Achillion Reports Positive Interim Data for ACH-4471 Phase 2 Trials and Provides Clinical Development Strategy Update

December 17, 2018

ACH-4471, First Generation Oral factor D Inhibitor
- Proof of Concept Validated in both PNH as Monotherapy and in Combination w/C5 Inhibitor
- Proof of Mechanism Validated in C3G, End of Phase 2 Meeting Targeted for 4Q 2019

ACH-5228, Next Generation Oral factor D Inhibitor
- Data Demonstrate 3x to 4x Greater Potency and Extended Half-life
- Phase 1Multiple Ascending Dose Trial targeted to begin January 2019
- USPTO has Issued Achillion a Patent Covering ACH-5528 Composition of Matter

Achillion to Host Conference Call and Webcast Today at 4:30 p.m. EST
Access to Webcast at www.achillion.com

NEW HAVEN, Conn., Dec. 17, 2018 (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 disorders, today reported interim results for the Company's Phase 2 trials of its first-generation oral factor D inhibitor, ACH-4471, as well as Phase 1 pharmacokinetics and potency data for its next-generation factor D inhibitors, ACH-5228 and ACH-5548.

"These compelling findings underscore the momentum for Achillion and our factor D portfolio as we advance three oral small molecule compounds through clinical development. Our oral factor D inhibitors have the potential to treat patients suffering from alternative pathway mediated diseases," said Joe Truitt, President and Chief Executive Officer at Achillion. "In C3G, we have completed patient dosing in our 14-day biomarker/dose ranging study and we are currently enrolling patients in our 6 and 12-month PoC trials. We now have 18 clinical sites open globally and plan to open additional sites in 2019. We are also delighted that the FDA has recently agreed to allow adolescents in our trials as C3G is a disease with unmet medical needs that often begins in childhood. Our C3G plan is to enroll up to 20 patients in our 6 and 12-month trials and to present our data to the FDA at an End of Phase 2 meeting in Q4 2019."

Truitt continued, "The PNH trial data show that factor D inhibition may play an important role in the future treatment paradigm for PNH patients. Our trials have demonstrated a positive impact on patient's hemoglobin, reticulocyte counts, LDH, FACIT-fatigue scores and reduced blood transfusions both as a monotherapy and in combination with a C5 inhibitor. Our hypothesis has been reinforced that if the alternative pathway is adequately inhibited then patient benefit can be achieved in fundamentally different ways than has been seen with C5 inhibitors. We believe this is an unmet medical need and a market segment we will continue to evaluate. Additionally, based on the Phase 1 pharmacokinetic and potency data, our next generation compounds, ACH-5228 and ACH-5548, allow for higher alternative pathway inhibition along with a reduced dosing frequency. These compounds have the potential to be transformative both for patients and Achillion and to deliver on the promise of alternative pathway inhibition across a wide spectrum of diseases."

The Company also confirmed today an expected YE 2018 cash and marketable securities balance of approximately $270 million and a projected 2019 cash burn of $80-85 million.

ACH-4471, Complement Factor D Inhibitor for PNH and C3G

The Company's first-generation oral complement factor D inhibitor, ACH-4471, is being evaluated for safety and efficacy with Phase 2 clinical programs in both PNH and C3G and has demonstrated preliminary proof-of-concept in both indications.

The C3G program consists of two Phase 2 clinical trials which are currently recruiting, a six-month blinded, placebo-controlled study, and a 12-month open label study.

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, we continue to dose patients in our PNH monotherapy extension trial.

More information is available at http://www.achillion.com/patients-and-clinicians/

ACH-5228 and ACH-5548, Next Generation Complement Factor D Inhibitors

ACH-5228 and ACH-5548 are our next-generation oral factor D inhibitors currently in Phase 1 clinical trials. These compounds demonstrated enhanced potency as well as improved pharmacokinetic properties that allow for higher alternative pathway inhibition along with a reduced dosing frequency.

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The live audio and subsequent archived webcasts of the Company's presentations will be accessible from the Company's investor relations website: http://ir.achillion.com.

The audio recording will be archived for 30 days following the live presentation. Please connect to Achillion's website several minutes prior to the start of the presentation to ensure adequate time for any software downloads that may be necessary.

Alternatively, the webcast and audio can be accessed directly as follows:

Webcast Link: https://edge.media-server.com/m6/p/kd6w84c6

US Toll-Free Dial-In Number: (866) 205-4820
International Dial-In Number: (419) 386-0004
Conference ID# 5987613

About Achillion Pharmaceuticals

Achillion Pharmaceuticals, Inc. (Nasdaq: ACHN) is a clinical-stage biopharmaceutical company focused on advancing its oral factor D inhibitors into late-stage development and commercialization. Factor D is an essential serine protease involved in the alternative pathway of the complement system, a part of the innate immune system. Achillion is initially focusing its drug development activities on alternative pathway-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 mediated membranoproliferative glomerulonephritis (IC-MPGN). Each of the product candidates in the Company's oral factor D 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 patients, payors, regulators and healthcare professionals. 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; 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; 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 Quarterly Report on Form 10-Q for the fiscal quarter ended September 30, 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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