



August 29, 2016

European Commission 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 European Commission has granted orphan drug designation (ODD) to ALXN1007, a novel anti-inflammatory monoclonal antibody targeting complement protein C5a, for the treatment of 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}

"Acute GI-GVHD is a devastating disease in which about one-third of severely affected patients die within the first six months following transplant despite best available care," said Martin Mackay, Ph.D., Executive Vice President and Global Head of R&D at Alexion. "We are pleased that ALXN1007 has received orphan drug designation in the EU, recognizing the urgent need for an effective treatment for GVHD and the potential of ALXN1007 to improve response rates in patients facing this severe and life-threatening disorder."

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. ALXN1007 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 ALXN1007 is approved to treat patients with acute GI-GVHD.

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 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 commercialized 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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