November 6, 2017

Achillion Announces ACH-4471 Granted Orphan Drug Designation by the FDA and Positive Opinion for Orphan Drug Designation in the European Union for the Treatment of Paroxysmal Nocturnal Hemoglobinuria

NEW HAVEN, Conn., Nov. 06, 2017 (GLOBE NEWSWIRE) -- **Achillion Pharmaceuticals, Inc.** (Nasdaq:ACHN) today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to ACH-4471 for the treatment of paroxysmal nocturnal hemoglobinuria (PNH). Furthermore, Achillion announced today 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 PNH.

"We are very pleased with the receipt of orphan drug designation from the FDA, and a positive opinion by COMP for orphan status, for ACH-4471, a first-in-class, oral inhibitor of complement factor D," commented Milind Deshpande, Ph.D., President and Chief Executive Officer of Achillion. "We believe that modulation of the complement alternative pathway by targeting factor D, could provide a novel and pharmacologically differentiated approach to treating diseases of the complement system, including PNH and C3G."

The FDA Orphan Drug Designation program provides incentives for the development of potentially promising drugs to treat, diagnose or prevent orphan diseases and disorders that affect fewer than 200,000 people in the U.S. This designation may provide, under specified conditions, for a seven-year marketing exclusivity period, as well as certain incentives, including federal grants, tax credits and a waiver of PDUFA filing fees.

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 Paroxysmal Nocturnal Hemoglobinuria

PNH is thought to be caused by a mutation resulting in the absence of receptors normally present on red blood cells (RBCs) that interact with the alternative pathway (AP). The AP of the complement system typically functions normally in these patients but due to the lack of key receptors, known as CD55 and CD59, on the surface of PNH RBCs, the AP treats these cells as foreign and destroys them via hemolysis in the circulatory system (intravascular hemolysis). Factor D is a critical protein of the AP, including the amplification loop of the complement system, and it is believed that inhibiting it could control the AP activity. Furthermore, this mechanism of action represents a potentially distinct and unique therapeutic approach for controlling both intravascular hemolysis, and extravascular hemolysis associated with C5 targeted therapies for PNH.

About the Achillion Alternative Pathway 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 platform 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 paroxysmal nocturnal hemoglobinuria (PNH), C3 glomerulopathy (C3G), immune complex-mediated membranoproliferative glomerulonephritis (IC-MPGN), and geographic atrophy (GA), an advanced form of dry age-related macular degeneration (dry AMD).

About Achillion Pharmaceuticals

Achillion Pharmaceuticals, Inc. (NASDAQ:ACHN) is a science-driven, patient-focused company seeking to leverage its strengths across the continuum from discovery to commercialization in its goal of providing better treatments for people with serious diseases. The company employs a highly-disciplined discovery and development approach that has allowed it to pursue best-in-class oral antiviral therapy for chronic hepatitis C (HCV) and build a platform of potent and specific

complement factor D inhibitors for AP-mediated diseases. Achillion is rapidly advancing its efforts to become a fully-integrated pharmaceutical company with a goal of bringing 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," "may," "potential," and similar expressions to identify such forward-looking statements. These forward-looking statements also include statements about: Achillion's ability to obtain and maintain orphan drug designation for ACH-4771 and the expected benefits of orphan drug designation; the potential benefits of, and potential indications for, Achillion's compounds that inhibit factor D, including ACH-4771; and statements concerning Achillion's strategic goals, efforts, plans, and prospects. 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: successfully obtain orphan drug designation from the EC for ACH-4771; maintain any orphan drug designation once obtained; avail itself of any of the benefits of orphan drug designation for ACH-4771, assuming that such designation is successfully obtained and maintained, because, for example, even if Achillion obtains orphan drug designation, exclusivity may not prevent the FDA, EMA or other regulatory authorities from approving competing products; 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, including completing the patent process such that patents issue with respect to composition of matter claims for compounds that inhibit complement factor D activity; demonstrate in any current and future clinical trials the requisite safety, efficacy and combinability of its drug candidates; obtain and maintain necessary regulatory approvals, and the granting of orphan designation does not alter the standard regulatory requirements and process for obtaining such approval; establish commercial manufacturing arrangements; identify, enter into and maintain collaboration and other commercial agreements with third-parties; compete successfully in the markets in which it seeks to develop and commercialize its product candidates and future products; manage expenses; 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 Quarterly Report on Form 10-Q for the fiscal quarter ended September 30, 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.

Investors & Media:
Glenn Schulman, PharmD, MPH
Executive Director, Investor Relations
Achillion Pharmaceuticals, Inc.
Tel. (203) 752-5510
gschulman@achillion.com