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Achillion Announces ACH-4471 Receives Positive Opinion for Orphan Drug Designation in the European Union for the Treatment of C3 Glomerulopathy

NEW HAVEN, Conn., Feb. 26, 2018 (GLOBE NEWSWIRE) -- **Achillion Pharmaceuticals, Inc.** (Nasdaq:ACHN), a biopharmaceutical company focused on advancing small molecule factor D inhibitors to modulate the complement alternative pathway, today announced that the European Medicines Agency (EMA) Committee for Orphan Medicinal Products (COMP) issued a positive opinion on ACH-4471 for orphan status in the European Union (EU) for the treatment of C3 glomerulopathy (C3G).

"We are very pleased that ACH-4471, a first-in-class, oral inhibitor of complement factor D, has received a positive orphan status opinion for the treatment of C3G. At Achillion, we continually work to enhance the awareness of C3G, a rare renal disorder consisting of dense deposit disease (C3G) and C3 glomerulonephritis (C3GN) for which there are no approved therapies," commented Milind Deshpande, Ph.D., Chief Executive Officer of Achillion.

Dr. Deshpande further stated, "The mechanism of action for our factor D inhibitors, highlighted by the safety and efficacy data observed to date with ACH-4471, we believe represents a truly novel and targeted approach to potentially treating C3G, a chronic and devastating disease affecting the kidney. By specifically targeting factor D, we believe we are targeting the root cause of disease, which experts attribute to overactivation of the alternative pathway. By inhibiting factor D, we believe ACH-4471 may be able to reduce the excessive formation of C3 fragments in the blood which in C3G are continually being deposited in the kidney leading to impaired function and potentially renal failure."

There are estimated to be more than 4,000 C3G patients in Europe for whom there are no approved therapies.

In the EU, the COMP adopts an opinion on the granting of orphan drug designation, after which the opinion is submitted to the European Commission (EC) for a decision on whether or not to accept the opinion. Orphan drug designation by the EC may provide for regulatory and financial incentives for companies to develop and market therapies that treat a life-threatening or chronically debilitating condition affecting no more than five in 10,000 persons in the EU, and where no satisfactory treatment is available. In addition to a 10-year period of marketing exclusivity in the EU after product approval, orphan drug designation provides incentives for companies seeking protocol assistance from the EMA during the product development phase, and direct access to the centralized authorization procedure.

About C3G

C3G is a devastating disease affecting the kidney for which there is no approved therapy. C3G affects persons of all ages with men and women equally affected. There are estimated to be approximately 4,000 C3G patients in the United States, more than 4,000 in Europe, and greater than 1,000 patients with this disease in Japan. C3G describes a rare renal disease characterized by the presence of C3 protein fragments in the filtering units (glomeruli) of the kidney. These C3 fragment deposits are thought to be the result of overactivation of the complement alternative pathway. The chronic deposition of C3 fragments results in inflammation in the glomeruli (glomerulonephritis) and subsequent permanent renal damage. An estimated 30-50% of C3G patients will require dialysis or a transplant within 10 years of diagnosis.

About the Complement Factor D Platform

Achillion has leveraged its internal discovery capabilities and a novel complement-related drug development platform to develop small molecule factor D inhibitor compounds that target the complement AP. Factor D is an essential serine protease involved in the AP, a part of the innate immune system. Achillion's complement portfolio is focused on seeking to advance small molecule compounds that inhibit factor D and can potentially be used in the treatment of immune-related diseases in which the AP plays a critical role. Potential indications currently being evaluated for these compounds include C3G, immune complex-mediated membranoproliferative glomerulonephritis (IC-MPGN), and paroxysmal nocturnal hemoglobinuria (PNH).

About Achillion Pharmaceuticals

Achillion Pharmaceuticals, Inc. (NASDAQ:ACHN) is a science-driven, patient-focused biopharmaceutical company seeking to leverage its believed strengths across the continuum from discovery through commercialization in its goal of meeting the needs of people with complement-mediated diseases. The company has employed a highly-disciplined discovery and

development approach that has allowed it to develop potent and specific complement factor D inhibitors for AP-mediated diseases. Achillion is rapidly advancing its efforts to bring potentially life-saving medicines to patients with rare diseases. More information is available at http://www.achillion.com.

Cautionary Note Regarding Forward-Looking Statements

This press release includes forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995 that are subject to risks, uncertainties and other important factors that could cause actual results to differ materially from those indicated by such forward-looking statements. Achillion may use words such as "expect," "anticipate," "project," "target," "intend," "plan," "aim," "believe," "seek," "estimate," "can," "could" "focus," "will," "look forward," "goal," and "may" and similar expressions to identify such forward-looking statements. These forward-looking statements also include statements about: the potential for a favorable decision by the EC granting orphan drug designation for ACH-4471; the expected benefits of orphan drug designation for ACH-4471 in the EU; Achillion's expected plans, timing, data readouts and results from ongoing and planned clinical trials of ACH-4471, ACH-5228 and ACH-5548. Among the important factors that could cause actual results to differ materially from those indicated by such forward-looking statements are risks relating to, among other things, Achillion's ability to: advance the preclinical and clinical development of its complement factor D inhibitors under the timelines it projects in current and future preclinical studies and clinical trials; obtain and maintain patent protection for its drug candidates and the freedom to operate under third party intellectual property; demonstrate in any current and future clinical trials the requisite safety, efficacy and combinability of its drug candidates; obtain and maintain necessary regulatory approvals; establish commercial manufacturing arrangements; identify and enter into collaboration agreements with third-parties; compete successfully in the markets in which it seeks to develop and commercialize its product candidates and future products; manage litigation; raise the substantial additional capital needed to achieve its business objectives; and successfully execute on its business strategies. These and other risks are described in the reports filed by Achillion with the U.S. Securities and Exchange Commission, including its Annual Report on Form 10-K for the fiscal year ended December 31, 2017, and any subsequent SEC filings.

In addition, any forward-looking statement in this press release represents Achillion's views only as of the date of this press release and should not be relied upon as representing its views as of any subsequent date. Achillion disclaims any duty to update any forward-looking statement, except as required by applicable law.

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