Researchers Present Final Data from Phase 2 Studies of Soliris® (eculizumab) in Patients with aHUS

Clinical Data from Retrospective Study in Pediatric Patients with aHUS Also Presented at EHA Congress

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN) today announced the presentation of final data from the two Phase 2 studies of Soliris® (eculizumab) as a treatment for patients with atypical hemolytic uremic syndrome (aHUS): (i) a study in patients who were resistant to plasma exchange/infusion and received eculizumab and (ii) a study in patients who were receiving chronic plasma exchange/infusion followed by late intervention with eculizumab. Consistent with previously announced data, both studies met their primary endpoints and key secondary endpoints with high levels of statistical and clinical significance. Separately, researchers presented for the first time findings from a retrospective clinical study of eculizumab in pediatric patients with aHUS. These three studies were presented at the 16th Congress of the European Hematology Association (EHA) in London.

aHUS is an ultra-rare, genetic, life-long disease in which chronic uncontrolled complement activation causes blood clots in small blood vessels throughout the body (thrombotic microangiopathy, or TMA), leading patients to suffer kidney failure, stroke, heart attack and death.[1,2,3] Approximately 60% of patients with aHUS require dialysis, undergo a kidney transplant, or die within one year of diagnosis.[4] Alexion has filed marketing applications with the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) for eculizumab, a first-in-class terminal complement inhibitor, as a treatment for patients with aHUS.

"Final data from these pivotal clinical trials suggest that complement inhibition therapy with eculizumab could potentially alter the course of aHUS," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "By considerably reducing TMA, investigators have demonstrated that eculizumab improved kidney function, reduced the need for interventions such as dialysis, and substantially improved quality of life in the studied patients. We continue to work closely with regulatory authorities and the aHUS community with the goal of offering a safe and effective treatment option for patients suffering with this ultra-rare, devastating, and life-threatening disease."

Patients Resistant to Plasma Exchange/Infusion

In a poster session today, researchers presented final data from a Phase 2 study of eculizumab in 17 adult and adolescent patients who were resistant or intolerant to plasma exchange/infusion and received eculizumab.[7] Fifteen patients received eculizumab therapy for 26 weeks; two patients did not complete the study but were included in the analysis. Of note, patients in this study had a median duration of aHUS from diagnosis to screening of 10 months (range: 1—236).

The study met its primary endpoint and key secondary endpoints with high levels of statistical and clinical significance, and findings were consistent with data reported at the 2010 American Society of Nephrology annual meeting. For the primary endpoint, platelet count increased from baseline through week 26 by a point estimate 73×10^9/L (p=0.0001). Platelet count was normalized in 13 out of 15 patients (87%) who had abnormal platelets at baseline; 100% of patients who were analyzed per protocol achieved normal platelet count.

With regard to secondary endpoints, 15 patients (88% of the total enrollment and 100% of those analyzed per protocol) achieved TMA event-free status, defined as at least 12 consecutive weeks of stable or increasing platelet counts, absence of plasma exchange/infusion, and no new dialysis. Median TMA intervention (plasma exchange/infusion/dialysis) rate decreased from 0.88 events per patient per day to 0 (p<0.0001). Four out of 5 dialysis patients became dialysis-free, and 10 patients (59%; 95% CI 33-82) had sustained improvement in chronic kidney disease by at least one stage. Eight out of 17 patients (47%; 95% CI 23-82) had increased renal function by at least 15 mL/min/1.73 m^2. Improvements in health-related quality of life (HRQoL) were highly statistically significant, with 80% of patients experiencing a clinically meaningful benefit. The mean change in HRQoL from baseline to week 26 of eculizumab treatment was 0.29±0.28 (p<0.002). Eculizumab was well tolerated in the study. The most frequently reported adverse events were headache, anemia and diarrhea (generally mild to moderate in severity). An extension trial continues.

Patients Receiving Chronic Plasma Exchange/Infusion

In another poster presentation today, researchers presented final data from a Phase 2 study of Soliris in aHUS patients who were receiving chronic plasma exchange/infusion.[5] In this 26-week study, 20 patients continued to receive plasma

exchange/infusion during an 8-week observation period, and then discontinued plasma exchange/infusion and commenced eculizumab treatment. Of note, patients in this study received late intervention with eculizumab: they had a median duration of aHUS from diagnosis to screening of 48 months (range: 0.66—286), with a long duration of renal insufficiency. In the study, 80% of patients achieved TMA event-free status, the primary endpoint, defined as at least 12 consecutive weeks of stable platelet count, absence of plasma exchange/infusion, and no new dialysis.

