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FDA Grants Orphan Drug Designation to ALXN1007 for the Treatment of Patients with Graft-Versus-Host Disease (GVHD)

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 ALXN1007, a novel anti-inflammatory monoclonal antibody targeting complement protein C5a, for the treatment of acute graft-versus-host disease (GVHD). Alexion is currently investigating ALXN1007 in patients with acute GVHD of the lower gastrointestinal tract (GI-GVHD), a severe and life-threatening rare autoimmune disease that can occur as a complication of stem cell or bone marrow transplantation.[1,2,3]

"There is an urgent need for an effective treatment for GI-GVHD, which causes severe symptoms and often leads to mortality following allogeneic hematopoietic stem cell transplantation. Currently, even with best available care, about one-third of severely affected patients die from this devastating complication within six months of onset," said Martin Mackay, Ph.D., Executive Vice President and Global Head of R&D at Alexion. "Alexion is pleased that the FDA has granted orphan drug designation to ALXN1007, recognizing its potential to improve response rates in patients facing acute GI-GVHD."

ALXN1007 is currently being evaluated in a Phase 2 study in patients with newly diagnosed acute GI-GVHD. More information on this trial is available at www.clinicaltrials.gov under the identifier NCT02245412. In August of 2016, ALXN1007 was granted ODD by the European Commission. ALXN1007 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 Graft-Versus-Host Disease of the Lower GI tract (GI-GVHD)

GI-GVHD is an immune-mediated disease that affects approximately 10 percent of patients who receive an allogeneic hematopoietic stem cell transplant or bone marrow transplant.[1,2,4] Patients with severe, acute GI-GVHD have a 30 to 40 percent mortality rate within the first six months post-transplant.[5] There are currently limited treatment options in acute GI-GVHD.

About ALXN1007

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

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 ALXN1007 for the treatment of graft-versus-host disease (GVHD). 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 June 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.

References

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Alexion Pharmaceuticals, Inc.
Media:
Stephanie Fagan, 475-230-3777
Senior Vice President, Corporate Communications
or
Kim Diamond, 475-230-3775
Executive Director, Corporate Communications
or
Investors:
Elena Ridloff, CFA, 475-230-3601
Vice President, Investor Relations

Source: Alexion Pharmaceuticals, Inc.