



February 28, 2018

Achillion Raises Awareness for C3 Glomerulopathy With Launch of WeC3G™ Initiative as Part of Rare Disease Day 2018

- | *February 28, 2018 is the 11th Annual Rare Disease Day*
- | *Achillion sponsors initiative to help shine the light on C3G to raise awareness and understanding of this rare, chronic disease affecting the kidneys*

NEW HAVEN, Conn., Feb. 28, 2018 (GLOBE NEWSWIRE) -- **Achillion Pharmaceuticals, Inc.** (Nasdaq:ACHN), a biopharmaceutical company focused on advancing oral small-molecule factor D inhibitors to modulate the complement alternative pathway, today announced the launch of a new patient support initiative called [WeC3G™](#), aimed at raising awareness and understanding of C3 glomerulopathy ("C3G") a rare and chronic disease affecting the kidneys.

[WeC3G](#), sponsored by Achillion, is an initiative to connect patients and families living with C3G to information, support, resources, and each other to help to shine the light on C3G. The initial launch includes a program website containing patient and caregiver resources, educational material, a digital toolkit, and the ability to sign up for ongoing news and updates.

Milind Deshpande, Ph.D., Chief Executive Officer of Achillion, commented, "On this Rare Disease Day, Achillion is proud of its sponsorship of WeC3G, along with other recent efforts, such as supporting a global natural history study of C3G to track the course of this disease, and sponsorship of last August's Patient Focused Drug-Development (PFDD) meeting for C3G hosted by the National Kidney Foundation. Achillion is steadfastly committed to innovative research with a goal of addressing unmet medical needs of patients with C3G by advancing potentially disease-modifying therapies."

To learn more, please visit the WeC3G website, <http://www.WeC3G.com>, or follow WeC3G on Facebook, <http://www.facebook.com/wec3g>, where patients and caregivers can join the conversation.

C3 glomerulopathy (glo-MER-u-LOP-ah-the), or C3G, is a rare, chronic disease in which experts believe that a specific part of the immune system, the alternative pathway of the complement system, is not well controlled, resulting in deposition of C3 protein fragments in the kidney often leading to damage and potential loss of function. It has been estimated that there are approximately 4,000 people in the United States living with C3G, a disease for which there are no approved treatments.

Achillion Pharmaceuticals is focused on modulation of the complement system via oral small-molecules that inhibit the alternative pathway (AP), a potentially transformative approach to overcoming a variety of immunological, hematologic and nephritic diseases for which patients have limited or no treatment options. We believe factor D inhibition represents a highly innovative and differentiated mechanism of action with the potential to address C3G.

About Rare Disease Day

Rare Disease Day is an annual event that takes place on the last day of February. Its goal is to help policy makers and the public understand the impact of rare diseases, such as C3G, on the lives of patients. To learn more, please visit <http://www.rarediseaseday.org>.

About C3G

C3G is a devastating disease affecting the kidneys for which there is no approved therapy. C3G affects men and women equally. There are estimated to be approximately 4,000 C3G patients in the United States, more than 4,000 in Europe, and more than 1,000 patients with this disease in Japan. C3G describes a rare renal disease characterized by the presence of predominantly C3 protein fragments in the filtering units (glomeruli) of the kidney. These C3 fragment deposits are thought by experts to be the result of overactivation of the complement alternative pathway. The chronic deposition of C3 fragments results in inflammation in the glomeruli (glomerulonephritis) and subsequent permanent renal damage. An estimated 30-50% of C3G patients will require dialysis or a transplant within 10 years of diagnosis.

For additional information on Achillion's C3G clinical trials, please visit:

- | Proof of Concept Study for 6 Month Treatment in Patients With C3 Glomerulopathy (C3G): [NCT03369236](#)
- | Proof-of-Mechanism Study to Determine the Effect of ACH-0144471 on C3 Levels in Patients With C3G or IC-MPGN: [NCT03124368](#)

About the Complement Factor D Platform

Achillion has leveraged its internal discovery capabilities and a novel complement-related drug development 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 C3G, immune complex-mediated membranoproliferative glomerulonephritis (IC-MPGN), and paroxysmal nocturnal hemoglobinuria (PNH).

About Achillion Pharmaceuticals

Achillion Pharmaceuticals, Inc. (NASDAQ:ACHN) is a science-driven, patient-focused company seeking to leverage its capabilities 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 also include statements about: the potential benefits of, and potential indications for Achillion's compounds that inhibit factor D; 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; obtain and maintain patent protection for its 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 Annual Report on Form 10-K for the fiscal year ended December 31, 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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