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Late-Breaking Data Presented at EHA: All Patients with PNH Treated with Once-Monthly Dosing of ALXN1210 in Phase 1/2 Study Exhibit Rapid and Sustained Reductions in LDH

Additional Late-Breaking Data Presented in Patients with Acute GI-GVHD Showing High Overall Response Rate with ALXN1007

NEW HAVEN, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) announced today that interim data were presented from a Phase 1/2 study of ALXN1210, an investigational, highly innovative longer-acting anti-C5 antibody, in patients with paroxysmal nocturnal hemoglobinuria (PNH), a debilitating, ultra-rare blood disorder characterized by complement-mediated hemolysis (destruction of red blood cells).[1] In this study, once-monthly dosing of ALXN1210 achieved rapid and sustained reductions in mean levels of lactate dehydrogenase (LDH), a marker of hemolysis, in 100 percent of treated patients, which were observed through up to five once-monthly dosing intervals. Researchers also reported that, at this time, 80 percent of patients who required at least 1 blood transfusion in the 12 months prior to treatment with ALXN1210 did not require transfusions while on treatment.[2] These findings were presented in a late-breaking poster at the 21st Congress of the European Hematology Association (EHA) in Copenhagen, Denmark.

In a separate late-breaking poster at EHA, additional interim results were presented from a Phase 2 trial evaluating ALXN1007, a novel anti-inflammatory antibody targeting complement protein C5a, in patients with acute graft-versus-host disease of the lower GI tract (GI-GVHD). Acute GI-GVHD is a severe and life-threatening rare autoimmune disease that can occur as a complication of stem cell or bone marrow transplantation.[3,4,5] The study showed an overall 28-day response rate—defined as improvement from diagnosis in any organ by ≥1 stage, without progression in any other organ and no need for additional therapy—of 77 percent in ALXN1007-treated patients.[6]

"Alexion has more than 20 years of experience in complement research and discovery, and we are pleased to have late-breaking data from two of our highly innovative, investigational complement inhibitors, ALXN1210 and ALXN1007, presented at EHA," said Martin Mackay, Ph.D., Executive Vice President and Global Head of R&D at Alexion. "Interim results from the Phase 1/2 study of ALXN1210 in patients with PNH showed rapid, complete, and sustained complement inhibition, as measured by reductions in LDH levels, with a once-monthly dosing regimen in all treated patients. A Phase 2 study is ongoing to evaluate the safety and efficacy of ALXN1210 in additional dosing cohorts evaluating longer dosing intervals."

ALXN1210, a Long-Acting C5 Inhibitor, Results in Rapid and Sustained Reduction of LDH with a Monthly Dosing Interval in Patients with PNH: Preliminary Data from a Dose-Escalation Study (Abstract LB2247)[2]

In a poster session, interim results were presented from a Phase 1/2, open-label, 24-week dose-escalating study of ALXN1210 in patients with PNH. The primary efficacy endpoint was the percent change in LDH levels from baseline; other efficacy endpoints included change in blood transfusion requirements and change in hematologic parameters from baseline. Patients with PNH (aged 18 and older; n=13) with mean LDH levels ≥3 times the upper limit of normal and who were complement inhibitor-naïve were separated into two study cohorts. Patients in Cohort 1 (n=6) received either 400 mg or 600 mg induction doses of ALXN1210, followed by a 900 mg maintenance dose once-monthly. Patients in Cohort 2 (n=7) received 600 mg and 900 mg induction doses of ALXN1210, followed by an 1,800 mg maintenance dose once-monthly.

All patients showed rapid reductions in mean LDH levels at Day 8 (the first evaluable time point of the study), which were sustained for up to five once-monthly dosing intervals. At the most recent evaluable time point, the mean percentage reduction in LDH levels from baseline was 85.4 percent in Cohort 1 (Day 148) and 86.0 percent in Cohort 2 (Day 85). Among five patients with one or more transfusions in the year prior to the study, only one patient, from Cohort 1, required a transfusion during treatment with ALXN1210. This patient received two units of packed red blood cells (RBC) while receiving ALXN1210, compared to 12 units of RBC in the six months prior to ALXN1210. In addition, mean levels of hemoglobin, another direct marker of intravascular hemolysis, were improved or stable in both cohorts.

