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Achillion Announces Initiation of Patient Dosing in Phase 2 Study of ACH-4471 for Paroxysmal Nocturnal Hemoglobinuria

NEW HAVEN, Conn., April 06, 2017 (GLOBE NEWSWIRE) -- **Achillion Pharmaceuticals, Inc.** (Nasdaq:ACHN) today announced initiation of patient dosing in a Phase 2 open-label study of ACH-4471, Achillion's first orally-administered, small molecule factor D inhibitor, for patients with paroxysmal nocturnal hemoglobinuria (PNH). This proof of concept study will assess the efficacy, safety, and pharmacokinetics of ACH-4471 in untreated patients with PNH.

"We are pleased to have begun this trial in PNH patients to evaluate ACH-4471, the first orally-administered factor D inhibitor to have demonstrated complement alternative pathway (AP) inhibition in humans, which represents a potentially novel and unique mechanistic approach to treating this life-threatening disease," commented Milind Deshpande, Ph.D., President and Chief Executive Officer of Achillion.

"As an oral, small molecule inhibitor of factor D, ACH-4471 has a unique mechanism of action to address the needs of PNH patients. By blocking the amplification loop of the complement alternative pathway, ACH-4471 has the potential to control intravascular hemolysis as well as prevent the development of extravascular hemolysis," said Professor Peter Browett, BMedSci, MBChB, FRACP, FRCPA; Department of Molecular Medicine and Pathology, University of Auckland, Auckland, New Zealand.

The primary objective of the study is to assess the change-from-baseline in serum lactate dehydrogenase (LDH) levels, a sensitive biomarker for intravascular hemolysis, during 28 days of dosing. The protocol allows for intra-patient dose-escalation with patients initially receiving 100 mg three times daily of ACH-4471 with the ability to increase dosage during the treatment period. Secondary endpoints being assessed include changes in hemoglobin and red blood cell levels, complement pathway biomarkers, such as Bb and factor D, levels, pharmacokinetics, and safety. Interim results from this study are anticipated during the second quarter of 2017.

David Apelian, M.D., Ph.D., Chief Medical Officer, commented, "In addition to PNH, we look forward to evaluating ACH-4471 in a Phase 2 trial for patients with C3 glomerulopathy, a disease due to over activation of the AP, that we plan initiate during the second half of this year. Furthermore, as we continue to evaluate the PK/PD profile of ACH-4471, we are also pursuing extended release formulations that have the potential of achieving desired exposures with less frequent dosing, potentially just once daily."

Further information on this study can be found on www.clinicaltrials.gov. Study identifier NCT03053102.

About Achillion's Complement Alternative Pathway (AP) Factor D Inhibitor 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 age-related macular degeneration (AMD).

About Paroxysmal Nocturnal Hemoglobinuria (PNH)

PNH is thought to be caused by a mutation resulting in the absence of receptors normally present on red blood cells (RBCs) that interact with the AP. The AP of the complement system typically functions normally in these patients but due to the lack of key receptors, known as CD55 and CD59, on the surface of PNH RBCs, the AP treats these cells as foreign and destroys them via hemolysis in the circulatory system (intravascular) and in the liver or spleen (extravascular). Complement factor D is a critical protein within the amplification loop of the AP and it is believed that inhibiting it could control the AP response. Furthermore, this mechanism of action represents a potentially distinct and unique therapeutic approach for controlling intravascular and extravascular hemolysis associated with PNH.

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: the potential benefits of, and potential indications for, Achillion's compounds that inhibit factor D, including the potential for ACH-4471 to treat PNH and C3G; the timing for interim results from the Phase 2 study of ACH-4771 in PNH; and statements concerning Achillion's strategic goals, efforts, plans, and prospects, including those relating to ACH-4771 and its complement factor D inhibitor program. 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; 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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