



November 3, 2016

Researchers to Present New Phase 1/2 Data on ALXN1210 in Patients with PNH at ASH 2016 Annual Meeting

Additional Data Enhancing the Understanding of PNH and aHUS also to be Presented

NEW HAVEN, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today announced that researchers will present new interim data from a Phase 1/2 dose-escalation study of ALXN1210, the Company's investigational, highly innovative longer-acting anti-C5 antibody, in patients with paroxysmal nocturnal hemoglobinuria (PNH). Researchers will also present data from the OPTIMA PNH observational study in Japan, the Global Atypical Hemolytic Uremic Syndrome (aHUS) Registry, and a single-center retrospective chart review of complement-amplifying conditions in patients with aHUS. These data will be presented at the 58th American Society of Hematology (ASH) Annual Meeting & Exposition, to be held December 3-6, 2016, in San Diego.

PNH is a debilitating, ultra-rare and life-threatening blood disorder characterized by complement-mediated hemolysis (destruction of red blood cells).¹ aHUS is a genetic, chronic, ultra-rare disease associated with vital organ failure and premature death.^{2,3,4}

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

PNH Abstracts

The following abstract will be presented in a poster session on Sunday, December 4, from 6:00 p.m. to 8:00 p.m., Pacific Standard Time (PST):

- | Abstract 2428: "Immediate, Complete, and Sustained Inhibition of C5 with ALXN1210 Reduces Complement-Mediated Hemolysis in Patients with Paroxysmal Nocturnal Hemoglobinuria (PNH): Interim Analysis of a Dose-Escalation Study," Lee, et al.

Accessible at: <https://ash.confex.com/ash/2016/webprogram/Paper90053.html>

The following abstract will be presented in a poster session on Monday, December 5, from 6:00 p.m. to 8:00 p.m., PST:

- | Abstract 3896: "The First Follow-Up Data Analysis of Patients with Acquired Bone Marrow Failure Harboring a Small Population of PNH-type Cells in the Japanese, Multicenter, Prospective Study OPTIMA," Ueda, et al.

Accessible at: <https://ash.confex.com/ash/2016/webprogram/Paper94565.html>

aHUS Abstracts

The following abstract will be presented in a poster session on Sunday, December 4, from 6:00 p.m. to 8:00 p.m., PST:

- | Abstract 2543: "Presentation and Time to Treatment of Atypical Hemolytic Uremic Syndrome Is Influenced By Complement Amplifying Conditions: A Single Center Experience," Cunningham, et al.

Accessible at: <https://ash.confex.com/ash/2016/webprogram/Paper93419.html>

The following abstract will be presented in a poster session on Monday, December 5, from 6:00 p.m. to 8:00 p.m., PST:

- | Abstract 3729: "Systemic Involvement at Entry into the Global Atypical Hemolytic Uremic Syndrome (aHUS) Registry," Scully, et al.

Accessible at: <https://ash.confex.com/ash/2016/webprogram/Paper89868.html>

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.⁵ 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 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.⁶ 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.⁷ 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 5 years from the time of diagnosis.⁸ PNH has been identified more commonly among patients with disorders of the bone marrow, including aplastic anemia (AA) and myelodysplastic syndromes (MDS).⁹⁻¹¹ In patients with thrombosis of unknown origin, PNH may be an underlying cause.¹²

About Atypical Hemolytic Uremic Syndrome (aHUS)

aHUS is a chronic, ultra-rare, and life-threatening disease in which a life-long and permanent genetic deficiency in one or more complement regulatory genes causes chronic uncontrolled complement activation, resulting in complement-mediated thrombotic microangiopathy (TMA), the formation of blood clots in small blood vessels throughout the body.^{2,3} Permanent, uncontrolled complement activation in aHUS causes a life-long risk for TMA, which leads to sudden, catastrophic, and life-threatening damage to the kidney, brain, heart, and other vital organs, and premature death.^{2,4} Seventy-nine percent of all patients with aHUS die, require kidney dialysis or have permanent kidney damage within three years after diagnosis despite plasma exchange or plasma infusion (PE/PI).¹⁵ Moreover, 33-40 percent of patients die or progress to end-stage renal disease with the first clinical manifestation of aHUS despite PE/PI.^{13,14} The majority of patients with aHUS who receive a kidney transplant commonly experience subsequent systemic TMA, resulting in a 90 percent transplant failure rate in these TMA patients.¹³

aHUS affects both children and adults. Complement-mediated TMA also causes reduction in platelet count (thrombocytopenia) and red blood cell destruction (hemolysis). While mutations have been identified in at least ten different complement regulatory genes, mutations are not identified in 40-50 percent of patients with a confirmed diagnosis of aHUS.^{13,15,16}

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.

[ALXN-G]

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