



## Alexion Announces Upcoming Data Presentations at American Society of Hematology Annual Meeting

November 1, 2018

- Nine abstracts accepted, including three oral presentations of ALXN1210 Phase 3 PNH data -

BOSTON--(BUSINESS WIRE)--Nov. 1, 2018-- [Alexion Pharmaceuticals, Inc.](#) (NASDAQ:ALXN) today announced that nine abstracts from its complement research and development program have been accepted for presentation at the American Society of Hematology (ASH) Annual Meeting in San Diego, December 1 to 4, 2018. Key data will include both new analyses and previously announced results from the two Phase 3 studies of ALXN1210, the Company's investigational long-acting C5 complement inhibitor, in patients with paroxysmal nocturnal hemoglobinuria (PNH), in addition to further data on Soliris® (eculizumab) for the treatment of PNH and atypical hemolytic uremic syndrome (aHUS). Collectively, the breadth of the data to be presented at ASH demonstrates continued progress extending the company's leadership in understanding and treating rare complement-mediated diseases.

The accepted abstracts are listed below and are now available on the ASH website.

### ALXN1210

[A Phase 3 Study of Ravulizumab \(ALXN1210\) versus Eculizumab in Adults with Paroxysmal Nocturnal Hemoglobinuria Naive to Complement Inhibitors: Results of a Subgroup Analysis with Patients Stratified by Baseline Hemolysis Level, Transfusion History, and Demographics.](#) Abstract ID#: 110623 – Oral Presentation, December 3, 2018, 11:00-11:15 a.m. PST, Grand Hall C.

[Results from a Phase 3, Multicenter, Non-Inferiority Study of Ravulizumab \(ALXN1210\) Versus Eculizumab in Adult Patients with Paroxysmal Nocturnal Hemoglobinuria Currently Treated with Eculizumab.](#) Abstract ID#: 119147 – Oral Presentation, December 3, 10:30-10:45 a.m. PST, Grand Hall C.

[Ravulizumab \(ALXN1210\) versus Eculizumab in Adults with Paroxysmal Nocturnal Hemoglobinuria: Pharmacokinetics and Pharmacodynamics Observed in Two Phase 3 Randomized, Multicenter Studies.](#) Abstract ID#: 110858 – Oral Presentation, December 3, 10:45-11:00 a.m. PST, Grand Hall C.

[A Prospective Analysis of Breakthrough Hemolysis in 2 Phase 3 Randomized Studies of Ravulizumab \(ALXN1210\) versus Eculizumab in Adults with Paroxysmal Nocturnal Hemoglobinuria.](#) Abstract ID#: 110874 – Poster Presentation, December 2, 6:00-8:00 p.m. PST, Hall GH.

### Soliris® (eculizumab)

[Efficacy of Eculizumab in Pediatric Patients with Paroxysmal Nocturnal Hemoglobinuria in the International PNH Registry.](#) Abstract ID#: 111306 – Poster Presentation, December 3, 6:00-8:00 p.m. PST, Hall GH.

[Economic Benefit of Early In-hospital Diagnosis and Treatment Initiation of Eculizumab in aHUS.](#) Abstract ID#: 112893 – Poster Presentation, December 2, 6:00-8:00 p.m. PST, Hall GH.

### PNH

[Prognostic Value of Clone Size in Paroxysmal Nocturnal Hemoglobinuria \(PNH\) for Thrombotic Events in Untreated Patients in the International PNH Registry.](#) Abstract ID#: 111324 – Poster Presentation, December 1, 6:15-8:15 p.m. PST, Hall GH.

**Baseline Characteristics of Patients with Paroxysmal Nocturnal Hemoglobinuria Identified in the Department of Defense Database.** Abstract ID#: 113478 – online.

**The Value of Population Based Data to Study Rare Diseases: An Example Using the Department of Defense Healthcare System.** Abstract ID#: 113497 – online.

### 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>4</sup>

### About ALXN1210

ALXN1210 is an innovative, investigational, long-acting C5 inhibitor discovered and developed by Alexion that works by inhibiting the C5 protein in the

terminal complement cascade, a part of the body's immune system that, when activated in an uncontrolled manner, plays a role in severe ultra-rare disorders like paroxysmal nocturnal hemoglobinuria (PNH), atypical hemolytic uremic syndrome (aHUS), and anti-acetylcholine receptor (AChR) antibody-positive myasthenia gravis (MG). In Phase 3 clinical studies in complement inhibitor-naïve patients with PNH, and patients with PNH who had been stable on Soliris®, intravenous treatment with ALXN1210 every eight weeks demonstrated non-inferiority to intravenous treatment with Soliris® every two weeks, with numeric results for all primary and key secondary endpoints favoring ALXN1210. ALXN1210 is also currently being evaluated in a Phase 3 clinical study in complement inhibitor-naïve patients with 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 and aHUS.

