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USPTO Grants Composition of Matter Patent to Achillion for ACH-4471, First-in-Class Small Molecule Complement Alternative Pathway Factor D Inhibitor

NEW HAVEN, Conn., Oct. 24, 2017 (GLOBE NEWSWIRE) -- **Achillion Pharmaceuticals, Inc.** (Nasdaq:ACHN) today announced that the U.S. Patent and Trademark Office (USPTO) has issued a U.S. Patent to Achillion for ACH-4471. U.S. Patent No. 9,796,741, entitled "Aryl, Heteroaryl, and Heterocyclic Compounds for Treatment of Complement Mediated Disorders," has been granted with claims to compositions of matter for ACH-4471 and additional compounds that inhibit complement factor D activity, a key mediator in the complement alternative pathway (AP). This issued patent will be the eighth patent that the USPTO has granted to Achillion this year on its complement factor D portfolio. Achillion currently has over one hundred global patent applications and patents covering its complement-related research program.

"We are very pleased with the granting of this composition of matter patent for our first-in-class, oral complement factor D inhibitor, ACH-4471," commented Milind Deshpande, Ph.D., President and Chief Executive Officer of Achillion. "As a leader in complement alternative pathway biology, we are building upon the recently reported efficacy data from our ongoing phase 2 trial with ACH-4471 for untreated PNH, and look forward to reporting the ongoing phase 2 14-day trial of ACH-4471 for patients with C3G / IC-MPGN."

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 plans ongoing clinical development plan including its phase 2 trial of ACH-4471 for untreated PNH and its phase 2 trial of ACH-4471 for C3G / IC-MPGN; Achillion's ability to obtain and maintain patent protection for its drug candidates and the expected benefit of its complement factor D inhibitor intellectual property portfolio; the potential benefits of, and potential indications for, Achillion's compounds that inhibit factor D; 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: 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; 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 Annual Report on Form 10-Q for the fiscal quarter ended June 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.

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