



November 1, 2017

## **New Phase 1/2 Study Data for ALXN1210 as a Potential Future Treatment for Patients with Paroxysmal Nocturnal Hemoglobinuria (PNH) to Be Presented at American Society of Hematology (ASH) Meeting**

*Data from the International PNH Registry Also to Be Presented at the ASH Meeting*

NEW HAVEN, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ: ALXN) announced today that researchers will present new data from two Phase 1/2 clinical studies of ALXN1210, the Company's investigational long-acting complement inhibitor, in patients with paroxysmal nocturnal hemoglobinuria (PNH). PNH is a chronic, progressive, debilitating and potentially life-threatening ultra-rare blood disorder characterized by complement-mediated hemolysis (destruction of red blood cells).<sup>1,2</sup> Researchers will also present data from the International PNH Registry, including efficacy and safety outcomes for Soliris® (eculizumab) treatment for patients with PNH. These data will be presented at the 59<sup>th</sup> American Society of Hematology (ASH) Annual Meeting & Exposition, to be held December 9-12, 2017, in Atlanta.

Abstracts summarizing these presentations can be accessed on the ASH website. The data will be presented in a poster session on Monday, December 11, 2017 from 6:00 p.m. to 8:00 p.m., Eastern Standard Time (EST):

- | Abstract 3482: "Optimization of Dose Regimen for ALXN1210, a Novel Complement C5 Inhibitor, in Patients with Paroxysmal Nocturnal Hemoglobinuria (PNH): Results of 2 Phase 1/2 Studies," Roeth A, Rottinghaus T, Hill A, et al.

Accessible at: <https://ash.confex.com/ash/2017/webprogram/Paper100715.html>

- | Abstract 3486: "Interim Analysis of Safety Outcomes During Treatment With Eculizumab: Results from the International Paroxysmal Nocturnal Hemoglobinuria Registry," Hill A, Roeth A, Socié G, et al.

Accessible at: <https://ash.confex.com/ash/2017/webprogram/Paper101230.html>

- | Abstract 3487: "Efficacy of Eculizumab in Patients with Paroxysmal Nocturnal Hemoglobinuria (PNH) and High Disease Activity with or Without History of Aplastic Anemia in the International PNH Registry," Lee JW, Peffault de Latour R, Brodsky RA, et al.

Accessible at: <https://ash.confex.com/ash/2017/webprogram/Paper101237.html>

- | Abstract 3488: "Analysis of Baseline Clinical Characteristics and Disease Burden in Patients Enrolled in the International Paroxysmal Nocturnal Hemoglobinuria Registry," Schrezenmeier H, Maciejewski JP, Roeth A, et al.

Accessible at: <https://ash.confex.com/ash/2017/webprogram/Paper101263.html>

- | Abstract 3472: "Comparison of Baseline Clinical Characteristics between Asian and non-Asian Patients with Paroxysmal Nocturnal Hemoglobinuria (PNH) from the International PNH Registry," Jang JH, Okamoto S, Sakurai M, et al.

Accessible at: <https://ash.confex.com/ash/2017/webprogram/Paper106034.html>

### **About Paroxysmal Nocturnal Hemoglobinuria (PNH)**

Paroxysmal Nocturnal Hemoglobinuria (PNH) is a chronic, progressive, debilitating and potentially life-threatening ultra-rare blood disorder that can strike men and women of all races, backgrounds and ages without warning, with an average age of onset in the early 30s.<sup>1,2,3</sup> PNH often goes unrecognized, with delays in diagnosis ranging from one to more than 10 years.<sup>2</sup> In patients with PNH, chronic, uncontrolled activation of the complement system, a component of the body's immune system, results in hemolysis (the destruction of red blood cells)<sup>4</sup>, which in turn can result in progressive anemia, fatigue, dark urine and shortness of breath.<sup>5,6,7</sup> The most devastating consequence of chronic hemolysis is thrombosis (the formation of blood

clots), which can damage vital organs and cause premature death.<sup>8</sup> Historically, it had been estimated that one in three patients with PNH did not survive more than five years from the time of diagnosis.<sup>2</sup> PNH is more common among patients with disorders of the bone marrow, including aplastic anemia (AA) and myelodysplastic syndromes (MDS).<sup>9,10,11</sup> In certain patients with thrombosis of unknown origin, PNH may be an underlying cause.<sup>12</sup>

### **About ALXN1210**

ALXN1210 is an innovative, long-acting anti-C5 antibody discovered and developed by Alexion that inhibits the C5 protein in the terminal complement cascade, which is a part of the body's immune system. In early studies, ALXN1210 demonstrated rapid, complete, and sustained reduction of free C5 levels.<sup>13</sup> ALXN1210 is currently being evaluated in Phase 3 clinical studies as a potential treatment for patients with paroxysmal nocturnal hemoglobinuria (PNH) and atypical hemolytic uremic syndrome (aHUS), administered intravenously every eight weeks. In addition, Alexion plans to initiate a Phase 3 clinical study of ALXN1210 delivered subcutaneously once per week as a potential treatment for patients with PNH or aHUS.

