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Achillion Initiates Phase I First-In-Human Study of ACH-5228, a Next-Generation Oral Small Molecule Inhibitor of Complement Factor D

NEW HAVEN, Conn., Dec. 20, 2017 (GLOBE NEWSWIRE) -- **Achillion Pharmaceuticals, Inc.** (Nasdaq:ACHN), a pharmaceutical company focused on advancing small molecule inhibitors of factor D in the complement alternative pathway, today announced that the Company has begun dosing subjects in a first-in-human phase 1 trial of ACH-5228, a next-generation, oral small-molecule factor D inhibitor.

"During 2017, we reported interim data from our on-going phase 2 clinical trials, in PNH and in C3G, which demonstrated that factor D inhibition represents a truly novel, first-in-class approach to potentially treating these devastating diseases. As we continue to execute on our global clinical development strategy with ACH-4471, our next-generation compounds, including ACH-5228, represent strategic optionality as we look to expand the breadth of diseases that could potentially be treated via AP inhibition," commented Milind Deshpande, Ph.D., President and Chief Executive Officer of Achillion.

ACH-5228: Phase 1 Healthy Volunteer Development Program

This initial phase 1 trial is a randomized, placebo-controlled, single-ascending dose study of ACH-5228 administered to healthy volunteers. Approximately 28 subjects are expected to be enrolled. The primary endpoint for the trial is evaluation of safety and tolerability. Secondary endpoints include assessments of PK, PD, and evaluation of alternative pathway inhibition in ex vivo laboratory assessments of blood samples from subjects in order to establish a PK/PD relationship for ACH-5228. The Company expects to report interim clinical data from this study during the second half of 2018. (ANZCTR UTN: U1111-1203-1371)

About the Achillion Complement Factor D Platform

Achillion has leveraged its internal discovery capabilities and a novel complement-related platform to develop small molecule drug candidates that are oral inhibitors of complement factor D. Factor D is an essential serine protease involved in the complement pathway, 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 complement alternative pathway plays a critical role. Potential indications being evaluated for these compounds include paroxysmal nocturnal hemoglobinuria (PNH), C3 glomerulopathy (C3G), immune complex-mediated membranoproliferative glomerulonephritis (IC-MPGN), and geographic atrophy (GA).

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 build a platform of potent and specific complement inhibitors. 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: the Phase I clinical trial for ACH-5228, Achillion's next generation Factor D inhibitor, the timing of reporting interim results from the Phase I study of ACH 5228 the potential benefits of, and potential indications for, Achillion's compounds that inhibit factor D, including ACH-4471 and ACH-5228; 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:

demonstrate in any current and future clinical trials the requisite safety, efficacy and combinability of its drug candidates; 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; 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 Quarterly Report on Form 10-Q for the fiscal quarter ended September 30, 2017, and any other SEC filings that Achillion makes from time to time.

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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