Achillion Announces Upcoming Scientific Presentation at the 24th Congress of the European Hematology Association

June 10, 2019

BLUE BELL, Pa., June 10, 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 final data set for ACH-4471 (newly designated "danicopan") proof of concept Phase 2 trial in untreated patients with paroxysmal nocturnal hemoglobinuria (PNH) was accepted for oral presentation at the 24thCongress of the European Hematology Association (EHA). The 24thCongress of the EHA is being held in Amsterdam, Netherlands from June 13 – 16, 2019. The oral presentation (abstract code S864) is scheduled for June 15, 12:15 pm local time and will be presented by Dr. Peter Browett, BMedSci, MBChB, FRACP, FRCPA from the University of Auckland, Auckland, New Zealand. Topline data from this trial was previously reported by Achillion in December 2018.

Additionally, the Company has received acceptance for the use of the name danicopan for its oral, small molecule factor D inhibitor, ACH-4471, from the United States Adopted Names (USAN) Council and the World Health Organization's International Nonproprietary Names (INN) Expert Committee.

Presentation Highlights:

The presentation titled "A Phase 2 Open-label Proof-of-concept Study of the Oral, Small Molecule Factor D Inhibitor, ACH-4471, in Untreated Patients with Paroxysmal Nocturnal Hemoglobinuria (PNH)" presents data showing:

- Reduction in lactate dehydrogenase (LDH) at Day 28 (n=10) compared to baseline (primary endpoint)
- Improvement in additional markers of PNH, including hemoglobin, reticulocyte count, bilirubin, clone size, and Functional Assessment of Chronic Illness Therapy (FACIT) Fatigue scores compared to baseline at Days 28 (n=10) and 84 (n=8)
- As previously described in December, one patient discontinued therapy for an SAE however the drug was generally well tolerated with 8 of 10 patients electing to continue into a long-term extension study with durability of effect and long-term tolerability up to 2 years

Following the Annual Congress of EHA, the full presentation will be available in the "Investor & News" section of Achillion's website: http://ir.achillion.com/events-and-presentations.

About Danicopan (ACH-4471) Phase 2 PNH Monotherapy Trial
The Company's first-generation oral complement factor D inhibitor, danicopan, was evaluated for safety and efficacy in untreated patients with PNH. This is a Phase 2, open-label, multiple dose trial in adult patients with anemia, defined as a hemoglobin <12 g/dL, and LDH ≥1.5X upper limit of normal (ULN) at enrollment. This clinical trial enrolled ten patients and was comprised of two Parts where patients were administered danicopan orally, three times a day at a dose determined by clinical response. In Part 1 of the study, patients received study medication for 28 days. The primary objective was the reduction in LDH from baseline at Day 28. In Part 2, based on a review of safety and efficacy data through Day 20 along with prespecified LDH reduction criteria, patients, who in the opinion of the Principal Investigator received benefit from danicopan, were offered continued dosing for up to 8 additional weeks. These patients were enrolled into a long-term extension study which began at the conclusion of Part 2.

About Paroxysmal Nocturnal Hemoglobinuria (PNH)
PNH is thought to be caused by a mutation resulting in the absence of receptors normally present on red blood cells (RBCs) that interact with the complement system. The complement system typically functions normally in these patients but due to the lack of key receptors, known as CD55 and CD59, on the surface of the PNH RBCs, the complement system treats these cells as foreign and destroys them via hemolysis in the circulatory system (intravascular) and in the liver or spleen (extravascular). The complement alternative pathway (AP) is a critical factor in the development of extravascular hemolysis. Complement factor D is a critical protein within the amplification loop of the AP and it is believed that inhibiting it could control the AP response. Furthermore, this mechanism of action represents a potentially distinct and unique therapeutic approach for controlling intravascular and extravascular hemolysis associated with PNH.

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

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 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, including danicopan (ACH-4471) for PNH; the potential benefits of, and indications for, Achillion’s compounds that inhibit factor D, including danicopan (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; the status of enrollment in Achillion’s ongoing clinical trials; 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; Achillion’s expectations regarding the timing of regulatory interactions and filings; Achillion’s anticipated cash expenditures for 2019 and the sufficiency of its existing cash resources; 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; 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 March 31, 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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