ALXN1210 has received Orphan Drug Designation (ODD) for the treatment of patients with PNH in the U.S., EU, and Japan, 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 C5 protein in the terminal part of the complement cascade, a part of the immune system that, when activated in an uncontrolled manner, plays a role in severe rare and 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 generalized MG (gMG) in adults who are anti-AChR antibody-positive, in the U.S. for the treatment of adult patients with gMG who are anti-AChR antibody-positive, and in Japan for the treatment of patients with gMG who are AChR antibody-positive and whose symptoms are difficult to control with high-dose intravenous immunoglobulin (IVIG) therapy or plasmapheresis (PLEX). 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, for the treatment of patients with refractory gMG in Japan, and for the treatment of patients with neuromyelitis optica spectrum disorder (NMOSD) in the U.S., EU, and 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 (≥10%) was musculoskeletal pain.

### **About Alexion**

Alexion is a global biopharmaceutical company focused on serving patients and families affected by rare diseases through the discovery, development and commercialization of life-changing therapies. As the global leader in complement biology and inhibition for more than 20 years, Alexion 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). Alexion also 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). In addition, the company is developing two late-stage therapies, a second complement inhibitor and a copper-binding agent for Wilson disease. 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. Alexion has been named to the Forbes list of the World's Most Innovative Companies seven years in a row and is headquartered in Boston, Massachusetts' Innovation District. The company also has offices around the globe and serves patients in more than 50 countries. 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" within the meaning of the Private Securities Litigation Reform Act of 1995 that involve risks and uncertainties relating to future events and the future performance of Alexion, including statements related to: the Company extending its leadership in understanding and treating rare complement-mediated diseases; future plans to initiate a clinical studies of ALXN1210 delivered

subcutaneously once per week as a potential treatment for patients with PNH and for studies of ALXN1210 for other indications; and the potential medical benefits of ALXN1210 for the treatment of PNH and other diseases. Forward-looking statements are subject to factors that may cause Alexion's results and plans to differ materially from those expected by these forward looking statements, including for example: our dependence on sales from our principal product (Soliris®); future competition from biosimilars and other products; decisions of regulatory authorities regarding the adequacy of our research, marketing approval or material limitations on the marketing of our products; delays or failure of product candidates to obtain regulatory approval; delays or the inability to launch product candidates due to regulatory restrictions, anticipated expense or other matters; 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; results in early stage clinical trials may not be indicative of full results or results from later stage or larger clinical trials (or broader patient populations) and do not ensure regulatory approval; the possibility that results of clinical trials are not predictive of safety and efficacy and potency of our products (or we fail to adequately operate or manage our clinical trials) which could cause us to halt trials, delay or prevent us from making regulatory approval filings or result in denial of approval of our product candidates; unexpected delays in clinical trials; future product improvements may not be realized due to expense or feasibility; uncertainty of long-term success in developing, licensing or acquiring other product candidates or additional indications for existing products; inability to complete planned acquisitions due to failure of regulatory approval or material changes in the target or otherwise; inability to complete acquisitions and investments due to increased competition for technology; the possibility that current rates of adoption of Soliris® in PNH, aHUS, gMG or other diseases are not sustained; the adequacy of our pharmacovigilance and drug safety reporting processes; failure to protect and enforce our data, intellectual property and proprietary rights and the risks and uncertainties relating to intellectual property claims and challenges against us; the risk that third party payers (including governmental agencies) will not reimburse or continue to reimburse for the use of our products at acceptable rates or at all; failure to realize the benefits and potential of investments, collaborations, licenses and acquisitions; the possibility that expected tax benefits will not be realized; assessment of impact of recent accounting pronouncements; potential declines in sovereign credit ratings or sovereign defaults in countries where we sell our products; delay of collection or reduction in reimbursement due to adverse economic conditions or changes in government and private insurer regulations and approaches to reimbursement; 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 estimates regarding the number of patients with PNH, aHUS, gMG, HPP and LAL-D and other future indications we are pursuing are inaccurate; the risks of changing foreign exchange rates; risks relating to the potential effects of the Company's restructuring; risks related to the acquisition of Syntimmune and other companies and co-development efforts; 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, 2018 and in our other filings with the SEC. Alexion disclaims any obligation 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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