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FDA 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 U.S. Food and Drug Administration (FDA) has granted orphan drug designation (ODD) to ALXN1210, a highly innovative, longer-acting anti-C5 antibody that inhibits terminal complement, which is being evaluated for the treatment of 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).[1]

"Alexion is committed to achieving the highest levels of innovation to address the needs of patients suffering from PNH, a devastating ultra-rare disorder," said Martin Mackay, Ph.D., Executive Vice President and Global Head of R&D at Alexion. "We are pleased that the FDA has recognized the potential for ALXN1210 to offer a significant therapeutic advantage for patients with PNH. Data from our ongoing clinical studies have shown rapid, complete, and sustained complement inhibition in treated patients, and we look forward to continuing to evaluate this highly innovative molecule in our Phase 3 trial of ALXN1210 administered every eight weeks."

Alexion is currently enrolling patients in Phase 3 trials of ALXN1210 in patients with PNH as well as in patients with atypical hemolytic uremic syndrome (aHUS), another ultra-rare and life-threatening disease caused by chronic uncontrolled complement activation. More information on these clinical trials is available at www.clinicaltrials.gov under the identifiers NCT02946463 and NCT02949128. In June 2016, ALXN1210 was granted ODD by the European Commission for the treatment of patients with PNH. ALXN1210 is protected by a composition of matter patent in the U.S. and Europe through 2035. ALXN1210 is not approved in any country.

The FDA, through its Office of Orphan Products Development (OOPD), grants orphan status to drugs and biologic products that are intended for the safe and effective treatment, diagnosis, or prevention of rare diseases or disorders that affect fewer than 200,000 people in the United States. ODD provides a drug developer with certain benefits and incentives, including a period of marketing exclusivity if regulatory approval is ultimately received for the designated indication.

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 treatment 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.[4] PNH has been identified more commonly among patients with disorders of the bone marrow, including aplastic anemia (AA) and myelodysplastic syndromes (MDS).[5,6,7] In patients with thrombosis of unknown origin, PNH may be an underlying cause.[8]

About ALXN1210

ALXN1210 is a highly innovative, longer-acting anti-C5 antibody discovered and developed by Alexion that inhibits terminal complement. In early studies, ALXN1210 demonstrated rapid, complete, and sustained reduction of free C5 levels.[9] Alexion has completed enrollment in two ongoing clinical studies of ALXN1210 in patients with PNH—a Phase 1/2 dose-escalating study and an open-label, multi-dose Phase 2 study that is also evaluating longer dosing intervals beyond 8 weeks.

ALXN1210 is currently in Phase 3 trials in patients with PNH and aHUS. In addition, Alexion is conducting a Phase 1 study to evaluate a new formulation of ALXN1210 administered subcutaneously in healthy volunteers.

In June 2016, the European Commission granted Orphan Drug Designation (ODD) to ALXN1210 for the treatment of patients with PNH.

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 press release contains forward-looking statements, including statements related to Alexion's development plans for ALXN1210, the medical benefits of ALXN1210 for the treatment of PNH and aHUS, medical and commercial potential of ALXN1210, and plans for regulatory filings for ALXN1210. Forward-looking statements are subject to factors that may cause Alexion's results and plans to materially 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, including for ALXN1210, 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 investigations of Alexion by the SEC and DOJ, the risk that anticipated regulatory filings are delayed, including for ALXN1210, 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 September 30, 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.

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