Achillion Granted Twentieth Patent for Factor D Portfolio

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USPTO grants composition of matter patent for ACH-5548, Achillion's 3rd clinical-stage asset

BLUE BELL, Pa., May 14, 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 U.S. Patent and Trademark Office (USPTO) issued U.S. Patent #10,287,301 to Achillion for ACH-5548, its next-generation orally administered complement factor D small molecule inhibitor product candidate. This patent covers the composition of matter of ACH-5548, and a pharmaceutical composition that includes ACH-5548, a key mediator in the complement alternative pathway (AP). This issued patent will be the twentieth patent that the USPTO has granted to Achillion for its complement factor D portfolio of small molecules. Achillion currently has over one hundred global patent applications and patents including the composition of matter and method of use for its complement-related research program.

"Our intellectual property estate continues to yield issued patents stemming from our pioneering efforts in the discovery and development of oral small molecule factor D inhibitors, both in the United States and foreign countries. Achillion now has issued U.S. patents claiming the composition of matter for all three of its clinical development compounds, ACH-4471, ACH-5228 and ACH-5548," commented Joseph Truitt, President and Chief Executive Officer of Achillion. "We believe this extensive patent portfolio provides a foundation to support the broader application of our factor D inhibitors in hematology and nephrology and potentially other therapeutic areas including ophthalmology and neurology."

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 paroxysmal nocturnal hemoglobinuria (PNH), C3 glomerulopathy (C3G), and immune complex-mediated membranoproliferative glomerulonephritis (IC-MPGN).

About Achillion Pharmaceuticals

Achillion Pharmaceuticals, Inc. 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 Achillion's website 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 expected advantages and benefits of the Company's patent portfolio; 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; 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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