ALXN1210 has received Orphan Drug Designation (ODD) for the intravenous treatment of patients with PNH in the U.S. and EU, and for the subcutaneous treatment of patients with aHUS in the U.S.

### **About Soliris® (eculizumab)**

Soliris® is a first-in-class complement inhibitor that works by inhibiting the terminal part of the complement cascade, a part of the immune system that, when activated in an uncontrolled manner, plays a role in serious ultra-rare disorders like paroxysmal nocturnal hemoglobinuria (PNH), atypical hemolytic uremic syndrome (aHUS) and anti-acetylcholine receptor (AChR) antibody-positive myasthenia gravis (MG). Soliris is approved in the U.S., EU, Japan and other countries as the first and only treatment for patients with PNH and aHUS, in the EU as the first and only treatment of refractory gMG in adults who are anti-AChR antibody-positive, and in the U.S. for the treatment of adult patients with gMG who are anti-AChR antibody-positive. Alexion's new drug application in Japan for Soliris as a treatment for patients with anti-AChR antibody-positive refractory gMG has been accepted for review by the Japanese Ministry of Health, Labour and Welfare (MHLW). Soliris is not indicated for the treatment of patients with Shiga-toxin E. coli-related hemolytic uremic syndrome (STEC-HUS).

Soliris has received Orphan Drug Designation (ODD) for the treatment of patients with PNH in the U.S., EU, Japan and many other countries, for the treatment of patients with aHUS in the U.S., EU and many other countries, for the treatment of patients with MG in the U.S. and EU, and for the treatment of patients with refractory gMG in Japan. Alexion and Soliris have received some of the pharmaceutical industry's highest honors for the medical innovation in complement inhibition: the Prix Galien USA (2008, Best Biotechnology Product) and France (2009, Rare Disease Treatment).

For more information on Soliris, please see full prescribing information for Soliris, including BOXED WARNING regarding risk of serious meningococcal infection, available at [www.soliris.net](http://www.soliris.net).

### **Important Soliris Safety Information**

The U.S. prescribing information for Soliris includes the following warnings and precautions: Life-threatening and fatal meningococcal infections have occurred in patients treated with Soliris. Meningococcal infection may become rapidly life-threatening or fatal if not recognized and treated early. Comply with the most current Centers for Disease Control (CDC)'s Advisory Committee on Immunization Practices (ACIP) recommendations for meningococcal vaccination in patients with complement deficiencies. Immunize patients with meningococcal vaccines at least two weeks prior to administering the first dose of Soliris, unless the risks of delaying Soliris therapy outweigh the risk of developing a meningococcal infection. Monitor patients for early signs of meningococcal infections and evaluate immediately if infection is suspected. Soliris is available only through a restricted program under a Risk Evaluation and Mitigation Strategy (REMS). Under the Soliris REMS, prescribers must enroll in the program. Enrollment in the Soliris REMS program and additional information are available by telephone: 1-888-SOLIRIS (1-888-765-4747) or at [www.solirisrems.com](http://www.solirisrems.com).

Patients may have increased susceptibility to infections, especially with encapsulated bacteria. Aspergillus infections have occurred in immunocompromised and neutropenic patients. Children treated with Soliris may be at increased risk of developing serious infections due to *Streptococcus pneumoniae* and *Haemophilus influenzae* type b (Hib). Soliris treatment of patients with PNH should not alter anticoagulant management because the effect of withdrawal of anticoagulant therapy during Soliris treatment has not been established. Administration of Soliris may result in infusion reactions, including anaphylaxis or other hypersensitivity reactions.

In patients with PNH, the most frequently reported adverse events observed with Soliris treatment in clinical studies were headache, nasopharyngitis, back pain and nausea. In patients with aHUS, the most frequently reported adverse events observed with Soliris treatment in clinical studies were headache, diarrhea, hypertension, upper respiratory infection,

abdominal pain, vomiting, nasopharyngitis, anemia, cough, peripheral edema, nausea, urinary tract infections, and pyrexia. In patients with gMG who are anti-AchR antibody-positive, the most frequently reported adverse reaction observed with Soliris treatment in the placebo-controlled clinical study ( $\geq 10\%$ ) was musculoskeletal pain.

## About Alexion

Alexion is a global biopharmaceutical company focused on serving patients and families affected by rare diseases through the innovation, development and commercialization of life-changing therapies. 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), atypical hemolytic uremic syndrome (aHUS), and anti-acetylcholine receptor (AchR) antibody-positive generalized myasthenia gravis (gMG). In addition, Alexion has two highly innovative enzyme replacement therapies for patients with life-threatening and ultra-rare metabolic disorders, hypophosphatasia (HPP) and lysosomal acid lipase deficiency (LAL-D). As the leader in complement biology for over 20 years, Alexion focuses its research efforts on novel molecules and targets in the complement cascade, and its development efforts on the core therapeutic areas of hematology, nephrology, neurology, and metabolic disorders. This press release and further information about Alexion can be found at: [www.alexion.com](http://www.alexion.com).

