



Achillion Announces Data Presentations at the 61st American Society of Hematology Annual Meeting

November 6, 2019

BLUE BELL, Pa., Nov. 06, 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 the acceptance of two abstracts about danicopan, the Company's lead oral factor D inhibitor, for poster presentation at the 61st American Society of Hematology (ASH) Annual Meeting to be held in Orlando, Florida from December 7-10, 2019.

"We are pleased to have two abstracts accepted for poster presentation at the upcoming ASH meeting; including the top-line 24-week combination data from the Phase 2 trial in PNH patients with a sub-optimal response to a C5 inhibitor. We aim to advance the development of oral danicopan for PNH patients with a suboptimal response to a C5 inhibitor," stated Executive Vice President and Chief Medical Officer Dr. Steven Zelenkofske. "Our second poster is an evaluation of pharmacokinetic and pharmacodynamic biomarker data from Phase 2 treatment-naïve PNH patients treated with oral danicopan."

Presentation Details

Poster Presentation: "A Phase 2 Open-Label Study of Danicopan (ACH-0144471) in Patients with Paroxysmal Nocturnal Hemoglobinuria (PNH) Who Have an Inadequate Response to Eculizumab Monotherapy"

Abstract Number: 3514

Session Name: 101. Red Cells and Erythropoiesis, Structure and Function, Metabolism, and Survival, Excluding Iron: Poster III

Session Date: Monday, December 9, 2019

Presentation Time: 6:00 PM – 8:00 PM

Location: Hall B

Poster Presentation: "Mechanistic Evaluation of Efficacy Using Biomarkers of the Oral, Small Molecule Factor D Inhibitor, Danicopan (ACH-4471), in Untreated Patients with Paroxysmal Nocturnal Hemoglobinuria (PNH)"

Abstract Number: 2226

Session Name: 101. Red Cells and Erythropoiesis, Structure and Function, Metabolism, and Survival, Excluding Iron: Poster II

Session Date: Sunday, December 8, 2019

Presentation Time: 6:00 PM – 8:00 PM

Location: Hall B

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 may not be able to address alone leaving some 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 prior 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 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; Achillion’s anticipated cash expenditures for 2019 and the sufficiency of its existing cash resources; the proposed acquisition of Achillion by Alexion, including the potential contractual contingent value right payment by Alexion in connection with the acquisition; 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 and risks and uncertainties related to the proposed acquisition of Achillion by Alexion include, among other things, risks related to the fact that the proposed acquisition may not be completed due to Achillion’s or Alexion’s failure to satisfy or waive the conditions to closing the proposed acquisition. 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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