December 5, 2016

Data Presented at American Society of Hematology Meeting Demonstrate Potential Advantages of Factor D Inhibition for the Treatment of Complement Alternative Pathway-Mediated Diseases

SAN DIEGO, Dec. 05, 2016 (GLOBE NEWSWIRE) -- **Achillion Pharmaceuticals, Inc.** (Nasdaq:ACHN) today announced novel research into complement biology that both expands the understanding of this important emerging area and demonstrates the potential advantage of factor D inhibitors like the company's lead candidate, ACH-4471, may have in treating complement mediated diseases. These data were presented at the 58th Annual Meeting of the American Society of Hematology (ASH) in San Diego, CA, taking place from December 3 - 6, 2016.

"The data to be presented at ASH address two key aspects associated with complement inhibition — the 'bystander effect' and managing risk of infection," said David Apelian, M.D., Ph.D. and Chief Medical Officer of Achillion Pharmaceuticals. "This, and other research recently presented on ACH-4471 highlight the important benefits this potential new treatment may provide for PNH and C3G patients. We are excited by the continued advancement of our portfolio of factor D inhibitors, including ACH-4471."

Achillion researchers have designed a library of more than 1,700 small molecule inhibitors of complement factor D. Factor D occupies a critical position in the alternative pathway in the complement system. Dysregulation of the complement system 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 earlier this year and is the first factor D inhibitor to demonstrate complement alternative pathway inhibition in humans after oral dosing.

Presentation Summaries

The poster titled **'Evaluation of bacteria-mediated potential "Bystander" hemolysis of PNH red cells in vitro: No evidence of significant complement classical or lectin pathway-mediated hemolysis induced by microorganisms.'** will be presented by Dr. Xuan Yuan from the laboratory of Dr. Robert Brodsky, Johns Hopkins University, a leading PNH expert. Other authors on the poster include: Guangwei Yang; Jane A. Thanassi; Manuel D. Galvan; Steven D. Podos and Mingjun Huang of Achillion, as well as Dr. Brodsky.

This research demonstrated that complement activation by pathogens leads to pathogen lysis and opsonization but does not cause "bystander" hemolysis or opsonization of PNH cells under these assay conditions in vitro. The researchers' in vitro results suggest that bacterial infections and accompanying complement activation in the setting of ACH-4471 therapy are unlikely to elicit complement-mediated breakthrough hemolysis by the "bystander" mechanism.

A second poster titled **'Effect of complement inhibition by anti-C5 (eculizumab) or a small molecule inhibitor of Factor D (ACH-4471) on survival of meningococci in blood from vaccinated adults'** will be presented by Dr. Dan M. Granoff, UCSF Benioff Children's Hospital, a world expert on meningococcal infections. Other authors are Monica Konar and Eduardo Lujan of UCSF Benioff Children's Hospital.

Data from this *in vitro* research concluded that ACH-4471 had significantly less inhibitory effect than eculizumab on killing of meningococci by plasma or whole blood. Further, this study showed the efficacy of meningococcal vaccines was greatly impaired by eculizumab and was much less impaired by ACH-4471, especially when high levels of bactericidal and opsonic antibodies are maintained.

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," "intend," "plan," "aim," "believe," "seek," "estimate," "can," "focus," "will," "look forward," "goal," and "may" and similar expressions to identify such forward-looking statements. These forward-looking statements also include statements about: the Company's expected plans, timing, data readouts and results from ongoing and planned research and clinical trials of ACH-4471 and other product candidates; and statements concerning the Company's strategic goals, milestone 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: 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. Furthermore, because Janssen is solely responsible for the development and commercialization of our HCV assets under the exclusive worldwide license we granted to it and has the deciding vote on all collaboration matters, Janssen generally has full discretion over all development plans and strategies and may not advance the HCV programs in the time frames Achillion or Janssen projects, or at all, including with regard to the current and planned phase 2a and phase 2b combination trials that include our licensed drug candidates. 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, 2016, and its 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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