Achillion Announces First Dosing in Phase 1 Multiple Ascending Dose Study of ACH-5228 Next-Generation Oral Factor D Inhibitor in Healthy Volunteers

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- ACH-5228, with improved potency and half-life, increases alternative pathway inhibition -
- Achillion planning Investigational New Drug (IND) submission in fourth quarter of 2019 -

NEW HAVEN, Conn., Jan. 31, 2019 (GLOBE NEWSWIRE) -- Achillion Pharmaceuticals, Inc. (Nasdaq: ACHN), a clinical-stage biopharmaceutical company dedicated to transforming the lives of patients and families affected by complement-mediated diseases, today announced dosing of the first healthy volunteer in a Phase 1 multiple ascending dose (MAD) study of ACH-5228, one of the company's next-generation oral, small-molecule factor D inhibitors.

In the completed single ascending dose study, ACH-5228 demonstrated enhanced potency as well as improved pharmacokinetic properties compared to the first-generation factor D inhibitor, ACH-4471. These attributes are projected to provide greater inhibition of the alternative pathway (AP) and reduced dosing frequency for patients with immune-related diseases associated with the AP of the complement system.

"This important clinical milestone for our next-generation oral factor D inhibition program marks an exciting time at Achillion as we pursue safe, effective and innovative treatments for patients with complement-mediated diseases," said Steven Zelenkofske D.O., Executive Vice President, Chief Medical Officer at Achillion. "With ACH-5228's profile, we aim to evolve our oral factor D portfolio to enable alternative pathway inhibition throughout a variety of rare diseases. I look forward to working with our clinical development team to potentially broaden treatment options for patients who may benefit by increased therapeutic availability."

About the ACH-5228 Phase 1 MAD Study
This Phase 1 trial is a randomized, placebo-controlled, multiple-ascending dose study of oral ACH-5228 administered to healthy volunteers outside the United States. Approximately 38 individuals are expected to be enrolled in four separate cohorts. The primary endpoint for the trial is an evaluation of safety and tolerability. Secondary endpoints include assessments of pharmacokinetics (PK), pharmacodynamics (PD), and evaluation of alternative pathway inhibition biomarkers to establish a PK/PD relationship for ACH-5228. Achillion anticipates completing the study in the third quarter of 2019 and submitting an IND to the U.S. Food and Drug Administration supporting continued development for ACH-5228 in the fourth quarter of 2019.

About the Achillion Complement Factor D Portfolio
Achillion has leveraged its internal discovery capabilities and a novel complement-related platform to develop oral small molecule drug candidates that are inhibitors of complement factor D. Factor D is an essential serine protease involved in the AP of the complement system, a part of the innate immune system. Achillion's complement platform is focused on seeking to advance oral small molecules that inhibit the AP and can potentially be used in the treatment of immune-related diseases in which complement AP plays a critical role. Potential indications currently being evaluated for these compounds include paroxysmal nocturnal hemoglobinuria (PNH), C3 glomerulopathy (C3G), and immune complex-mediated membranoproliferative glomerulonephritis (IC-MPGN).

About Achillion Pharmaceuticals
Achillion Pharmaceuticals, Inc. (Nasdaq: ACHN) is a clinical-stage biopharmaceutical company focused on advancing its oral factor D inhibitors into late-stage development and commercialization. Achillion is initially focusing its drug development activities on alternative pathway-mediated diseases where there are no approved therapies or where existing therapies are inadequate for patients. Potential indications being evaluated for its compounds include paroxysmal nocturnal hemoglobinuria (PNH), C3 glomerulopathy (C3G), and immune complex mediated membranoproliferative glomerulonephritis (IC-MPGN). Each of the product candidates in the Company's oral factor D portfolio was discovered in its laboratories and is wholly owned. To advance its investigational product candidates into Phase 3 clinical trials and commercialization, the Company plans to work closely with key stakeholders including healthcare professionals, patients, regulators and payors. 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," "continue," "goal," "strategy," "objective," "may," "potential," and similar expressions to identify such forward-looking statements. These forward-looking statements also include statements about: the potential benefits of factor D inhibition as a treatment for complement-mediated diseases; the potential benefits of, and indications for, Achillion's compounds that inhibit factor D, including ACH-4471, ACH-5228 and ACH-5548; Achillion's belief that its portfolio of compounds could expand factor D portfolio opportunities, provide strategic optionality or create significant value; Achillion's expectations regarding the advancement of, and timeline for reporting results from, clinical trials of its product candidates as well as its ability to advance additional compounds; and other 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 product 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; enroll patients in its clinical trials on its projected timelines; replicate in later stage clinical trials favorable data demonstrated in preclinical and early-stage clinical trials; obtain and maintain patent protection for its product candidates and the freedom to operate under third party intellectual property; 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, 2018, 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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