



Achillion Initiates Phase I First-in-Human Study of ACH-5548, a Third Oral Small Molecule Inhibitor of Complement Factor D

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- Additional clinical compounds expand factor D portfolio opportunities -

NEW HAVEN, Conn., July 10, 2018 (GLOBE NEWSWIRE) -- **Achillion Pharmaceuticals, Inc.**(Nasdaq:ACHN), a clinical-stage pharmaceutical company focused on developing small molecule inhibitors of factor D in the complement alternative pathway (AP), today announced that the Company had begun dosing healthy volunteer subjects in a first-in-human phase 1 trial of ACH-5548, an oral small-molecule complement factor D inhibitor. ACH-5548 is the third clinical compound discovered and developed by Achillion from its complement factor D platform.

ACH-5548, like the second compound from Achillion's factor D portfolio, ACH-5228, is structurally designed to achieve significant improvements in potency and pharmacokinetic properties over first-generation ACH-4471. In pre-clinical studies, both ACH-5228 and ACH-5548 demonstrate multiple-fold increased potency with pharmacokinetic profiles that offer potential for less frequent dosing than ACH-4471.

"The advancement of ACH-5548 into the clinic is an important milestone for Achillion as we continue to broaden our clinical stage factor D portfolio. Clinical data generated to date demonstrate that factor D inhibition represents a novel, first-in-class approach to developing potential treatments for patients suffering from alternative pathway-mediated diseases," commented Joe Truitt, President and Chief Executive Officer of Achillion. "As we continue our transition to a late-stage clinical and commercial company in our global phase 2 clinical programs with ACH-4471 in both PNH and C3G, we also continue to advance next generation compounds in an effort to bring new treatments to patients. We believe this expanded portfolio of three clinical stage factor D inhibitors will provide important strategic optionality for Achillion."

ACH-5548: Phase 1 Healthy Volunteer Development Program

This initial phase 1 trial is a randomized, placebo-controlled, single-ascending dose study of ACH-5548 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-5548. (ANZCTR UTM: 126180-0089-6279)

Achillion anticipates presenting interim phase 1 clinical results from both ACH-5228 and ACH-5548 in the second half of 2018. In addition, the company continues to anticipate presenting results from a 14-day phase 2 clinical trial of ACH-4471 in C3G in the third quarter of 2018, and interim results from longer-duration phase 2 clinical trials of ACH-4471 in both C3G and PNH in the second half of 2018.

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), and immune complex-mediated membranoproliferative glomerulonephritis (IC-MPGN).

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 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-5228 and ACH-5548; Achillion's belief that its portfolio of compounds could expand factor D portfolio opportunities or provide strategic optionality; Achillion's expectations regarding the advancement of, and timeline for reporting results from, clinical trials of ACH-4471 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 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, 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 March 31, 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.

Investors & Media:

Mary Kay Fenton
Achillion Pharmaceuticals, Inc.
Tel. (203) 752-5510
mfenton@achillion.com

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