August 8, 2017

Achillion Reports Second Quarter 2017 Financial Results and Proof-of-Concept With a First-in-Class, Oral Factor D Inhibitor

- Interim results demonstrate ability of ACH-4471 to reduce LDH and improve hemoglobin and fatigue scores in patients with untreated PNH -

- Global program expansion planned in PNH, C3G and IC-MPGN -

- Conference call scheduled for today at 4:05 p.m. ET -

NEW HAVEN, Conn., Aug. 08, 2017 (GLOBE NEWSWIRE) -- **Achillion Pharmaceuticals, Inc.** (Nasdaq:ACHN) today reported financial results for the three and six months ended June 30, 2017. For the second quarter of 2017, Achillion reported a net loss of $22.5 million or $0.16 per share, compared with a net loss of $18.5 million or $0.14 per share for the second quarter of 2016. Cash, cash equivalents, marketable securities, and interest receivable as of June 30, 2017 were $369.9 million.

"Our focus in early clinical development with ACH-4471 has been on achieving proof-of-concept via factor D inhibition, and we are pleased to report that we believe we have achieved this goal. The emerging interim results from our phase 2 PNH trial have demonstrated a dose response to treatment with what we believe to be meaningful improvements in LDH, hemoglobin, fatigue score and other markers of response. To date, orally administered ACH-4471 has been well tolerated in this PNH clinical trial with four patients enrolled and treated with ACH-4471, two of whom have now received more than four months of dosing," commented Milind S. Deshpande, Ph.D., President and Chief Executive Officer of Achillion. "We believe that inhibition of factor D represents a highly innovative and differentiated mechanism of action with the potential to address multiple diseases of the alternative pathway, including PNH, C3G, IC-MPGN and geographic atrophy, an advanced form of dry age-related macular degeneration."

"I am very excited by the clinical performance of ACH-4471 in the first patients to receive treatment, particularly the improvements in hemoglobin and self reported well-being that have been observed in my patients," commented Peter Browett, M.D., Professor of Pathology, Haematologist, and principal investigator in the ACH-4471 phase 2 study. "The unique mechanism of action via factor D inhibition by ACH-4471 may also be able to control both intravascular breakdown of PNH red blood cells, as well as extravascular hemolysis, leading to improved patient outcomes."

Second Quarter Financial Results

For the three months ended June 30, 2017, Achillion reported a net loss of $22.5 million compared with a net loss of $18.5 million during the same period of 2016. Research and development expenses were $18.3 million for the three months ended June 30, 2017, compared with $14.2 million for the same period of 2016. The increase was primarily due to increased clinical trial costs related to ACH-4471 combined with an increase in manufacturing costs for ACH-5228 and increased discovery research costs related to our ophthalmic factor D inhibitors, partially offset by decreased ACH-4471 manufacturing costs.

For the three months ended June 30, 2017, general and administrative expenses were $5.4 million, compared with $5.2 million incurred during the same period in 2016. The increase was primarily due to increased corporate legal fees and market related consulting fees. These amounts were partially offset by a decrease in corporate taxes.

Non-cash stock compensation expense totaled $2.8 million for the second quarter of 2017 as compared with $2.6 million for the second quarter of 2016 and is included in research and development expenses and general and administrative expenses.

Six Month Financial Results

For the six months ended June 30, 2017, Achillion reported a net loss of $42.7 million, compared to a net loss of $36.6 million in the same period in 2016. For the six months ended June 30, 2017, research and development expenses totaled $33.8 million, compared with $27.4 million during the same period in 2016. The increase was primarily due to increased clinical trial costs related to ACH-4471 combined with an increase in manufacturing costs for ACH-5228 and increased discovery research costs related to our ophthalmic factor D inhibitors, partially offset by decreased ACH-4471

manufacturing costs.

General and administrative expenses were $11.0 million for the six months ended June 30, 2017, increased from $10.6 million in the same period in 2016. The increase was primarily due to increased corporate legal fees and market related consulting fees. These amounts were partially offset by a decrease in corporate taxes.

Non-cash stock compensation expense totaled $6.0 million for the six months ended June 30, 2017 as compared with $5.6 million for the same period in 2016, and is included in both research and development and general and administrative expenses.

The Company expects that research and development expense during the second half of 2017 will increase somewhat, consistent with previous guidance. Annual total research and development expense is expected to be in the range of $75-78 million and annual total general and administrative expense in the range of $22-24 million.

