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## **USPTO Grants Composition of Matter Patent to Achillion for Small Molecule Complement Alternative Pathway Factor D Inhibitors**

NEW HAVEN, Conn., March 21, 2017 (GLOBE NEWSWIRE) -- **Achillion Pharmaceuticals, Inc.** (Nasdaq:ACHN) today announced that the U.S. Patent and Trademark Office (USPTO) has issued the first U.S. Patent to Achillion resulting from its complement factor D research program. U.S. Patent No. 9,598,446 has been granted with claims to compositions of matter for compounds that inhibit complement factor D activity, a key mediator in the complement alternative pathway (AP). In addition, the USPTO has issued Notices of Allowance of four additional U.S. patent applications owned by Achillion, also with claims to compositions of matter, that are expected to issue as patents over the next few months.

"Achillion's aim is to establish a broad patent portfolio for its complement program. With the granting of this first patent we are pleased that the USPTO has confirmed that Achillion possesses a multitude of patentable complement factor D inhibitors," commented Milind Deshpande, Ph.D., President and Chief Executive Officer of Achillion. "As a leader in AP biology, we are proud to have been the first to clinically demonstrate, in healthy volunteers, the ability to suppress AP activity following oral dosing of our complement factor D inhibitor, ACH-4471. The receipt of this patent, along with a number of additional patent applications under review by the USPTO, strengthens our position as a leader in factor D inhibitors for the potential treatment of AP-mediated diseases, such as C3G, PNH and geographic atrophy."

### **About the Achillion Alternative Pathway Complement Factor D Platform**

Achillion has leveraged its internal discovery capabilities and a novel complement-related 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), 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 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: complete the patent process such that patents issue from the Notices of Allowance with respect to composition of matter claims for compounds that inhibit complement factor D activity; 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, enter into and maintain collaboration agreements with third-parties, including the current collaboration with Janssen; 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-K for the fiscal year ended December 31, 2016, 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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