



September 11, 2017

## **Achillion Announces Termination of Worldwide Collaboration for Hepatitis C With Janssen**

*-Conference call to be held today at 9:00 a.m. ET-*

NEW HAVEN, Conn., Sept. 11, 2017 (GLOBE NEWSWIRE) -- **Achillion Pharmaceuticals, Inc.** (Nasdaq:ACHN) announced today that on Saturday, September 9, 2017, it received notice of termination of the worldwide license and collaboration arrangement on hepatitis C with Janssen Pharmaceuticals, Inc. (Janssen), one of the Janssen Pharmaceutical Companies of Johnson & Johnson. The notice follows the decision by Janssen announced today to discontinue the development of the investigational hepatitis C treatment regimen JNJ-4178, a combination of three direct acting antivirals: AL-335, odalasvir and simeprevir.

"We are disappointed by Janssen's decision to discontinue HCV development given the positive data presented in phase 2a with JNJ-4178<sup>1</sup>, demonstrating a 100% cure rate after only six weeks of therapy," stated Milind Deshpande, Ph.D., President and Chief Executive Officer of Achillion. "While we believe that patients worldwide would benefit from convenient, short-duration therapies like JNJ-4178, we remain fully focused on advancing our factor D portfolio of complement alternative pathway inhibitors in areas where patient needs are greatest, and using our strong balance sheet of almost \$370 million in cash and cash equivalents at June 30, 2017 to do so."

Since entering into the Janssen license arrangement in 2015, Achillion has focused on and advanced multiple small molecule factor D inhibitors of the complement alternative pathway. The first oral factor D inhibitor to demonstrate complete suppression of the alternative pathway, ACH-4471, is now in development for PNH, as well as C3G and IC-MPGN. In August 2017, Achillion announced positive interim data from a phase 2 three-month, dose-ranging trial with ACH-4471 for patients with untreated PNH. In addition to its plans to continue this study of untreated PNH patients, Achillion anticipates initiating a second phase 2 study in PNH patients who sub-optimally respond to treatment with eculizumab, the current standard of care.

Also during the second half of 2017, Achillion anticipates initiating patient dosing in two phase 2 trials of ACH-4471 for patients with low C3 levels due to C3G or IC-MPGN. The first is an open-label, 14-day trial expected to enroll up to 10 patients, while the second is a placebo-controlled, double-blinded six-month trial expected to enroll 20 patients. "We believe the data we have generated to date in our factor D platform are compelling and point to the potential long-term value of our portfolio. We look forward to advancing our goal of bringing factor D inhibitors to patients in need of such therapies," commented Dr. Deshpande.

### **Webcast and Dial-in Information**

Achillion will host a conference call and simultaneous webcast on Monday, September 11, 2017 at 9:00 a.m. ET. To participate in the conference call, please dial (866) 205-4820 in the U.S. or (419) 386-0004 for international callers. A live audio webcast of the call will be accessible at <http://www.achillion.com> or <http://ir.achillion.com>. Please connect to Achillion's website several minutes prior to the start of the broadcast to ensure adequate time for any software download that may be necessary.

A replay of the webcast will be available for 30 days on <http://www.achillion.com>. Alternatively, a telephonic replay of the conference call will be available starting at 11:30 a.m. ET on September 11, 2017, through 12:05 p.m. ET on September 15, 2017 by dialing (855) 859-2056 or (404) 537-3406. The replay passcode is 84176694.

### **About Achillion's Complement Alternative Pathway (AP) Factor D Inhibitor Platform**

Achillion has leveraged its internal discovery capabilities and a novel complement-related platform to develop small molecule factor D inhibitor compounds that target the complement AP. Factor D is an essential serine protease involved in the AP, 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 the AP plays a critical role. Potential indications currently being evaluated for these compounds include paroxysmal nocturnal hemoglobinuria (PNH), C3 glomerulopathy (C3G), immune-complex membranoproliferative glomerulonephritis (IC-MPGN), and geographic atrophy (GA), an advanced form of dry age-related macular degeneration.

## 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 AP. The AP of 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 PNH RBCs, the AP treats these cells as foreign and destroys them via hemolysis in the circulatory system (intravascular) and in the liver or spleen (extravascular). 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.

## About C3 Glomerulopathy (C3G)

C3G is a rare renal disease which is believed to be the result of over-activity of the AP. There is currently no cure available for C3G, no approved treatment to prevent disease progression and a poor prognosis for patients, of whom approximately 30-50% require dialysis or kidney transplant 10 years after diagnosis. ACH-4471 has been shown *in vitro* to inhibit alternative pathway activity, potentially decreasing the formation of C3 protein fragments. This mechanism of action is believed to be a distinct approach to potentially controlling the underlying cause of this disease.

## 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 factor D inhibitors for AP-mediated diseases. 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 include statements about: the potential benefits of, and potential indications for, Achillion's compounds that inhibit factor D, including the potential for its compounds to treat PNH, C3G, IC-MPGN, geographic atrophy and other diseases; plans to continue the phase 2 trial of ACH-4471 in untreated PNH patients; the timing for initiation of clinical trials in PNH, C3G and IC-MPGN with ACH-4471; the timing for interim or final results from Achillion's clinical trials; statements concerning Achillion's ability to achieve its strategic goals, and statements concerning its plans, and prospects, including those relating to ACH-4771, ACH-5228 and its complement factor D inhibitor program; and the Company's beliefs regarding the potential benefits of JNJ-4178. 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, Janssen's termination of the collaboration agreement, and 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; obtain and maintain patent protection for its technologies and drug candidates and the freedom to operate under third party intellectual property; demonstrate in any current and future clinical trials the requisite safety, efficacy and combinability of its drug candidates; obtain and maintain necessary regulatory approvals; establish commercial manufacturing arrangements; identify, enter into and maintain collaboration 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 June 30, 2017, and any subsequent SEC filings.

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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<sup>1</sup> Gane, E. *et al.* Short duration treatment with AL-335 and odalasvir, with or without simeprevir, in treatment naïve patients with hepatitis C infection with or without cirrhosis. *Journal of Hepatology*. 2017: 66(1):S82.

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