



Alexion to Acquire Achillion

October 16, 2019

- Adds clinical-stage portfolio of oral small molecule Factor D inhibitors to Alexion's pipeline -

- Provides opportunity to enhance treatment for PNH patients experiencing extravascular hemolysis (EVH), potential first-in-class C3 glomerulopathy (C3G) therapy & promising development platform for Factor D inhibition in additional alternative pathway complement-mediated rare diseases -

- Initial all-cash transaction for \$6.30 per share; total transaction of up to \$8.30 per share with potential additional contingent considerations -

- Conference call and webcast scheduled for today, October 16, 2019, at 8:00 a.m. EDT -

BOSTON & BLUE BELL, Pa.--(BUSINESS WIRE)--Oct. 16, 2019-- [Alexion Pharmaceuticals, Inc.](#) (NASDAQ:ALXN) and [Achillion Pharmaceuticals, Inc.](#) (NASDAQ:ACHN) today announced that they have entered into a definitive agreement for Alexion to acquire Achillion, a clinical-stage biopharmaceutical company focused on the development of oral small molecule Factor D inhibitors to treat people with complement alternative pathway-mediated rare diseases, such as paroxysmal nocturnal hemoglobinuria (PNH) and C3 glomerulopathy (C3G). Achillion currently has two clinical-stage medicines in development, including danicopan (ACH-4471) in Phase 2 and ACH-5228 in Phase 1.

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"Alexion has demonstrated the transformative impact that inhibiting C5 can have on multiple rare and devastating diseases. However, we believe this is just the beginning of what's possible with complement inhibition," said Ludwig Hantson, Ph.D., Chief Executive Officer of Alexion. "Targeting a different part of the complement system – the alternative pathway – by inhibiting Factor D production addresses uncontrolled complement activation further upstream in the complement cascade, and importantly, leaves the rest of the complement system intact, which is critical in maintaining the body's ability to fight infection. We believe this approach has the opportunity to help patients with diseases not currently addressed through C5 inhibition. We look forward to applying our nearly three decades of complement and development expertise to unlock the potential of oral Factor D inhibitors and bring these benefits to patients."

"We have established great momentum – discovering and advancing several small molecules into clinical development that have the potential to treat immune-related diseases associated with the alternative pathway of the complement system," said Joe Truitt, President and Chief Executive Officer at Achillion. "Having already demonstrated proof-of-concept and proof-of-mechanism with our lead candidate, danicopan (ACH-4471), in PNH and C3G, respectively, we believe there is significant opportunity for Factor D inhibition in the treatment of other diseases as well. Alexion is an established leader in developing medicines for complement-mediated diseases, and we look forward to working together to accelerate our objective of bringing novel therapies to patients as quickly as possible and ensuring that the broad promise of this approach is fully realized. We thank our employees, investigators and partners for their incredible work and commitment."

Transaction Details

The initial consideration of approximately \$930 million, or \$6.30 per share of Achillion common stock, will be funded with cash on hand. As part of the acquisition, Alexion will also be acquiring the cash currently on Achillion's balance sheet. As of September 30, 2019, this was approximately \$230 million; the actual amount will be determined as of the transaction close. The transaction includes the potential for additional consideration in the form of non-tradeable contingent value rights (CVRs), which will be paid to Achillion shareholders if certain clinical and regulatory milestones are achieved within specified periods. These include \$1.00 per share for the U.S. FDA approval of danicopan and \$1.00 per share for ACH-5228 Phase 3 initiation.

Alexion's acquisition of Achillion is subject to the approval of Achillion shareholders and satisfaction of customary closing conditions and approval from relevant regulatory agencies, including clearance under the Hart-Scott Rodino Antitrust Improvements Act. Pending these approvals, the transaction is expected to close in the first half of 2020.

Conference Call

Alexion will host a conference call and webcast today, October 16, 2019, 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 5426458, 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 Factor D

Factor D is an essential serine protease and critical control point in the alternative pathway (AP) of the complement system, a part of the innate immune system. Achillion's complement platform is focused on advancing oral small molecules that inhibit the AP and can potentially be used in the treatment of immune-related diseases in which complement AP plays a critical role. Potential indications currently being evaluated for these compounds include PNH, C3G and immune complex-mediated membranoproliferative glomerulonephritis (IC-MPGN).

