interim Analysis of a Phase 2 Trial," summarized interim results from a study of 20 adolescent and adult patients with aHUS who were receiving plasma therapy chronically prior to entering the study.[4] The primary endpoint of the study is TMA Event-Free Status, as defined by stable platelet counts, absence of plasma therapy and no new dialysis. In this interim analysis of 15 patients treated with eculizumab for at least 12 weeks, a significant 87% (95% CI 60-98) of patients achieved TMA Event-Free Status. Key secondary clinical endpoints were also positive. Interim data from this study will be presented in a poster session at the ASN annual meeting on Friday, November 19 at 10:00 a.m. MST.

In both studies, interim results were reported at the last captured timepoint. Eculizumab appeared to be well-tolerated in the studies, with the most common adverse events including anemia, diarrhea, headache, nausea and hypertension.

About the Studies

These two open-label Phase 2 clinical studies investigate eculizumab for the treatment of patients with aHUS who (i) were resistant or intolerant to plasma therapy, or (ii) were receiving plasma therapy chronically. The studies include adolescent and adult patients and are ongoing.

Pediatric Study

Alexion has commenced a Phase 2, open-label, single-arm, multi-center study of eculizumab in pediatric patients with aHUS in the United States, European Union and Canada. Information about the trial is posted to www.clinicaltrials.gov, Identifier Number NCT01193348. Physicians and families who are interested in participating in this clinical trial can learn more by contacting Alexion by e-mail at clinicaltrials@alxn.com, or by visiting the Alexion website at www.alexionpharma.com and clicking on the clinical trials link.

About aHUS

aHUS is a chronic, ultra-rare disease characterized by thrombotic microangiopathy (TMA), the formation of blood clots in small blood vessels throughout the body, causing a reduction in platelet count (thrombocytopenia) and life-threatening damage to

the kidney, brain, heart and other vital organs.[5-7] Approximately 60 percent of patients with aHUS require dialysis, a kidney transplant or die within a year of diagnosis, despite currently available care.[2] The majority of patients with aHUS who receive a kidney transplant experience severe complications of the disease, and more than 90 percent of these patients experience failure of the donor kidney.[8]

aHUS is caused by uncontrolled activation of the complement system. When naturally occurring complement inhibitors are absent or do not function normally, the complement system becomes chronically uncontrolled, causing ongoing inflammation and blood clots in vital organs[1,9] In patients with aHUS, uncontrolled complement activation results in an ongoing risk of sudden and catastrophic life-threatening complications.

About Soliris

Soliris (eculizumab) is not approved for the treatment of patients with aHUS and is being provided to patients in clinical studies on an investigational basis. Soliris has been approved by the healthcare authorities in the United States, European Union, Japan and other countries as the first treatment for patients with paroxysmal nocturnal hemoglobinuria (PNH), a rare, debilitating and life-threatening blood disorder defined by hemolysis, or the destruction of red blood cells. Prior to these approvals, there was no therapy specifically available for the treatment of PNH. Soliris is a first-in-class terminal complement inhibitor developed from the laboratory through regulatory approval and commercialization by Alexion.

Patients with PNH in more than 20 countries now have access to Soliris therapy through national or private healthcare providers. As the first terminal complement inhibitor to be approved in countries around the world for any indication, Soliris represents a long-sought breakthrough in medical innovation. Alexion's innovative 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 working to develop and deliver life-changing drug therapies for patients with serious and life-threatening medical conditions. Alexion is engaged in the discovery, development and commercialization of therapeutic products aimed at treating patients with a wide array of severe disease states, including hematologic and kidney diseases, transplant, other inflammatory disorders, and cancer. Soliris (eculizumab) is Alexion's first marketed product. Alexion is evaluating other potential indications for eculizumab as well as other formulations of eculizumab for additional clinical indications, and is pursuing development of other antibody product candidates in early stages of development. This press release and further information about Alexion Pharmaceuticals, Inc. can be found at: www.alexionpharma.com.

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Safe Harbor Statement

This news release contains forward-looking statements, including statements related to anticipated clinical development milestones and potential health and medical benefits of Soliris (eculizumab) for the potential treatment of patients with aHUS. 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, the possibility that results of published reports or clinical trials are not

predictive of safety and efficacy results of Soliris in broader patient populations, the risk that clinical trials may not be completed successfully, the possibility that initial results of commercialization are not predictive of future rates of adoption of Soliris, the risk that third parties won't agree to license any necessary intellectual property to Alexion on reasonable terms or at all, the risk that third party payors will not reimburse for the use of Soliris at acceptable rates or at all, 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 June 30, 2010, 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.

References

(1) Hosler GA, Cusumano AM, Hutchins GM. Thrombotic thrombocytopenic purpura and hemolytic uremic syndrome are distinct pathologic entities. A review of 56 autopsy cases. Arch Pathol Lab Med 2003 Jul;127(7):834-9.

(2) Loirat C, Noris M, Fremeaux-Bacchi V. Complement and the atypical hemolytic uremic syndrome in children. Pediatr Nephrol. 2008 Nov;23(11):1957-72.

(3) Abstract 1338 entitled "Safety and Efficacy of Eculizumab in aHUS Patients Resistant to Plasma Therapy: Interim Analysis from a Phase II Trial," presented in an oral presentation at the American Society of Nephrology (ASN) Annual Meeting on Saturday, November 20, 2010 at 5:30 p.m. by Dr. Christophe Legendre.

(4) Abstract 157 entitled "Safety and Efficacy of Eculizumab in aHUS Patients on Chronic Plasma Therapy: Interim Analysis of a Phase II Trial," presented in a poster presentation at the American Society of Nephrology (ASN) Annual Meeting on Friday, November 19, 2010 from 10:00 a.m. - 2:30 p.m. by Dr. Petra Muus.

(5) Alexion Pharmaceuticals. Soliris in Other Kidney Disorders. Alexion Pharmaceuticals Corporate Website 2010 September 24 Available from: URL: http://www.alexionpharma.com/RandD/Soliris%20In%20Other/kidney%20disorders.aspx#A1

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(11) Atypical Hemolytic Uremic Syndrome. The National Organization for Rare Disorders (NORD) Rare Disease Database, Feb. 11, 2009. Available for purchase at http://www.rarediseases.org/search/rdbdetail_abstract.html?disname=Atypical%20Hemolytic%20Uremic%20Syndrome.

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