



December 18, 2017

Achillion Announces ACH-4471 Granted Orphan Drug Designation for the Treatment of C3 Glomerulopathy (C3G) and the Initiation of a Phase 1 Extended-Release Bioavailability Study

- *Orphan drug designation for the treatment of C3G granted by the U.S. Food and Drug Administration (FDA) —*
- *Initiated bioavailability study evaluating extended-release formulations of ACH-4471 in healthy volunteers—*

NEW HAVEN, Conn., Dec. 18, 2017 (GLOBE NEWSWIRE) -- **Achillion Pharmaceuticals, Inc.** (Nasdaq:ACHN), a pharmaceutical company focused on advancing small molecule inhibitors of factor D in the complement alternative pathway, today announced the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to ACH-4471 for the treatment of patients with C3 Glomerulopathy (C3G).

C3G is a devastating renal disease for which there is no approved therapy. There are estimated to be approximately 4,000 C3G patients in the United States, approximately 4,000 in Europe, and more than 1,000 patients with this devastating disease in Japan.

The FDA Orphan Drug Designation program provides incentives for the development of potentially promising drugs to treat, diagnose or prevent orphan diseases and disorders that affect fewer than 200,000 people in the U.S. This designation may provide, under specified conditions, for a seven-year marketing exclusivity period, as well as certain incentives, including federal grants, tax credits and a waiver of PDUFA filing fees.

On November 14, 2017, Achillion announced interim data from the first two patients in an on-going Phase 2 study in C3G patients demonstrated that the current formulation of ACH-4471 achieved complement alternative pathway inhibition resulting in greater than 50% reduction in proteinuria over the 14-day treatment period and a favorable tolerability profile.

Additionally, Achillion announced the initiation of bioavailability study of extended release formulations of ACH-4471 in healthy volunteers. The extended release oral tablet formulations may have the potential to achieve once or twice daily dosing in patients.

"We are pleased that the FDA has granted orphan drug designation to ACH-4471 for treatment of C3G, and we look forward to additional data from the ongoing extended release formulation study. We are keenly aware of the unmet need for patients and we are committed to advancing ACH-4471 for C3G as we believe we have an opportunity to develop a potentially disease-modifying therapy, based on the unique mechanism of action, for ACH-4471," commented Milind Deshpande, Ph.D., President and CEO.

ACH-4471: Phase 1 Bioavailability Study of Extended-Release Formulations

The purpose of this study is to evaluate and compare the pharmacokinetic profiles of multiple extended-release formulations of ACH-4471 in healthy subjects, after oral administration, with the goal of identifying an extended-release formulation to enable once or twice daily dosing regimens of ACH-4471. Interim results are anticipated during the second quarter of 2018. (EudraCT: 2017-003525-15)

About the Achillion Complement Factor D Platform

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

About Achillion Pharmaceuticals

Achillion Pharmaceuticals, Inc. (NASDAQ:ACHN) is a science-driven, patient-focused company seeking to leverage its strengths across the continuum from discovery to commercialization in its goal of providing better treatments for people with serious diseases. The company employs a highly-disciplined discovery and development approach that has allowed it to build a platform of potent and specific complement inhibitors. Achillion is rapidly advancing its efforts to become a fully-integrated pharmaceutical company with a goal of bringing life-saving medicines to patients with rare diseases. 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," "goal," "may," "potential," and similar expressions to identify such forward-looking statements. These forward-looking statements also include statements about: the expected benefits of orphan drug designation; the potential for development of an extended release formulation for ACH-4471; the potential benefits of, and potential indications for, Achillion's compounds that inhibit factor D, including ACH-4771; and 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 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; avail itself of the benefits of orphan drug designation for ACH-4771, because, for example, orphan drug designation exclusivity may not prevent the FDA or other regulatory authorities from approving competing products; develop an extended release formulation of ACH-4471 that is able to achieve once or twice daily dosing regimens; demonstrate in any current and future clinical trials the requisite safety, efficacy and combinability of its drug 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; obtain and maintain patent protection for its drug 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, 2017, 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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