



Alexion Completes Acquisition of Achillion

January 28, 2020

– Acquisition adds two clinical-stage Factor D inhibitors to Alexion’s pipeline and provides promising development platform for additional complement-mediated diseases –

BOSTON--(BUSINESS WIRE)--Jan. 28, 2020-- [Alexion Pharmaceuticals, Inc.](#) (NASDAQ:ALXN) today announced it has completed its acquisition of Achillion Pharmaceuticals, Inc. The acquisition adds two clinical-stage oral small molecule Factor D inhibitors to Alexion’s pipeline and provides the foundation and expertise for a broader oral Factor D inhibition development platform with the potential to treat numerous additional complement-mediated diseases.

“The acquisition of Achillion adds two clinical-stage Factor D inhibitors to our growing pipeline, representing important continued momentum in expanding and diversifying our portfolio and advancing our mission of transforming the lives of people with rare diseases,” said Ludwig Hantson, Ph.D., Chief Executive Officer of Alexion. “We believe oral Factor D inhibition holds great promise in treating people with multiple rare, complement-mediated diseases, providing the opportunity to significantly expand our portfolio into new therapeutic areas and to help many more patients. We look forward to the expertise the Achillion team brings to Alexion, which, combined with our own complement biology and rare disease development and commercialization experience, will enable us to collectively accelerate progress of the development programs. We are committed to maintaining continuity in the programs currently underway and will be moving quickly to advance Achillion’s efforts.”

Alexion will continue development of Achillion’s oral Factor D inhibitor portfolio, which includes two clinical-stage medicines-in-development – danicopan (ACH-4471) and ACH-5228 – as well as multiple compounds in preclinical development. Phase 3 development is being initiated for danicopan as an add-on therapy for PNH patients with extravascular hemolysis (EVH). Danicopan is also in Phase 2 development for C3G, and ACH-5228 is in Phase 2 development for PNH.

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 paroxysmal nocturnal hemoglobinuria (PNH) and C3 glomerulopathy (C3G).

About Paroxysmal Nocturnal Hemoglobinuria (PNH)

Paroxysmal nocturnal hemoglobinuria (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 red blood cells, 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 medicines. As the global leader in complement biology and inhibition for more than 20 years, Alexion has developed and commercializes two approved complement inhibitors to treat patients with paroxysmal nocturnal hemoglobinuria (PNH) and atypical hemolytic uremic syndrome (aHUS), as well as the first and only approved complement inhibitor to treat 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 copper-binding agent for Wilson disease, an anti-neonatal Fc receptor (FcRn) antibody for rare Immunoglobulin G (IgG)-mediated diseases and an oral Factor D inhibitor as well as several early-stage therapies, including one for light chain (AL) amyloidosis, a second anti-FcRn therapy, a second oral Factor D inhibitor and a third complement inhibitor. 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, metabolic disorders and cardiology. Headquartered in Boston, Massachusetts, Alexion 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 includes forward-looking statements related to Alexion's acquisition of Achillion, including: the acquisition provides the foundation for a broader oral Factor D inhibition development platform with the potential to treat numerous additional complement-mediated diseases; the acquisition of Achillion represents important continued momentum in expanding and diversifying our portfolio; oral Factor D inhibition holds great promise in treating multiple rare, complement-mediated diseases, providing the opportunity to significantly expand our portfolio into new therapeutic areas; we are committed to maintaining continuity in the programs currently underway and will be moving quickly to accelerate Achillion's efforts; and Alexion will continue development of Achillion's oral Factor D inhibitor portfolio. A number of important factors could cause actual results to differ materially from those indicated by such forward-looking statements, including: 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 September 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 September 30, 2019 and in its 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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