"PNH is a devastating, ultra-rare blood disorder caused by uncontrolled activation of complement, putting patients at risk for severe and life-threatening consequences," said lead author Jong-Wook Lee, M.D., of The Catholic University of Korea, Seoul St. Mary's Hospital, Seoul, Korea. "The interim data presented at EHA suggest that treatment with ALXN1210 results in effective blockade of complement-mediated hemolysis and reduces transfusion requirements in patients with PNH. All patients achieved rapid decreases in LDH levels that were sustained through extended, once-monthly dosing intervals, consistent with the longer half-life of ALXN1210."

No serious adverse events or study withdrawals were observed in either patient cohort. The most common treatment-related adverse events were headache and upper respiratory tract infection (each occurring in 3 patients), which resolved during ongoing treatment with ALXN1210.

Phase 2A Study of ALXN1007, A Novel C5a Inhibitor, in Subjects with Newly Diagnosed Acute Graft-Versus-Host Disease (GVHD) Involving the Lower Gastrointestinal Tract (Abstract LB2269)[6]

In a poster session, additional interim results were presented from an ongoing Phase 2, open-label study of ALXN1007 in patients with newly diagnosed acute GI-GVHD. The primary efficacy endpoint is the overall acute GVHD response rate at Day 28. Other efficacy endpoints include complete GI-GVHD response rate at Day 28 and Day 56. Patients were treated once-weekly with 10 mg/kg of ALXN1007 for eight weeks in combination with methylprednisolone or equivalent, with one year of follow-up.

At both Day 28 and Day 56, the overall acute GVHD response rate was 77 percent in 13 evaluable patients. Complete GI-GVHD response rates at Days 28 and 56 were 69 percent and 77 percent, respectively. Additionally, at Day 180, the non-relapse mortality rate from causes other than the underlying malignancy was 12.5 percent, and the overall survival rate was 69.2 percent, among 13 evaluable patients.

The study also evaluated the degree of C5a inhibition relative to PK and acute GI-GVHD response suggesting that higher doses and frequency may be needed to optimize C5a inhibition and maximize clinical response. The trial protocol was subsequently amended to evaluate an ALXN1007 dose of 20 mg/kg weekly and twice-weekly.

Two patients (13 percent) experienced serious treatment-related adverse events and one patient had a grade 2 infusion-related reaction. There were no grade 3 or higher non-serious adverse events related to treatment with ALXN1007. One patient withdrew from the study due to a treatment-emergent adverse event (relapse of T-cell lymphoma). Six deaths were reported, none of which were considered related to treatment with ALXN1007.

About Paroxysmal Nocturnal Hemoglobinuria (PNH)

PNH is an ultra-rare blood disorder in which chronic, uncontrolled activation of complement, a component of the normal immune system, results in hemolysis (destruction of the patient's red blood cells). PNH strikes people of all ages, with an average age of onset in the early 30s.[1] Approximately 10 percent of all patients first develop symptoms at 21 years of age or younger.[7] PNH develops without warning and can occur in men and women of all races, backgrounds and ages. PNH often goes unrecognized, with delays in diagnosis ranging from one to more than 10 years.[8] In the period of time before Soliris® (eculizumab) was available, it had been estimated that approximately one-third of patients with PNH did not survive more than five years from the time of diagnosis.[1] PNH has been identified more commonly among patients with disorders of the bone marrow, including aplastic anemia (AA) and myelodysplastic syndromes (MDS).[9,10,11] In patients with thrombosis of unknown origin, PNH may be an underlying cause.[1]

About ALXN1210

ALXN1210 is a highly innovative, longer-acting C5 antibody being evaluated by Alexion for the treatment of patients with PNH. In early studies, ALXN1210 has demonstrated rapid, complete, and sustained reduction of free C5 activity and a terminal half-life of more than 30 days, which may facilitate a monthly or longer dosing interval.[12] Alexion is conducting two clinical studies of ALXN1210 in patients with PNH—a Phase 1/2 dose-escalating study and an open-label, multi-dose Phase 2 study.

