Achillion Announces Clearance of Investigational New Drug Application for ACH-5228

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BLUE BELL, Pa., Nov. 11, 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 the U.S. Food and Drug Administration (FDA) has reviewed and cleared the Investigational New Drug Application (IND) for ACH-5228, the Company's second-generation oral factor D inhibitor. The opening IND study in the United States will be a drug-drug interaction study with ACH-5228. The Company expects to begin a Phase 2 proof-of-concept paroxysmal nocturnal hemoglobinuria (PNH) clinical trial of ACH-5228 in the first half of 2020.

"As we continue to develop oral factor D inhibitors, the clearance of an IND is an important regulatory milestone as we advance ACH-5228 from discovery in our laboratories to clinical development," said Dr. Mingjun Huang, Senior Vice President Head of Research. "Patients suffering with PNH, and other devastating complement-mediated diseases, could benefit from a factor D inhibitor capable of sustained inhibition of the Alternative Pathway (AP) with a convenient oral administration."

In a completed, randomized Phase 1 multiple ascending dose study, ACH-5228 was administered to 43 healthy volunteers outside of the United States. The results demonstrated that ACH-5228, when dosed at 120 mg or higher twice a day (BID), achieved near complete and sustained AP inhibition with a mean value of >95% at steady state concentrations as measured by AP Hemolysis and AP Wieslab assays. In the study, ACH-5228 was generally well-tolerated over the dose ranges tested, which included the doses expected to be evaluated in Phase 2 clinical trials.

About Paroxysmal Nocturnal Hemoglobinuria (PNH)

PNH is a rare, acquired blood disease caused by a somatic mutation resulting in the absence of key receptors, CD55 and CD59, on the surface of red blood cells (RBCs). The alternative pathway (AP) of the complement system recognizes these unprotected RBCs as foreign and destroys them in the circulatory system (intravascular hemolysis) and in the liver or spleen (extravascular hemolysis). The current standard of care for PNH targets intravascular hemolysis by inhibiting C5 complement protein (C5), leaving some patients with persistent extravascular hemolysis from early phases of complement activation (AP Activity) which C5 inhibition may not be able to address alone leaving some patients with partial control of their PNH. Up to seventy-five percent of PNH patients treated with C5 inhibitors remain anemic during treatment, with up to one-third of those patients reporting the need for blood transfusions within the prior year. Factor D is the critical, rate-limiting protein within the AP. By targeting factor D, proximal AP inhibition may disable both downstream terminal complement activation (IVH) and upstream C3 fragment opsonization (EVH). Achillion is developing a potentially more complete approach to PNH with factor D inhibition to selectively block alternative pathway activity and protect against both destructive processes of RBCs in PNH with convenient oral therapies.

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

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 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 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 FDA's Breakthrough Designation for danicopan; the potential benefits of factor D inhibition as a treatment for complement-mediated diseases, including danicopan (ACH-4471) for PNH; Achillion's expectations regarding the advancement of, and timeline for reporting results from, clinical trials of its product candidates (including danicopan and ACH-5228); 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: continue to meet the clinical development program criteria for Breakthrough Designation; accelerate the development timeline for danicopan utilizing benefits available through the Breakthrough Designation; 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 September 30, 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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