August 4, 2016

Achillion Reports Second Quarter 2016 Financial Results

NEW HAVEN, Conn. , Aug. 04, 2016 (GLOBE NEWSWIRE) -- **Achillion Pharmaceuticals, Inc.** (Nasdaq:ACHN) today reported financial results for the three and six months ended June 30, 2016. For the second quarter of 2016, Achillion reported a net loss of $18.5 million or $0.14 per share, compared with a net loss of $29.0 million or $0.25 per share for the second quarter of 2015. Cash, cash equivalents, marketable securities, and interest receivable as of June 30, 2016 were $426.5 million.

"During the second quarter, we continued to advance the clinical development program for ACH-4471, our internally discovered and developed factor D inhibitor candidate for complement-mediated rare diseases, with the initiation of a 14-day multiple-ascending dose study in healthy volunteers," commented Milind Deshpande, Ph.D., President and Chief Executive Officer of Achillion. "We anticipate that the data from the ACH-4471 phase 1 program will be used to select dosing regimens for the phase 2 trials in paroxysmal nocturnal hemoglobinuria (PNH) and C3 glomerulopathy (C3G), both targeted for initiation during the fourth quarter of this year."

Second Quarter Financial Results

For the three months ended June 30, 2016, Achillion reported a net loss of $18.5 million compared with a net loss of $29.0 million during the same period of 2015. During the three months ended June 30, 2016 Achillion did not recognize any revenue, compared to revenue of $711,000 under an agreement with Janssen that was recognized in the same period of 2015.

Research and development expenses were $14.2 million for the three months ended June 30, 2016, compared with $19.8 million for the same period of 2015. The decrease was primarily due to lower manufacturing, clinical trial and consulting costs related to our HCV compounds which were licensed to Janssen in 2015, partially offset by increased manufacturing and clinical costs related to ACH-4471 and increased personnel costs due to the addition of personnel. For the three months ended June 30, 2016, general and administrative expenses were $5.2 million, compared with $10.1 million incurred during the same period in 2015. The decrease was primarily due to lower business consulting and corporate legal fees related to the Janssen agreement, partially offset by increased personnel and non-cash stock compensation largely related to the addition of personnel.

Non-cash stock compensation expense totaled $2.6 million for the second quarter of 2016 as compared with $2.4 million for the second quarter of 2015 and is included in research and development and general and administrative expenses.

Six Month Financial Results

For the six months ended June 30, 2016, Achillion reported a net loss of $36.5 million, compared to a net loss of $48.2 million in the same period in 2015. During the six months ended June 30, 2016 Achillion did not recognize any revenue, compared to revenue of $711,000 under an agreement with Janssen that was recognized in the same period of 2015.

For the six months ended June 30, 2016, research and development expenses totaled $27.4 million, compared with $34.9 million during the same period in 2015. The decrease was primarily due to lower manufacturing, clinical trial and consulting costs related to our HCV compounds which were licensed to Janssen in 2015, partially offset by increased manufacturing and clinical costs related to ACH-4471 and increased personnel costs due to the addition of personnel. General and administrative expenses were $10.6 million for the six months ended June 30, 2016, decreased from $14.4 million in the same period in 2015. The decrease primarily due to lower business consulting and corporate legal fees related to the Janssen agreement, partially offset by increased personnel and non-cash stock compensation largely related to the addition of personnel.

Non-cash stock compensation expense totaled $5.6 million for the six months ended June 30, 2016 as compared with $5.3 million for the same period in 2015, and is included in research and development and general and administrative expenses.

ACH-4471 Phase 1 Clinical Program

- ***Phase 1 Single Ascending Dose Study to Assess the Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of ACH-4471 in Healthy Volunteers***

In June 2016, Achillion announced interim results from its phase 1 single ascending dose clinical trial to evaluate the safety, pharmacokinetics and pharmacodynamics of ACH-4471 in healthy volunteers. Across all four dose groups, ACH-4471 achieved peak plasma concentrations between 1 and 2.5 hours after oral dosing. Up to 100% inhibition of complement activity was achieved in all dose groups, and duration of inhibition was dose dependent. In addition, Group 4, which evaluated 1,200 mg of ACH-4471 given every twelve hours for 2 doses, achieved a median 99.5% inhibition (with a range of 96% to 100%) of hemolysis at 24 hours.

