



Alexion to Acquire Syntimmune

September 26, 2018

- Brings in clinical-stage anti-FcRn antibody SYNT001 with potential to address a number of rare IgG-mediated diseases -
- SYNT001 is first and only anti-FcRn asset currently in clinical development for warm autoimmune hemolytic anemia (WAIHA) -
- Conference call and webcast scheduled for today, September 26, 2018, at 8:00 a.m. EDT -

BOSTON--(BUSINESS WIRE)--Sep. 26, 2018-- [Alexion Pharmaceuticals, Inc.](#) (NASDAQ:ALXN) and Syntimmune today announced that they have entered into a definitive agreement for Alexion to acquire Syntimmune, a clinical-stage biotechnology company developing antibody therapeutics targeting the neonatal Fc receptor (FcRn). SYNT001 – a humanized monoclonal antibody that inhibits the interaction of FcRn with Immunoglobulin G (IgG) and IgG immune complexes – has the potential to improve treatment in a number of rare IgG-mediated diseases. 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. Under the terms of the agreement, Alexion will acquire Syntimmune for an upfront payment of \$400 million, with the potential for additional milestone-dependent payments of up to \$800 million, for a total value of up to \$1.2 billion.

This press release features multimedia. View the full release here: <https://www.businesswire.com/news/home/20180926005248/en/>

“Targeting FcRn holds great promise in transforming the treatment of IgG-mediated diseases. SYNT001 has successfully demonstrated proof of mechanism – the ability to rapidly lower IgG levels – in early clinical studies and has the potential to treat a number of rare IgG-mediated diseases,” said Ludwig Hantson, Ph.D., Chief Executive Officer of Alexion. “The acquisition of Syntimmune represents a critical step in rebuilding Alexion’s pipeline and further diversifying the company’s clinical-stage rare disease portfolio. It offers a strong strategic fit with Alexion’s existing rare disease franchises and provides the opportunity to transform patient care in diseases like warm autoimmune hemolytic anemia, where SYNT001 is the first, and currently the only, anti-FcRn therapy in clinical development.”

“Since the company’s founding in 2013, the team at Syntimmune has been focused on developing transformative therapies for patients with autoimmune diseases. We see tremendous promise for SYNT001, which is being evaluated in multiple IgG-mediated autoimmune diseases in ongoing clinical trials,” said Seth Harrison, M.D., Chairman of Syntimmune and Managing Partner of Apple Tree Partners. “Alexion’s demonstrated rare disease expertise and development and commercial capabilities provide an ideal foundation for continued advancement of SYNT001 and, we believe, will ensure its broad potential is realized.”

Terms of the Transaction

Alexion’s acquisition of Syntimmune is subject to the satisfaction of customary closing conditions, including approval from relevant regulatory agencies. Pending these approvals, the transaction is expected to close in the fourth quarter of 2018. Alexion intends to finance the acquisition through cash on hand.

Foley Hoag LLP is serving as legal counsel to Alexion, Goodwin Procter LLP is serving as legal counsel to Syntimmune, and Sullivan & Cromwell LLP is serving as legal counsel for Apple Tree Partners, which is the majority shareholder in Syntimmune.

Conference Call

Alexion will host a conference call/webcast today, September 26, 2018 at 8:00 a.m. EDT to discuss the acquisition. To participate in this call, dial (866) 762-3111 (USA) or (210) 874-7712 (International), passcode 7449227, shortly before 8:00 a.m. EDT. A replay of the call will be available for a limited period of time following the call. The audio webcast can be accessed on the Investors page of Alexion’s website at: <http://ir.alexion.com>.

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 Syntimmune

Syntimmune is a clinical-stage biotechnology company developing differentiated drug candidates in a wide range of autoimmune diseases. Drawing on the pioneering research of its scientific founders, the company is advancing novel therapies based on its deep expertise in the biology of the neonatal Fc receptor (FcRn) and its complex role in the pathogenesis of IgG-mediated autoimmune diseases. Syntimmune's lead candidate, SYNT001, is a monoclonal antibody that specifically blocks FcRn-IgG interactions and is being studied in multiple Phase 1b/2a trials for the treatment of IgG-mediated autoimmune diseases. Syntimmune is headquartered in Boston, Mass., and was founded in 2013 by Richard Blumberg, M.D., and Laurence Blumberg, M.D. Syntimmune has raised \$78 million in private financing from lead investor Apple Tree Partners, with participation from additional investors Partners Innovation Fund, FMB Research and AFB Fund. For more information on Syntimmune, please visit the company's website at www.syntimmune.com.

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, including 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.

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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 improve the treatment paradigm in a number of rare IgG-mediated diseases, targeting FcRn holds great promise in transforming the IgG treatment landscape, SYNT001 has the potential to treat a number of rare IgG-mediated diseases, the planned acquisition of Syntimmune is a critical step in rebuilding Alexion's pipeline and further diversifying the company's clinical-stage rare disease portfolio, Syntimmune provides the opportunity to transform patient care in diseases like WAIHA, all necessary approvals necessary to complete the acquisition of Syntimmune will be obtained and obtained in a timely manner (including the necessary regulatory approvals), the acquisition of Syntimmune is expected to close in the fourth quarter of 2018, SYNT001 has the potential to exert a rapid therapeutic effect in a wide range of IgG-mediated autoimmune diseases, 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, delay by regulatory authorities to approve transaction (or a decision not to approve the transaction), the closing conditions to complete the acquisition may not be satisfied, 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 PNH, aHUS, gMG, HPP, IgG-mediated autoimmune diseases (including WAIHA) and LAL-D 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 June 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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