About Paroxysmal Nocturnal Hemoglobinuria (PNH)

PNH is a chronic, progressive, debilitating and life-threatening ultra-rare blood disorder characterized by hemolysis (destruction of red blood cells) that is mediated by uncontrolled activation of the complement system, a component of the body's immune system. Patients with PNH may experience a wide range of signs and symptoms, such as fatigue, difficulty swallowing, shortness of breath, abdominal pain, erectile dysfunction, dark-colored urine and anemia. The most devastating consequence of chronic hemolysis is thrombosis, which can occur in blood vessels throughout the body, damaging vital organs and causing premature death. PNH is primarily a disease of intravascular hemolysis (IVH), where the red blood cell destruction occurs within the blood vessels. C5 inhibition addresses the complications of IVH and the increases in LDH that cause thrombosis and even death in patients with PNH. However, a small portion of patients – less than 10 percent – receiving a C5 inhibitor continue to experience clinical extravascular hemolysis (EVH), where the red blood cell destruction occurs outside the blood vessels. As a result, these patients are transfusion dependent despite treatment

but do not have bone marrow failure or aplastic anemia. Inhibiting Factor D in the alternative pathway (AP) of the complement system offers the possibility of selectively blocking AP activity and protecting against the destruction of RBCs, while leaving the rest of the complement system intact to fight infection.

About C3 Glomerulopathy (C3G)

C3G is an ultra-rare kidney disease for which there is no approved treatment. The disease is characterized by the deposition of C3 protein fragments in the filtering units (glomeruli) of the kidney, caused by overactivation of the complement alternative pathway (AP). Over time, the chronic deposition of C3 fragments results in permanent kidney damage and kidney failure. Today, C3G patients are treated with steroids and broad-acting immunosuppressants to slow the progression of kidney damage. Oral Factor D inhibitors have demonstrated proof-of-mechanism to interrupt the overactivation of the AP and reduce C3 fragment deposition, providing a potential treatment approach for targeting the underlying cause of C3G.

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 commercialized two approved complement inhibitors to treat patients with paroxysmal nocturnal hemoglobinuria (PNH) as well as the first and only approved complement inhibitor to treat atypical hemolytic uremic syndrome (aHUS), anti-acetylcholine receptor (AChR) antibody-positive generalized myasthenia gravis (gMG) and 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 as well as several early-stage therapies, including one for light chain (AL) amyloidosis and a second anti-FcRn therapy. 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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About Achillion Pharmaceuticals

Achillion is a clinical-stage biopharmaceutical company focused on advancing its oral small molecule complement inhibitors into late-stage development and commercialization. Research has shown that an overactive complement system plays a critical role in multiple disease conditions including the therapeutic areas of nephrology, hematology, ophthalmology and neurology. Achillion is initially focusing its drug development activities on complement-mediated diseases where there are no approved therapies or where existing therapies are inadequate for patients. Potential indications being evaluated for its compounds include paroxysmal nocturnal hemoglobinuria (PNH), C3 glomerulopathy (C3G), and immune complex membranoproliferative glomerulonephritis (IC-MPGN). The company has received Breakthrough Therapy designation for danicopan for treatment in combination with a C5 monoclonal antibody for patients with paroxysmal nocturnal hemoglobinuria (PNH) who are sub-optimal responders to a C5 inhibitor alone. Each of the product candidates in the company's oral small molecule portfolio was discovered in its laboratories and is wholly owned. To advance its investigational product candidates into Phase 3 clinical trials and commercialization, the company plans to work closely with key stakeholders including healthcare professionals, patients, regulators and payors. More information is available at <http://www.achillion.com>.

[ACHN-G]

