European Medicines Agency Grants PRIME Designation to Danicopan for Treatment of Paroxysmal Nocturnal Hemoglobinuria Patients Who Are Not Adequately Responding to a C5 Inhibitor

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– Phase 3 PNH initiation planned early 2020 –

BLUE BELL, Pa., Nov. 19, 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 European Medicines Agency (EMA) has granted access to support through the PRIME (PRIority MEdicines) program for danicopan (ACH-4471) in the treatment of paroxysmal nocturnal hemoglobinuria (PNH) in patients who are not adequately responding to a C5 inhibitor. The decision from the EMA was based on danicopan safety and efficacy data from the Phase 2 PNH combination trial. The top-line 24-week results from this combination trial was accepted for poster presentation at the 61st American Society of Hematology (ASH) Annual Meeting scheduled for December 9th in Orlando, FL.

"We are very pleased that the European Medicines Agency has granted PRIME designation for danicopan, our lead oral factor D inhibitor," said Dr. Kevin P. Malobisky, Senior Vice President Regulatory Affairs, Quality & Compliance. "The EMA's decision further highlights the unmet medical need for PNH patients who are suboptimal responders to a C5 inhibitor, the current standard of care. We appreciate the review and decision by the EMA and plan to continue working closely with the Agency through Scientific Advice to advance the development of danicopan into a global Phase 3 trial in early 2020."

The EMA launched the PRIME program to ensure that promising medicines that may provide a therapeutic advantage over existing treatments have a pathway to accelerate development through early interaction and dialogue. The program is intended to optimize development plans and expedite the review and approval process so that these medicines may reach patients as early as possible. The Agency will confirm eligibility to the centralized procedure at the time of a marketing authorization application (MAA) and will appoint a Rapporteur from the Committee for Medicinal Products for Human Use (CHMP) to provide continuous support and help to build knowledge ahead of a marketing authorization application. To be eligible for PRIME, medicines must target an unmet medical need and show potential benefit for patients based on early clinical data. Danicopan (ACH-4471) has previously received orphan drug designation and Breakthrough Therapy designation from the U.S. Food & Drug Administration (FDA) for the treatment of PNH, and orphan status from the European Medicines Agency.

About Paroxysmal Nocturnal Hemoglobinuria
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 complement system recognizes these unprotected RBCs as foreign and destroys them in the circulatory system (intravascular hemolysis [IVH]) and in the liver or spleen (extravascular hemolysis [EVH]). The current standard of care for PNH targets IVH by inhibiting C5 complement protein (C5), leaving some patients with persistent EVH from early phases of complement activation (alternative pathway [AP] activity) which C5 inhibition cannot address. This may leave patients with partial control of their PNH symptoms. 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 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 EMA's PRIME designation for danicopan,

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 initiation of, 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 PRIME designation and for Breakthrough Designation; accelerate the development timeline for danicopan utilizing benefits available through the Prime designation and 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.

Investor Relations:
Clayton Robertson
Achillion Pharmaceuticals, Inc.
Tel. 215-709-3078
crobertson@achillion.com

Media:
Susanne Heinzinger
Senior VP, Corporate Communications
Achillion Pharmaceuticals, Inc.
Tel. 215-709-3032
sheinzinger@achillion.com

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