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Achillion Pharmaceuticals to Support a Natural History Study of C3 Glomerulopathy, a Rare Renal Disorder, Conducted by Experts at Imperial College London

- *Three-year study commenced to include up to 400 patients*
- *Natural history studies track course of a disease over time; can inform and support development and approval of new treatments for patients*
- *C3 glomerulopathy currently has no cure or approved treatment; affects about 8,000 people across Europe and the United States*

NEW HAVEN, Conn., Feb. 28, 2017 (GLOBE NEWSWIRE) -- **Achillion Pharmaceuticals, Inc.** (Nasdaq:ACHN) announced that it has entered into an agreement with Imperial College London to conduct a natural history study of C3 glomerulopathy (C3G), a rare renal disorder which includes dense deposit disease (DDD) and C3 glomerulonephritis (C3GN). C3G affects an estimated 8,000 people across Europe and the United States.

"Currently there is no available cure for C3G, nor any approved treatments to prevent its progression toward renal failure," said Dr. David Apelian, M.D., Ph.D., and Chief Medical Officer at Achillion. "This natural history study will provide important insights into both C3G as a disease and the experiences of patients. We expect this information to be valuable as we continue to progress development of ACH-4471, our first-in-class oral factor D inhibitor."

This study, funded by Achillion, is being conducted by a team of researchers led by Dr. Matthew Pickering and Dr. H. Terry Cook, both of Imperial College. The title of the study is "Natural History Study of C3 Glomerulopathy (C3G): Discovery of Histological Predictors of Outcome." The study is expected to be three years in length. It will include up to 400 participants.

"C3G is a devastating illness for which a treatment or cure is urgently needed. Approximately 10 years after diagnosis, about half of all patients will progress to renal failure, ultimately requiring dialysis or transplantation," said Dr. Pickering. "This natural history study will help to more clearly characterize this disease, and its results will be shared publicly with the hope that its findings will support research and development of a treatment."

A natural history study tracks the course of a disease over time. The aim of such studies is to collect data on disease progression, and this knowledge can inform and support product development and approval. Without a natural history study, this type of information is often not available, or is incomplete, for many rare diseases, including C3G.

C3 glomerulopathy (C3G) describes a rare renal disease characterized by the presence of fragments of a protein called C3 in the filtering units (glomeruli) of the kidney. C3 fragment accumulation results from over-activation of part of the immune system known as the complement alternative pathway. This accumulation results in inflammation in the glomeruli (glomerulonephritis) and subsequent permanent damage (need for dialysis or transplant) in 30-50% of patients within 10 years of diagnosis. C3G affects persons of all ages, although the mean age appears to be lower in DDD patients as compared to C3GN patients. The incidence of C3G is estimated at 2-3 per 1,000,000 people.

Achillion researchers have designed a library of nearly 2,000 small molecule inhibitors of complement factor D. Factor D occupies a critical position in the complement alternative pathway. Dysregulation of the alternative pathway can induce inflammation and tissue damage and is associated with a variety of diseases, including PNH and C3G. Achillion's lead candidate, ACH-4471, entered the clinic last year and is reported to be the first factor D inhibitor to demonstrate complement alternative pathway inhibition in humans after oral dosing.

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 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 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 expected benefits of the C3G natural history study and the information to be derived from such study; 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 the ability of researchers at Imperial College to successfully enroll, complete and extract relevant data from the C3G natural history study, as well as 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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