Alexion Completes Acquisition of Syntimmune

November 2, 2018

- Acquisition adds clinical-stage anti-FcRn antibody SYNT001 to company's growing pipeline -

- SYNT001 is first and only anti-FcRn asset currently in clinical development for warm autoimmune hemolytic anemia (WAIHA) -

BOSTON--(BUSINESS WIRE)--Nov. 2, 2018-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today announced that the previously announced acquisition of Syntimmune has been successfully completed. The acquisition adds to the company's growing pipeline with the addition of clinical-stage SYNT001, a humanized monoclonal antibody that inhibits the interaction of neonatal Fc receptor (FcRn) with Immunoglobulin G (IgG) and IgG immune complexes with the potential to treat a number of rare IgG-mediated diseases.

"We've made significant progress rebuilding our pipeline this year, and the acquisition of Syntimmune is another critical step in continuing to expand and diversify our portfolio," said Ludwig Hantson, Ph.D., Chief Executive Officer of Alexion. "We're very excited to continue the development of SYNT001, which we believe holds great promise in transforming patient care across a number of rare IgG-mediated diseases. We are rapidly advancing the current development programs and look forward to beginning two pivotal programs next year, including one in warm autoimmune hemolytic anemia."

SYNT001 is currently being evaluated in Phase 1b/2a studies in patients with warm autoimmune hemolytic anemia (WAIHA) and in patients with pemphigus vulgaris (PV) or pemphigus foliaceus (PF) and has demonstrated proof of mechanism showing rapid IgG reduction. Alexion plans to initiate two pivotal trials in 2019 – one in WAIHA, following successful completion of the current Phase 1b/2a study, and one in an undisclosed indication.

About FcRn

Antibodies play an important role in a healthy body's defense by fighting infections from bacteria and other invaders. In autoimmune diseases, however, the body mistakenly attacks itself through the production of pathogenic (disease-causing) antibodies of the Immunoglobulin G (IgG) subtype. Neonatal Fc receptor (FcRn) rescues IgGs from lysosomal degradation by binding them to endosomes and returning them to the bloodstream. This helps prolong the half-life of IgG. In healthy individuals, this function contributes to a normal immune response. In many autoimmune conditions, however, FcRn prevents lysosomal degradation of pathogenic IgGs associated with driving the disease. Therefore, blocking the FcRn-IgG interaction has the potential to drive degradation of IgG within cells and rapidly reduce circulating pathogenic IgG.

About WAIHA

Warm autoimmune hemolytic anemia (WAIHA) is a rare autoimmune disorder caused by pathogenic Immunoglobulin G (IgG) antibodies that react with and cause the premature destruction of red blood cells at normal body temperature. The disease is often characterized by profound, and potentially life-threatening anemia and other acute complications, including severe and life-threatening hemolysis, severe weakness, enlarged spleen and/or liver, rapid heart rate (tachycardia), chest pain, heart failure and fainting (syncope). There are approximately 65,000 patients across the United States, France, Germany, Italy, Spain and the United Kingdom. There are currently no approved treatments for WAIHA.

About SYNT001

SYNT001 is an investigational humanized IgG4 monoclonal antibody optimized to inhibit FcRn binding to IgG at both neutral and acidic pH. Studies have shown that SYNT001 rapidly facilitates clearance of IgG and IgG circulating immune complexes (CICs), with the potential to block innate immune responses induced by IgG and CIC, as well as inhibit T-cell and B-cell activation in response to CIC. Additionally, studies suggest that SYNT001 accomplishes its effects on IgG without destroying immune cells or impacting other types of immunoglobulin. SYNT001 has the potential to exert a rapid therapeutic effect in a wide range of IgG-mediated autoimmune diseases.

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), and is also developing it for patients with neuromyelitis optica spectrum disorder (NMOSD). 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 several mid-to-late-stage therapies, including a second complement inhibitor, a copper-binding agent for Wilson disease and an anti-neonatal Fc receptor (FcRn) antibody for rare Immunoglobulin G (IgG)-mediated diseases. 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.

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Forward-Looking Statement

This press release contains forward-looking statements for purposes of the Private Securities Litigation Reform Act of 1995, including statements related to: the benefits of SYNT001, the potential of SYNT001 to treat a number of rare IgG-mediated diseases, the potential benefits of targeting

FcRn, SYNT001 holds great promise in transforming patient care across a number of rare IgG-mediated diseases, the acquisition of Syntimmune is a critical step in expanding and diversifying Alexion's portfolio, Syntimmune provides the opportunity to transform patient care in diseases like WAIHA, Alexion's clinical development plans, and the potential benefits of the transaction. Forward-looking statements are subject to factors that may cause Alexion's results and plans to differ materially from those forward-looking statements, including for example, the technology acquired from Syntimmune may not confer the expected therapeutic benefits (particularly with respect to treatment of IgG-mediated diseases), future clinical trials of SYNT001 may not prove that the therapy is safe and effective to the level required by regulators, decisions of regulatory authorities regarding the adequacy of our and Syntimmune's research and clinical tests, marketing approval or material limitations on the marketing of products, delays, 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, the possibility that results of clinical trials are not predictive of safety and efficacy results of products in broader patient populations, the possibility that clinical trials of our product candidates could be delayed or terminated prior to completion, the adequacy of our pharmacovigilance and drug safety reporting processes, 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 anticipated regulatory filings are delayed, the risk that estimates regarding the number of patients with IgG-mediated diseases (including WAIHA) are inaccurate, risks related to the acquisition of Syntimmune and other acquisitions 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.

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