Developing ACH-4471, Complement Factor D Inhibitor for Rare Diseases

- ***Phase 2 clinical trials for untreated paroxysmal nocturnal hemoglobinuria (PNH)***

  In April 2017, Achillion announced the initiation of a phase 2 three-month, dose-ranging trial with ACH-4471 for patients with untreated PNH. The primary objective of the trial is to assess the change-from-baseline in serum lactate dehydrogenase (LDH) levels, a sensitive biomarker for intravascular hemolysis, after dosing with ACH-4471. Secondary endpoints being assessed include changes in hemoglobin, PNH red blood cells, fatigue score (FACIT scale), changes in levels of complement pathway biomarkers such as Bb and factor D, pharmacokinetics, and safety. The protocol allows for intra-patient dose-escalation with patients initially receiving 100 mg or 150 mg of ACH-4471 three times daily with the ability to increase dosage during the treatment period. To date, 200 mg three times daily has been the highest dose administered. After completion of three months of treatment with ACH-4471 and following investigator assessment of safety and clinical benefit, patients may be enrolled into the long-term extension trial. The protocol is designed for enrollment of four to twelve patients.

  To date, Achillion has data for four patients with PNH, two of whom have completed the three-month trial and have entered the long-term extension trial. One additional patient continues to receive dosing in the three-month trial and a fourth patient voluntarily withdrew from the trial on day 41 for reasons unrelated to safety. In summary, interim data from these ongoing trials demonstrated that ACH-4471 achieved clinically meaningful complement inhibition and demonstrated a favorable tolerability profile with no reports of clinically meaningful increases in liver enzymes. In this emerging data set, ACH-4471 has improved LDH, hemoglobin, fatigue score and other measures of response including PNH clone size. These interim results support the Company's global expansion plans for the PNH clinical program.

  "We believe these interim data in four patients are compelling. They confirm the mechanism of action of factor D inhibition, and fully support development of ACH-4471 and our other factor D inhibitors. We look forward to continuing our investigation of ACH-4471 for treatment of PNH," commented Dr. Deshpande.

  PNH is a rare, acquired, life-threatening disease characterized by destruction of red blood cells (hemolytic anemia), blood clots (thrombosis), impaired bone marrow function, and a risk of developing leukemia. Preclinical studies suggest ACH-4471 has a distinct mechanism of action inhibiting factor D within the alternative pathway of the complement cascade leading to blockade of C3 convertase production. Furthermore, unlike C5 inhibitors, ACH-4471 is also thought to prevent C3 fragment deposition on PNH cells and may confer a pharmacological advantage by protecting PNH cells from both intravascular and extravascular hemolysis.

- ***C3 glomerulopathy (C3G)***

  Achillion has an on-going agreement with Imperial College London to conduct a natural history study of C3G, a rare renal disorder which includes dense deposit disease (DDD) and C3 glomerulonephritis (C3GN). This study, conducted by a team of researchers led by Dr. Matthew Pickering and Dr. H. Terry Cook, both of Imperial College, tracks the course of this disease over time. The aim of this study is to collect data on disease progression. Data from this study, which began earlier this year and will run in parallel with other C3G clinical studies, will inform and support product development and approval.

  During the second half of 2017, Achillion anticipates initiating patient dosing in a phase 2 open-label trial of ACH-4471 for patients with low C3 levels due to C3G or immune-complex membranoproliferative glomerulonephritis (IC-MPGN). This 14-day trial is expected to enroll approximately 10 patients.

  "We believe alternative pathway complement inhibition via factor D uniquely may be able to prevent the formation of

C3 fragments, the deposition of which appears to be the underlying cause of this devastating disease," said Dr. Deshpande. "Having just sponsored an externally-led, patient-focused drug development meeting in this disease led by the National Kidney Foundation in which the goal was to gain the C3G patient's perspective on their disease, we are keenly aware that our compounds may have an opportunity to meaningfully benefit patients who suffer from this disease for which there are no currently approved treatments."

Developing ACH-5228, Complement Factor D Inhibitor for Rare Diseases

ACH-5228 is one of Achillion's next-generation factor D inhibitors being developed for oral administration. The compound has demonstrated complete inhibition of the complement alternative pathway after repeat, twice-daily dosing in non-human primates over a seven-day period. The compound also has the following preclinical characteristics based on the Company's research to date:

- *Potency*. ACH-5228 is also specific for factor D inhibition, and had a two to three-fold greater potency than ACH-4471 in preclinical studies, delivering similar inhibition of the complement alternative pathway at inhibitory concentrations of approximately half that of ACH-4471.
- *Pharmacokinetics and Metabolism*. Pharmacokinetic characteristics for ACH-5228 suggest the possibility of once or twice daily dosing frequency.
- *Safety*. Achillion has completed short-term non-clinical studies in rats and dogs in which ACH-5228 demonstrated tolerability and safety margins supportive of progressing into clinical development.

Achillion anticipates initiating a first-in-human phase 1 clinical trial with ACH-5228 by year-end 2017.

