



Achillion Receives Breakthrough Therapy Designation from FDA for Danicopan for Treatment of Paroxysmal Nocturnal Hemoglobinuria

September 25, 2019

– Danicopan Phase 2 PNH combination therapy topline data expected Q4 2019 –
– Initiation of Phase 3 planned for early 2020 –

BLUE BELL, Pa., Sept. 25, 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 granted Breakthrough Therapy designation for danicopan (ACH-4471) for treatment in combination with a C5 monoclonal antibody for patients with paroxysmal nocturnal hemoglobinuria (PNH) who are sub-optimal responders to a C5 inhibitor alone. The FDA's decision was based on positive safety and efficacy data from the ongoing danicopan Phase 2 PNH combination trial. Interim data was reported at the New Era of Aplastic Anemia and PNH Meeting in May 2019. The top line data from this combination trial is expected in the fourth quarter of 2019.

"The FDA's granting of Breakthrough Therapy designation for our lead oral factor D inhibitor, danicopan, underscores the urgent need for new treatment options for patients living with PNH," said Joe Truitt, President and Chief Executive Officer at Achillion. "Danicopan, with its demonstrated ability to limit both intravascular and extravascular hemolysis with oral administration, has the potential to benefit a significant number of patients with PNH that continue to have an unmet medical need on standard of care. We appreciate the review and decision by the FDA and plan to work closely with the Agency in advancing the development of danicopan into Phase 3 in early 2020."

FDA Breakthrough Therapy designation is designed to expedite the development and review of medicines for serious or life-threatening conditions. Receiving Breakthrough Therapy designation from the FDA indicates preliminary clinical evidence has demonstrated the drug may provide substantial improvement on at least one clinically significant endpoint compared with currently available therapy. The benefits of this Breakthrough Therapy designation include more intensive guidance from FDA on an efficient drug development program, access to a scientific liaison to help accelerate review time and eligibility for Accelerated Approval and Priority Review if relevant criteria are met. Danicopan (ACH-4471) has previously received orphan drug designation for the treatment of PNH in 2017.

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 cannot address leaving 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 last 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 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 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 June 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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