Forward-Looking Statements

This press release includes forward-looking statements related to the proposed acquisition of Achillion by Alexion, including: the acquisition provides an opportunity to enhance treatment for PNH patients experiencing extravascular hemolysis (EVH); therapeutic benefits of Achillion products, including potential first-in-class C3 glomerulopathy (C3G) therapy and promising development platform for Factor D inhibition in additional alternative pathway complement-mediated rare diseases; Alexion believes its work on C5 complement inhibition is just the beginning of what's possible with complement inhibition; Alexion believes inhibiting a different part of the complement pathway has the opportunity to help patients with diseases not currently addressed through C5 inhibition; using its complement inhibitor platform, Achillion has several small molecules in clinical development that have the potential to treat immune-related diseases associated with the alternative pathway of the complement system; Alexion believes there is significant opportunity for Factor D inhibition in the treatment of other diseases as well; Alexion will ensure the broad promise of Achillion's approach is fully realized; the anticipated closing date of the acquisition; and Achillion's complement platform is focused on advancing oral small molecules that inhibit the AP and can potentially be used in the treatment of immune-related diseases in which complement AP plays a critical role. A number of important factors could cause actual results to differ materially from those indicated by such forward-looking statements, including: the risk that the proposed acquisition of Achillion by Alexion may not be completed; the failure to receive the required stockholder approval necessary to complete the acquisition; the failure (or delay) to receive the required regulatory approvals of the proposed acquisition; the failure of the closing conditions set forth in the acquisition agreement to be satisfied (or waived); the anticipated benefits of the Achillion platform and therapies not being realized; future clinical trials of Achillion products not proving that the therapies are safe and effective to the level required by regulators; decisions of regulatory authorities regarding the adequacy of the research and clinical tests, marketing approval or material limitations on the marketing of Achillion products; delays or failure of product candidates to obtain regulatory approval; delays or the inability to launch product candidates due to regulatory restrictions; unanticipated expenses; interruptions or failures in the manufacture and supply of products and 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 product candidates could be delayed or terminated prior to completion for a number of reasons; the adequacy of pharmacovigilance and drug safety reporting processes; and a variety of other risks set forth from time to time in Alexion's or Achillion'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, 2019 and in its other filings with the SEC and the risks discussed in Achillion's Quarterly Report on Form 10-Q for the period ended June 30, 2019 and in its other filings with the SEC. Alexion and Achillion disclaim 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.

Additional Information about the Proposed Transaction and Where to Find It

In connection with the proposed transaction, Achillion will file a proxy statement on Schedule 14A with the SEC. Additionally, Achillion plans to file other relevant materials with the SEC in connection with the proposed transaction. This material is not a substitute for the proxy statement or any other document which Achillion may file with the SEC. The definitive proxy statement will be sent or given to the stockholders of Achillion and will contain

important information about the proposed transaction and related matters. INVESTORS IN AND SECURITY HOLDERS OF ACHILLION ARE URGED TO READ THE PROXY STATEMENT AND ANY OTHER RELEVANT DOCUMENTS THAT ARE FILED OR FURNISHED OR WILL BE FILED OR WILL BE FURNISHED WITH THE SEC, AS WELL AS ANY AMENDMENTS OR SUPPLEMENTS TO THESE DOCUMENTS, CAREFULLY AND IN THEIR ENTIRETY BEFORE MAKING ANY VOTING OR INVESTMENT DECISION WITH RESPECT TO THE PROPOSED TRANSACTION BECAUSE THEY CONTAIN OR WILL CONTAIN IMPORTANT INFORMATION ABOUT THE TRANSACTION, RELATED MATTERS AND THE PARTIES TO THE TRANSACTION. The materials to be filed by Achillion with the SEC may be obtained free of charge at the SEC's website at www.sec.gov or in the "Investors & News" section of Achillion's website at www.achillion.com.

Centerview Partners served as Achillion's exclusive financial advisor, while Skadden, Arps, Slate, Meagher & Flom LLP served as its legal advisor.

Participants in the Solicitation

This communication does not constitute a solicitation of a proxy from any stockholder with respect to the proposed transaction. However, Alexion, Achillion and their respective directors and executive officers may be considered participants in the solicitation of proxies in connection with the proposed transaction. Information about the directors and executive officers of Alexion is set forth in its Annual Report on Form 10-K for the year ended December 31, 2018, which was filed with the SEC on February 6, 2019, and its proxy statement for its May 14, 2019 annual meeting of stockholders, which was filed with the SEC on March 26, 2019. Information about the directors and executive officers of Achillion is set forth in its Annual Report on Form 10-K for the year ended December 31, 2018, which was filed with the SEC on March 7, 2019, and its proxy statement for its May 30, 2019 annual meeting of stockholders, which was filed with the SEC on April 15, 2019. Additional information concerning the interests of the participants in the solicitation, which may, in some cases, be different than those of Achillion's stockholders generally, will be set forth in the proxy statement relating to the transaction when it becomes available. You may obtain these documents (when they become available) free of charge through the website maintained by the SEC at <http://www.sec.gov>. Copies of the documents filed with the SEC by Alexion will be available free of charge on Alexion's internet website at <http://www.alexion.com> under the tab, "Investors" and under the heading "SEC Filings" or by contacting Alexion's Investor Relations Department at investorrelations@alexion.com. Copies of the documents filed with the SEC by Achillion will be available free of charge on Achillion's internet website at <http://www.Achillion.com> under the tab "Investors and News" and under the heading "SEC Filings" or by contacting Achillion's Investor Relations Department through <http://ir.achillion.com/contact-us>.

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