Achillion's ACH-5228 Achieves Positive Results in Phase 1 Multiple Ascending Dose Study in Healthy Volunteers

July 22, 2019

- *ACH-5228 delivers near complete and sustained inhibition of the complement alternative pathway*
- *Twice-daily oral administration to move forward in Phase 2 trials*
- *Investigational New Drug (IND) application to be submitted in fourth quarter of 2019*

BLUE BELL, Pa., July 22, 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 results from their Phase 1 multiple ascending dose (MAD) study with ACH-5228 outside of the United States.

In the randomized Phase 1 MAD study, ACH-5228 was administered to 43 healthy volunteers. Study subjects received oral doses of ACH-5228 ranging from 40 mg to 200 mg twice a day (BID) for fourteen days. The study also included a single dose cohort of 240 mg. The results demonstrated that ACH-5228, when dosed 120 mg BID or higher, achieved near complete and sustained Alternative Pathway (AP) inhibition with a mean value of >95% at steady state concentrations as measured by AP Hemolysis and AP Wieslab assays.

The study demonstrated that ACH-5228 was generally well tolerated over the dose ranges tested, which include the doses expected to be evaluated in Phase 2 trials. The Company expects to submit an IND application to the U.S. Food and Drug Administration supporting clinical development for ACH-5228 in the fourth quarter of 2019.

"We are pleased the ACH-5228 Phase 1 MAD data exceeded our expectations of our second-generation oral small molecule factor D inhibitor. With its improved potency and longer durability of effect, we believe that oral ACH-5228 has the potential to be the best-in-class alternative pathway inhibitor. Our plan is to advance ACH-5228 into Phase 2 clinical trials in multiple diseases. Danicopan, our first-generation alternative pathway inhibitor, validated factor D as a target and we plan to begin a Phase 3 registrational study in early 2020," said Joe Truitt, Chief Executive Officer of Achillion Pharmaceuticals.

An updated corporate presentation which refers to the Phase 1 MAD results is available in the "Investor & News" section of Achillion's website: http://ir.achillion.com/events-and-presentations.

About the Achillion Complement Factor D Portfolio
Achillion has leveraged its internal discovery capabilities and a novel complement-related platform to develop oral small molecule drug candidates that are inhibitors of complement factor D. Factor D is an essential serine protease involved in the alternative pathway (AP) of the complement system, a part of the innate immune system. Achillion's complement platform is focused on seeking to advance oral small molecules that inhibit the AP and can potentially be used in the treatment of immune-related diseases in which complement AP plays a critical role. Potential indications currently being evaluated for these compounds include paroxysmal nocturnal hemoglobinuria (PNH), C3 glomerulopathy (C3G), and immune complex-mediated membranoproliferative glomerulonephritis (IC-MPGN).

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 achieve its goal of advancing 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 include statements about: the potential benefits of factor D inhibition as a treatment for complement-mediated diseases, including danicopan (ACH-4471) for PNH; the potential benefits of, and indications for, Achillion's compounds that inhibit factor D, including danicopan and ACH-5228; Achillion's belief that its portfolio of compounds could expand factor D portfolio opportunities, provide strategic optionality or create significant value; and its belief that oral ACH-5228 has the potential to be the best-in-class alternative pathway inhibitor; the status of enrollment in Achillion's ongoing clinical trials; Achillion's expectations regarding the advancement of, and timeline for reporting results from, clinical trials of its product candidates (including danicopan and ACH-5228) as well as its ability to advance additional compounds; Achillion's expectations regarding the timing of regulatory interactions and filings; 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, including danicopan and ACH-5228; 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; whether interim results from a

clinical trial will be predictive of the final results of that trial or whether results of early clinical trials or preclinical studies will be indicative of the results of later clinical trials; enroll patients in its clinical trials on its projected timelines; 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 quarterly period ended March 31, 2019, 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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