Achillion Announces Completion of Enrollment in Phase 2 PNH Combination Trial

March 27, 2019

- Completed enrollment in ACH-4471 Phase 2 PNH trial in combination with eculizumab -
- Interim data to be released in May 2019 -
- Targeting FDA end-of-Phase 2 meeting in second half of 2019 -

BLUE BELL, Pa., March 27, 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 achievement of enrollment in their ACH-4471 Phase 2 paroxysmal nocturnal hemoglobinuria (PNH) trial in combination with eculizumab.

In the Phase 2 trial, patients with an inadequate response to eculizumab as monotherapy are being evaluated for increases in hemoglobin and reduced transfusions over 24-weeks. Early data released in December 2018 provided evidence that ACH-4471 in combination with eculizumab had a positive impact on patient hemoglobin and FACIT-fatigue scores while reducing transfusions, reticulocyte counts and LDH levels.

The company expects to present interim data for the first 10 patients on May 17, 2019 at The New Era of Aplastic Anemia and Paroxysmal Nocturnal Hemoglobinuria meeting in Napoli, Italy. This specialized meeting is sponsored by AIEPEN Onlus, the Italian PNH Association.

“We are excited to present interim data on the first 10 patients enrolled and to discuss these results with the FDA,” said Steven Zelenkofske, D.O., Executive Vice President, Chief Medical Officer at Achillion. “This is an important clinical milestone for our first-generation oral factor D inhibitor program, as it adds to the growing body of ACH-4471 data in PNH patients who remain anemic or transfusion dependent while receiving eculizumab. We believe this data supports the hypothesis that alternative pathway inhibition can potentially provide additional benefit to patients who are receiving C5 inhibitors, such as eculizumab.”

The data from the ACH-4471 Phase 2 combination study along with the prior completed 10 patient Phase 2 monotherapy study will be submitted for review by regulatory authorities in an end of Phase 2 meeting in the second half of 2019.

About the ACH-4471 Phase 2 Combination Study
A Phase 2 open-label study of ACH-4471 in up to 12 patients with Paroxysmal Nocturnal Hemoglobinuria (PNH) who have an inadequate response to eculizumab as monotherapy. The purpose of this dose-ranging study is to determine the effectiveness of ACH-4471 in improving anemia, as measured by increased blood hemoglobin, when given with eculizumab (a C5 inhibitor commonly used for treatment of PNH) for 24 weeks. In December 2018, Achillion announced interim data for the first four patients in this trial, which showed trial participants reduced blood transfusions to zero from an aggregate of 14 in the prior 52 weeks. In addition, early data provided evidence that ACH-4471 in combination with eculizumab had a positive impact on patient’s hemoglobin, reticulocyte counts, LDH, and FACIT-fatigue scores. The Company believes this data helps support its hypothesis that if the alternative pathway is adequately inhibited, therapeutic benefit can be achieved in fundamentally different ways than has been seen with C5 inhibitors alone. Achillion expects to report updated interim results for this Phase 2 clinical trial in May 2019 and is targeting a meeting with the FDA in the second half of 2019.

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 a number of 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 advance 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 also include statements about: the potential benefits of factor D inhibition as a treatment for complement-mediated diseases; the potential benefits of, and indications for, Achillion’s compounds that inhibit factor D, including ACH-4471, ACH-5228 and ACH-5548; Achillion’s belief that its portfolio of compounds could expand factor D portfolio opportunities, provide strategic optionality or create significant value; Achillion’s expectations regarding the advancement of, and timeline for reporting results from, clinical trials of its product candidates as well as its ability to advance additional compounds; 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; 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; enroll patients in its clinical trials on its projected timelines; replicate in later stage clinical trials favorable data demonstrated in preclinical and early-stage clinical trials; 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 Annual Report on Form 10-K for the fiscal year ended December 31, 2018, 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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Source: Achillion Pharmaceuticals, Inc.