About Graft-Versus-Host Disease of the Lower GI tract (GI-GVHD)

GI-GVHD is an immune-mediated disease that affects 10 to 12 percent of patients who receive an allogeneic hematopoietic stem cell transplant.[3,4] Patients with severe, acute GI-GVHD have a 30 to 40 percent mortality rate within the first six months post-transplant.[13] There are no approved treatments for GI-GVHD.

About ALXN1007

ALXN1007 is a novel anti-inflammatory antibody targeting complement protein C5a being evaluated in a Phase 2 trial for patients with acute GI-GVHD.

About Soliris® (eculizumab)

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 U.S. (2007), European Union (2007), Japan (2010) and other countries as the first and only treatment for patients with paroxysmal nocturnal hemoglobinuria (PNH) to reduce hemolysis. PNH is 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 U.S. (2011), European Union (2011), Japan (2013) and other countries as the first and only treatment for patients with atypical hemolytic uremic syndrome (aHUS) to inhibit complement-mediated thrombotic microangiopathy, or TMA (blood clots in small vessels). aHUS is a debilitating, ultra-rare and life-threatening genetic disorder characterized by complement-mediated TMA. Soliris is not indicated for the treatment of patients with Shiga-toxin E. coli-related hemolytic uremic syndrome (STEC-HUS). For the breakthrough medical innovation in complement inhibition, Alexion and Soliris have received some of the pharmaceutical industry's highest honors: the Prix Galien USA (2008, Best Biotechnology Product) and France (2009, Rare Disease Treatment).

More information, including the full U.S. prescribing information, on Soliris is available at www.soliris.net.

Important Safety Information

The U.S. 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 [see Warnings and Precautions (5.1)]. 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 two 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 Warnings and Precautions (5.1) for additional guidance on the management of the risk 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 [see Warnings and Precautions (5.2)]. 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 headache, diarrhea, hypertension, upper respiratory infection, abdominal pain, vomiting, nasopharyngitis, anemia, cough, peripheral edema, nausea, urinary tract infections, and pyrexia. Soliris is not indicated for the treatment of patients with Shiga-toxin E. coli-related hemolytic uremic syndrome (STEC-HUS). Please see full prescribing information for Soliris, including BOXED WARNING regarding risk of serious meningococcal infection.

About Alexion

Alexion is a global biopharmaceutical company focused on developing and delivering life-transforming therapies for patients with devastating and rare disorders. Alexion is the global leader in complement inhibition and has developed and commercializes the first and only approved complement inhibitor to treat patients with paroxysmal nocturnal hemoglobinuria (PNH) and atypical hemolytic uremic syndrome (aHUS), two life-threatening ultra-rare disorders. In addition, Alexion's metabolic franchise includes two highly innovative enzyme replacement therapies for patients with life-threatening and ultra-rare disorders, hypophosphatasia (HPP) and lysosomal acid lipase deficiency (LAL-D). Alexion is advancing the most robust rare disease pipeline in the biotech industry with highly innovative product candidates in multiple therapeutic areas. This press release and further information about Alexion can be found at: www.alexion.com.

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Forward-Looking Statements

This news release contains forward-looking statements, including statements related to potential medical benefits of ALXN1210 for paroxysmal nocturnal hemoglobinuria (PNH), and ALXN1007 for gastrointestinal graft versus host disease (GI-GVHD). Forward-looking statements are subject to factors that may cause Alexion's results and plans to differ from those expected, including, for example, risks and uncertainties of drug development, decisions of regulatory authorities regarding marketing approval or material limitations on the marketing of ALXN1210 for PNH and ALXN1007 for GI-GVHD, delays in arranging satisfactory manufacturing capabilities and establishing commercial infrastructure for our products and product candidates, the possibility that results of clinical trials are not predictive of safety and efficacy results of our products in broader or different patient populations, the risk that estimates regarding the number of patients with PNH and GI-GVHD, 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, 2016 and in Alexion's other filings with the SEC. 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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