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European Commission Grants Orphan Drug Designation to ALXN1210 for the Treatment of Patients with Paroxysmal Nocturnal Hemoglobinuria (PNH)

NEW HAVEN, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today announced that the European Commission has granted orphan drug designation (ODD) to ALXN1210, a highly innovative, longer-acting C5 antibody being evaluated in patients with paroxysmal nocturnal hemoglobinuria (PNH). PNH is a debilitating, ultra-rare, life-threatening blood disorder in which uncontrolled activation of complement, a component of the immune system, results in hemolysis (destruction of a patient's red blood cells).

"Patients with PNH are at continuous risk for severe and life-threatening consequences resulting from the ongoing complement-mediated destruction of red blood cells," said Martin Mackay, Ph.D., Executive Vice President and Global Head of R&D at Alexion. "Soliris has been approved for the treatment of patients with PNH since 2007 and has dramatically changed the outlook for patients with this disease. Preliminary results from our ongoing clinical studies of ALXN1210, which has a half-life nearly three times that of Soliris, have shown rapid, complete, and sustained complement inhibition in treated patients with PNH. We are pleased that the Committee for Orphan Medicinal Products recognized that ALXN1210 has the potential to offer a meaningful clinical advantage for patients living with this devastating ultra-rare disorder."

Alexion is evaluating ALXN1210 in a Phase 1/2 study and a Phase 2 study in patients with PNH. Both studies have exceeded target enrollment. More information on these clinical trials is available at www.clinicaltrials.gov under the identifiers NCT02598583 and NCT02605993. Alexion also plans to initiate a clinical program with ALXN1210 in patients with atypical hemolytic uremic syndrome (aHUS), another ultra-rare and life-threatening disease caused by chronic uncontrolled complement activation, in 2016. ALXN1210 is not approved in any country.

The European Commission grants orphan medicinal product status to provide incentives to develop medicinal products to treat, prevent or diagnose diseases or conditions that affect no more than five in 10,000 persons in the EU. The orphan medicinal product status designation provides Alexion with certain benefits and incentives in the EU, including a period of market exclusivity if ALXN1210 is approved to treat patients with PNH.

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.[2] 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.[3] In the period of time before Soliris 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).[4,5,6] 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. 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 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 on Soliris, including the full U.S. prescribing information, 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 the treatment of paroxysmal nocturnal hemoglobinuria (PNH). 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 our products, delays, interruptions or failures in the manufacture and supply of our products and our product candidates, progress in establishing and developing commercial infrastructure, failure to satisfactorily address matters raised by the FDA and other regulatory agencies, the possibility that results of clinical trials are not predictive of safety and efficacy results of our products in broader patient populations in the disease studied or other diseases, the risk that strategic transactions will not result in short-term or long-term benefits, the possibility that current results of commercialization are not predictive of future rates of adoption of Soliris in PNH, aHUS or other diseases, the possibility that clinical trials of our product candidates could be delayed or that additional research and testing is required by regulatory agencies, the adequacy of our pharmacovigilance and drug safety reporting processes, the risk that third party payors (including governmental agencies) will not reimburse or continue to reimburse for the use of our products at acceptable rates or at all, risks regarding government investigations, including the SEC and DOJ investigations, the risk that estimates regarding the number of patients with PNH, aHUS, HPP and LAL-D are inaccurate, the risks of shifting foreign exchange rates, and a variety of other risks set forth from time to time

in Alexion's filings with the U.S. 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 our other filings with the U.S. 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

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Alexion Pharmaceuticals, Inc.
Media
Stephanie Fagan, 203-271-8223
Senior Vice President, Corporate Communications
OR
Amanda Fahey, 203-699-7240
Associate Director, Corporate Communications
OR
Investors
Elena Ridloff, CFA, 203-699-7722
Vice President, Investor Relations

Source: Alexion Pharmaceuticals, Inc.