Achillion Announces Enrollment Milestone in Phase 2 Trials for C3 Glomerulopathy

April 3, 2019

- Enrolled 23 patients in ACH-4471 Phase 2 C3G six and 12-month clinical trials -
- Targeting end-of-Phase 2 meeting with FDA in fourth quarter of 2019 -

BLUE BELL, Pa., April 03, 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 that it achieved threshold enrollment in the Phase 2 trials of ACH-4471 for patients with C3 glomerulopathy (C3G), a devastating disease affecting the kidney for which there is no approved therapy. ACH-4471 will be evaluated in the six-month blinded, placebo-controlled trial (11 patients) and the 12-month open-label trial (12 patients).

"Enrolling our ACH-4471 Phase 2 trials is an important milestone for Achillion, and we would like to thank all of the patients, families, investigators and participating clinical trial sites for their outstanding contributions," said Joe Truitt, President and Chief Executive Officer at Achillion. "The six and 12-month Phase 2 trials for C3G bring us one step closer to evaluating our first-generation factor D inhibitor in patients who suffer from this chronic disease. We remain on track to present data from these two Phase 2 trials, along with real-world C3G data, to the U.S. Food and Drug Administration in the fourth quarter of 2019."

About the ACH-4471 Phase 2 C3G Trials
The objective of the six and 12-month proof-of-concept trials is to evaluate the safety and efficacy of the oral, small molecule factor D inhibitor, ACH-4471, in patients diagnosed with C3G. In the six-month trial, patients are blinded and randomized 1:1 to receive either ACH-4471 or placebo. The 12-month trial is open label. The measures being evaluated include changes in clinical manifestations of C3G including proteinuria and estimated glomerular filtration rate (eGFR) at the end of the treatment period and changes in kidney biopsy from baseline.

ACH-4471, Complement Factor D Inhibitor
The Company's first-generation oral complement factor D inhibitor, ACH-4471, is currently being evaluated for efficacy with Phase 2 clinical programs in both paroxysmal nocturnal hemoglobinuria (PNH) and C3G and has demonstrated preliminary proof-of-concept in both indications. The PNH program consists of a Phase 2 clinical trial evaluating ACH-4471 in patients who are inadequately controlled or sub-optimally responding to eculizumab. Additionally, patients continue to dose in the PNH monotherapy extension trial. The C3G program consists of two Phase 2 clinical trials – a six-month blinded, placebo-controlled trial and a 12-month open-label trial, described above.
More information is available at http://www.achillion.com/patients-and-clinicians/

About C3 Glomerulopathy (C3G)
C3G is a devastating disease affecting the kidney for which there is no approved therapy. C3G affects persons of all ages with men and women equally affected. There are estimated to be approximately 4,000 C3G patients in the United States, 4,000 in Europe, and 1,000 patients with this disease in Japan. C3G describes a rare kidney disease characterized by the presence of C3 protein fragments in the filtering units (glomeruli) of the kidney. These C3 fragment deposits are thought to be the result of overactivation of the complement alternative pathway. The chronic deposition of C3 fragments results in inflammation in the glomeruli (glomerulonephritis) and subsequent permanent kidney damage. An estimated 30 to 50% of C3G patients will require dialysis and/or a kidney transplant within 10 years of diagnosis.

About Achillion Pharmaceuticals
Achillion Pharmaceuticals, Inc. (Nasdaq: ACHN) is a clinical-stage biopharmaceutical company focused on advancing its oral small molecule complement inhibitors into late-stage development and commercialization. Research has shown that an overactive complement system plays a critical role in multiple disease conditions including the therapeutic areas of nephrology, hematology, ophthalmology and neurology. Achillion is initially focusing its drug development activities on complement-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 membranoproliferative glomerulonephritis (IC-MPGN). Each of the product candidates in the Company's oral small molecule 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; Achillion's expectations regarding the timing of regulatory interactions and submissions; 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 Annual Report on Form 10-K for the fiscal year ended December 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.

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