Next-generation Factor D Inhibitors for Geographic Atrophy (GA), an Advanced Form of Dry Age-related Macular Degeneration

To date, Achillion has selected several compounds from its factor D inhibitor platform with physicochemical properties that may be advantageous for delivery to the back of the eye for the treatment of GA, a disease with no currently approved therapies, with the goal of achieving treatment intervals of 3 months or longer. Achillion is advancing a number of these compounds in preclinical studies as well as a number of delivery technologies to optimize treatment duration. The Company anticipates selecting one or more lead compounds and delivery technologies by year-end 2017.

Update on World-wide Collaboration with Janssen for Chronic Hepatitis C Viral Infection (HCV)

In May 2015, Achillion announced an exclusive worldwide collaboration with Janssen Pharmaceuticals, Inc. (Janssen), one of the Janssen Pharmaceutical Companies of Johnson & Johnson, for the treatment of HCV. Janssen is currently completing OMEGA-1, a phase 2b, multicenter, randomized, open-label study to investigate the efficacy, safety and pharmacokinetics of different treatment regimens of JNJ-4178, a once-daily combination of AL-335, odalasvir, a compound licensed from Achillion, and simeprevir, in treatment-naive and treatment-experienced subjects with HCV genotype 1, 2, 4, 5, and 6 infection, with and without cirrhosis.

In April 2017, Achillion reported that Janssen's OMEGA-1 global phase 2b clinical trial was fully enrolled with a total of 365 subjects. Results from this trial are anticipated during the second half of 2017.

Webcast and Dial-in Information

Achillion will host a conference call and simultaneous webcast on Tuesday, August, 8, 2017 at 4:05 p.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 8:00 p.m. ET on August 8, 2017, through 7:05 p.m. ET on August 12, 2017 by dialing (855) 859-2056 or (404) 537-3406. The replay passcode is 63792663.

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 Chronic Hepatitis C Viral Infection (HCV)

HCV is one of the most common causes of viral hepatitis, which is an inflammation of the liver. The World Health Organization currently estimates that more than 71 million people are living with HCV worldwide and that each year, 1.75 million people newly acquire HCV. Three-quarters of the HCV patient population is undiagnosed; it is a silent epidemic and a major global health threat. Chronic hepatitis, if left untreated, can lead to permanent liver damage that can result in the development of liver cancer, liver failure or death. Despite available treatments, there remains a significant unmet need for millions of patients globally infected with HCV.

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 pursue best-in-class oral antiviral therapy for HCV and 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, including the potential for its compounds to treat PNH, C3G, IC-MPGN, geographic atrophy and other diseases; the timing for initiation of clinical trials in PNH, C3G and IC-MPGN with ACH-4471, the timing for the first-in-human phase 1 clinical trial with ACH-5528; the timing for interim or final results from Achillion's clinical trials or those of its collaborator; and 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. 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 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, including the current collaboration with Janssen; 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. Furthermore, because Janssen is solely responsible for the development and commercialization of Achillion's HCV assets under the exclusive worldwide license Achillion granted to it and has the deciding vote on all collaboration matters, Janssen generally has full discretion over all development plans and strategies and may not advance the HCV programs in the time frames Achillion or Janssen projects, or at all, including with regard to the current phase 2b combination trials that include Achillion's licensed drug candidates. Moreover, Janssen may not demonstrate in any current and future clinical trials the requisite safety, efficacy and combinability of drug candidates that incorporate Achillion's HCV assets, or obtain and maintain necessary regulatory approvals for such programs. 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.

-- Financial Tables Attached --

ACHILLION PHARMACEUTICALS INC. (ACHN)

Statements of Operations
(Unaudited, in thousands, except per share amounts)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	**2016**	**2017**	**2016**
Revenue	$ -	$ -	$ -	$ -
Operating expenses:				
Research and development	18,253	14,154	33,747	27,433
General and administrative	5,363	5,155	11,011	10,595
Total operating expenses	23,616	19,309	44,758	38,028
Loss from operations	(23,616)	(19,309)	(44,758)	(38,028)
Other income (expense):				
Interest income	1,085	828	2,092	1,507
Interest expense	(12)	(12)	(29)	(26)
Net loss	$ (22,543)	$ (18,493)	$ (42,695)	$ (36,547)
Net loss per share - basic and diluted	$ (0.16)	$ (0.14)	$ (0.31)	$ (0.27)
Weighted average shares outstanding - basic and diluted	136,736	136,680	136,729	136,647

Balance Sheets
(Unaudited, in thousands)

	June 30,	December 31,

	2017	2016
Cash, cash equivalents, marketable securities and interest receivable	$ 369,916	$ 392,486
Working capital	341,136	368,564
Total assets	376,919	413,875
Long-term liabilities	323	450
Total liabilities	14,343	14,421
Total stockholders' equity	362,576	399,454

Investors & Media:
Glenn Schulman, PharmD, MPH
Executive Director, Investor Relations
Achillion Pharmaceuticals, Inc.
Tel. (203) 752-5510
gschulman@achillion.com