Key secondary endpoints were also achieved with high clinical and statistical significance. Platelet count normalization was achieved in 18 out of 20 patients (90%); all patients who had normal platelet count at baseline (17 out of 20) maintained that level after discontinuation of plasma exchange/infusion and commencement of eculizumab treatment. TMA intervention rate (number of plasma exchange/infusion and new dialysis events per patient per day) decreased from a median of 0.23 events per patient per day to zero events ($p<0.0001$). Seven out of 20 patients (35%, 95% CI 15 — 59) experienced a sustained improvement of at least one stage of chronic kidney disease (CKD). During treatment with eculizumab, all patients (100%) discontinued plasma exchange/infusion and did not require new dialysis.

In addition, treatment with eculizumab significantly improved quality of life, with 73% of patients achieving a clinically meaningful benefit. Mean change from baseline to week 26 was 0.11 ± 0.19 ($p<0.002$). Eculizumab was well tolerated by patients in the study. The most frequently reported adverse events were diarrhea, headache, hypertension and nausea (generally mild to moderate in severity).

"The data presented today demonstrate again that eculizumab has a profound impact on TMA — the underlying cause of life-threatening events in patients with aHUS — which leads to permanent discontinuation of plasma exchange, significant improvements in kidney function and other critical measures in these patients," said Chantal Loirat, M.D., Pediatric Nephrology Department, Hôpital Robert Debre, Paris.

Pediatric Patients

In a poster session on June 10, 2011, researchers presented data for the first time from a retrospective clinical study of 15 pediatric patients with aHUS who received at least one dose of eculizumab outside of prospective clinical trials between 2007 and 2009.[6] All patients were under the age of 12 (under 2 years [n=5], 2 to 4 years [n=3], 5 to 11 years [n=7]).

The analysis showed that eculizumab treatment significantly reduced TMA in pediatric patients with aHUS, as platelet counts were normalized in 93% (14/15) and 73% (11/15) of patients achieved TMA-event free status. Importantly, 53% of patients had improved renal function with eculizumab treatment. In addition, four of six patients with dialysis in the 30 days prior to eculizumab did not require dialysis during eculizumab treatment. Eculizumab efficacy was similar across all pediatric age groups. The most frequently reported adverse events were fever, upper respiratory tract infection, diarrhea and cough. The results in this pediatric population are consistent with those from controlled trials in adult and adolescent patients in demonstrating that eculizumab controlled TMA, improved kidney function and reduced the need for plasma exchange/infusion.

Separately, Alexion is currently enrolling patients in a Phase 2, open-label, single-arm, multi-center study of Soliris in pediatric patients with aHUS in the United States, European Union and Canada. Information about the trial is available at www.clinicaltrials.gov, Identifier Number NCT01193348, or by contacting Alexion at clinicaltrials@alxn.com.

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.[1-3] Approximately 60 percent of patients with aHUS require dialysis or a kidney transplant or die within a year of diagnosis, despite currently available care.[4] 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 a progressive disease caused by chronic uncontrolled activation of the complement system due to genetic deficiency in complement regulatory genes. With genetic deficiency of naturally occurring complement inhibitors, patients experience life-long uncontrolled activation of the complement system, causing ongoing inflammation and blood clots in vital organs.[9,10] In patients with aHUS, uncontrolled complement activation results in an ongoing risk of sudden and catastrophic life-threatening complications.

About Soliris

Soliris is a first-in-class terminal complement inhibitor developed from the laboratory through regulatory approval and commercialization by Alexion. Soliris has been approved in the U.S., European Union, Japan and other territories as the first treatment for patients with PNH, a debilitating, ultra-rare 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. Soliris (eculizumab) is not approved for the treatment of aHUS or any indication 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. Alexion is the global leader in complement inhibition, and has developed and markets Soliris® (eculizumab) as a treatment for patients with PNH, a debilitating, ultra-rare and life-threatening blood disorder. Soliris is approved in more than 35 countries. 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: www.alexionpharma.com.

Safe Harbor Statement

This news release contains forward-looking statements, including statements related to anticipated clinical development, regulatory and commercial 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, 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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(5) Abstract 1587 entitled "A phase II study of eculizumab in patients with atypical hemolytic uremic syndrome receiving chronic plasma exchange/infusion," presented by Dr. Chantal Loirat at the 16th Congress of the European Hematology Association, Sunday, June 12, 2011.

(6) Abstract 396 entitled "Eculizumab therapy for atypical hemolytic uremic syndrome in pediatric patients: Efficacy and safety outcomes from a retrospective study," presented by Dr. Giacomo Simonetti at the 16th Congress of the European Hematology Association, Friday, June 10, 2011.

(7) Abstract 1588 entitled "Eculizumab efficacy and safety in patients with atypical hemolytic uremic syndrome resistant to plasma exchange/infusion," presented by Dr. Chantal Loirat at the 16th Congress of the European Hematology Association, Sunday, June 12, 2011.

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Alexion Pharmaceuticals, Inc.
Irving Adler, 203-271-8210
Senior Director, Corporate Communications and Public Policy
or
Media:
Makovsky & Company
Mark Marmur, 609-354-8135
or
Investors:
Rx Communications
Rhonda Chiger, 917-322-2569

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