[ALXN-G]

## Forward-Looking Statement

This press release contains forward-looking statements, including statements related to the potential medical benefits of ALXN1210 for the treatment of PNH. 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 the adequacy of our research, 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, 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, the possibility that current rates of adoption of Soliris in PNH, aHUS or other diseases are not sustained, the possibility that clinical trials of our product candidates could be delayed, 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, uncertainties surrounding legal proceedings, company investigations and government investigations, including investigations of Alexion by the U.S. Securities and Exchange Commission (SEC) and U.S. Department of Justice, the risk that anticipated regulatory filings are delayed, the risk that estimates regarding the number of patients with PNH, aHUS, gMG, HPP and LAL-D are inaccurate, the risks of changing foreign exchange rates, risks relating to the potential effects of the Company's restructuring and relocation of its corporate headquarters, and a variety of other risks set forth from time to time in Alexion's filings with the SEC, including but not limited to the risks discussed in Alexion's Quarterly Report on Form 10-Q for the period ended September 30, 2017 and in our other filings with the SEC. 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

- 
- <sup>1</sup> Hill A, Richards SJ, Hillmen P. Recent developments in the understanding and management of paroxysmal nocturnal haemoglobinuria. *Br J Haematol*. 2007 May;137(3):181-92.
  - <sup>2</sup> Hillmen P, Lewis SM, Bessler M, et al. Natural history of paroxysmal nocturnal hemoglobinuria. *N Engl J Med*. 1995 Nov 9;333(19):1253-8.
  - <sup>3</sup> Socié G, Mary JY, de Gramont A, et al. Paroxysmal nocturnal haemoglobinuria: long-term follow-up and prognostic factors. *Lancet*. 1996;348:573-577.
  - <sup>4</sup> Rosse W. Paroxysmal Nocturnal Hemoglobinuria. Hoffman: Hematology: Basic Principles and Practice. 3rd ed. Churchill Livingstone, Inc.; 2000: 331-342.
  - <sup>5</sup> Nishimura J, Kanakura Y, Ware RE, et al. Clinical course and flow cytometric analysis of paroxysmal nocturnal hemoglobinuria in the United States and Japan. *Medicine (Baltimore)* 2004 May;83(3):193-207.
  - <sup>6</sup> Weitz I, Meyers G, Lamy T, et al. Cross-sectional validation study of patient-reported outcomes in patients with paroxysmal

nocturnal haemoglobinuria. *Intern Med J.* 2013;43:298-307.

<sup>7</sup> Parker C, Omine M, Richards S, et al. Diagnosis and management of paroxysmal nocturnal hemoglobinuria. *Blood.* 2005 Dec 1;106(12):3699-709.

<sup>8</sup> Hillmen P, Muus P, Duhrsen U, et al. Effect of the complement inhibitor eculizumab on thromboembolism in patients with paroxysmal nocturnal hemoglobinuria. *Blood.* 2007 Dec 1;110(12):4123-8.

<sup>9</sup> Wang H, Chuhjo T, Yasue S, et al. Clinical significance of a minor population of paroxysmal nocturnal hemoglobinuria-type cells in bone marrow failure syndrome. *Blood.* 2002;100 (12):3897-3902.

<sup>10</sup> Iwanga M, Furukawa K, Amenomori T, et al. Paroxysmal nocturnal haemoglobinuria clones in patients with myelodysplastic syndromes. *Br J Haematol.* 1998;102(2):465-474.

<sup>11</sup> Maciejewski JP, Rivera C, Kook H, et al. Relationship between bone marrow failure syndromes and the presence of glycosylphosphatidylinositol-anchored protein-deficient clones. *Br J Haematol.* 2001;115:1015-1022.

<sup>12</sup> Hill A, Kelly RJ, Hillmen P. Thrombosis in paroxysmal nocturnal hemoglobinuria. *Blood.* 2013;121:4985-4996.

<sup>13</sup> Sahelijo L, Mujeebuddin A, Mitchell D, et al. First in human single-ascending dose study: safety, biomarker, pharmacokinetics and exposure-response relationships of ALXN1210, a humanized monoclonal antibody to C5, with marked half-life extension and potential for significantly longer dosing intervals. *Blood.* 2015;126 (23):4777.

View source version on [businesswire.com](http://www.businesswire.com/news/home/20171101006036/en/): <http://www.businesswire.com/news/home/20171101006036/en/>

Alexion Pharmaceuticals, Inc.

Media

Arne Naeveke, PhD, 475-230-3774

Executive Director, Product Communications

or

Investors

Elena Ridloff, CFA, 475-230-3601

Vice President, Investor Relations

or

Catherine Hu, 475-230-3599

Director, Investor Relations

Source: Alexion Pharmaceuticals, Inc.

News Provided by Acquire Media