May 19, 2016

Researchers to Present Late-Breaking Data on ALXN1210 in Patients with PNH at EHA Annual Congress

-Additional Data, Including Late-Breaking Results from Phase 2 Trial of ALXN1007 in Patients with GI-GVHD, Also to be Presented-

NEW HAVEN, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today announced that researchers will present late-breaking data from a Phase 1/2 dose-escalating study of ALXN1210, the Company's highly innovative longer-acting C5 antibody, in patients with paroxysmal nocturnal hemoglobinuria (PNH). In another late-breaking poster, researchers will present additional results from a Phase 2 trial evaluating ALXN1007 in patients with acute graft-versus-host disease involving the lower gastrointestinal tract (GI-GVHD). Data will also be presented from an observational study in Japan (OPTIMA) evaluating change in PNH clone size in patients with bone marrow failure. These findings will be presented at the 21st Congress of the European Hematology Association (EHA), being held June 9-12, 2016, in Copenhagen, Denmark.

PNH is a debilitating, ultra-rare and life-threatening blood disorder characterized by complement-mediated hemolysis (destruction of red blood cells).[1] Acute GI-GVHD is a severe and life-threatening rare auto-immune disease that can occur as a complication of stem cell transplantation.[2,3]

Abstracts summarizing these presentations were published on the EHA website and can be accessed using the links below.

The following late-breaking abstracts will be presented in a poster session available from Friday, June 10, 2016, at 9:30 a.m., Central European Summer Time (CEST) to Saturday, June 11, 2016, at 7:00 p.m., CEST:

- Abstract LB2247: "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," Lee, et al.
- Accessible at: http://learningcenter.ehaweb.org/eha/2016/21st/135358/jong.wook.lee.alxn1210.a.long-acting.c5.inhibitor.results.in.rapid.and.html?f=m3
- Abstract LB2269: "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," Alousi, et al.
- Accessible at: http://learningcenter.ehaweb.org/eha/2016/21st/135380/amin.majid.alousi.phase.2a.study.of.alxn1007.a.novel.c5a.inhibitor.in.subjects.html?f=m3

The following abstract also will be presented during the poster session from Friday, June 10, 2016, at 9:30 a.m., CEST to Saturday, June 11, 2016, at 7:00 p.m., CEST:

- Abstract P634: "A Clinical Significance and Time-Dependent Change of PNH Clone Size in Patients with Bone Marrow Failure Syndrome: Japanese Multi-Centre Prospective Study," Shirasugi, et al.
- Accessible at: http://learningcenter.ehaweb.org/eha/2016/21st/133522/yukari.shirasugi.a.clinical.significance.and.time-dependent.change.of.pnh.html?f

About 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.[4] 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.[5] 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).[6,7,8] 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 in a Phase 1/2 and Phase 2 study for the treatment of patients with PNH.

About GI-GVHD

Acute GI-GVHD is an immune-mediated disease and a complication of stem cell transplantation occurring in 10 to 12 percent of allogeneic hematopoietic stem cell transplants.[2,3] Patients with severe acute GI-GVHD have a 30 to 40 percent mortality rate within the first six months post-transplant.[9] 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 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.

[ALXN-G]

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