- ***Phase 1 Multiple Ascending Dose Study to Assess the Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of ACH-4471 in Healthy Volunteers***

  During the second quarter of 2016 Achillion initiated enrollment and dosing in a phase 1 multiple ascending dose study with ACH-4471. The study is evaluating up to 14 days of dosing in healthy volunteers. Interim results from this study are expected to be reported during the third quarter of 2016.

Developing ACH-4471 Complement-Based Therapeutics for Rare Diseases

- ***PNH (Paroxysmal Nocturnal Hemoglobinuria)***

  Following the completion of phase 1 studies in healthy volunteers, Achillion plans to initiate phase 2 trials in patients with PNH evaluating both treatment-naïve patients and patients who sub-optimally respond to current therapies. We anticipate interim results in treatment-naïve patients by the end of 2016.

  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 inhibits 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.

- ***C3G (C3 Glomerulopathy)***

  C3G is a rare renal disease which is believed to be the result of over-activity of the alternative pathway. As ACH-4471 has been shown *in vitro* to inhibit alternative pathway activity, potentially decreasing the formation of C3 protein fragments, the company plans to initiate a phase 2 study of ACH-4471 in C3G patients by the end of 2016. Achillion also plans to support a natural history study of C3G with a leading international research institution to add to the understanding of this devastating disease.

  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 transplant 10 years after diagnosis.

About HCV

The hepatitis C virus (HCV) is one of the most common causes of viral hepatitis, which is an inflammation of the liver. It is currently estimated that more than 150 million people are infected with HCV worldwide including more than 5 million people in the United States. 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. Few therapeutic options currently exist for the treatment of HCV infection.

About Complement Factor D Platform

Achillion has leveraged its internal discovery capabilities and a novel complement-related platform to develop drug candidates that are oral inhibitors of complement factor D. Factor D is an essential serine protease involved in the complement pathway, 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 complement plays a critical role. Potential indications being evaluated for these compounds include paroxysmal nocturnal hemoglobinuria (PNH), C3 Glomerulopathy (C3G), dry age-related macular degeneration (dry AMD), and chronic obstructive pulmonary disease (COPD). Achillion anticipates that its platform could play a role in addressing the needs of all PNH patients, including patients who have suboptimal response to, or fail to respond to, the currently available treatments, as well as for patients suffering from other alternative pathway complement-mediated diseases. Achillion nominated ACH-4471 for clinical development in December 2015, and initiated clinical development in February 2016 with a phase 1 healthy volunteer trial assessing single-ascending doses of ACH-4471.

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 chronic hepatitis C (HCV) and build a platform of potent and specific complement inhibitors. 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," "intend," "plan," "aim," "believe," "seek," " estimate," "can," "focus," "will," "look forward," "goal," and "may" and similar expressions to identify such forward-looking statements. These forward-looking statements also include statements about: the Company's expected plans, timing, data readouts and results from ongoing and planned clinical trials of both ACH-4471 and HCV development candidates being advanced by Janssen under the Company's collaboration with Janssen; and statements concerning the Company's strategic goals, milestone 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, 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 our HCV assets under the exclusive worldwide license we 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 and planned phase 2a and phase 2b combination trials that include our licensed drug candidates. 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 March 31, 2016, and its 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,	
	2016	2015	2016	2015
Revenue	$ -	$ 711	$ -	$ 711
Operating expenses:				
Research and development	14,154	19,772	27,433	34,928
General and administrative	5,155	10,127	10,595	14,370
Total operating expenses	19,309	29,899	38,028	49,298
Loss from operations	(19,309)	(29,188)	(38,028)	(48,587)

Other income (expense):				
Interest income	828	225	1,507	377
Interest expense	(12)	(15)	(26)	(31)
Net loss	$ (18,493)	$ (28,978)	$ (36,547)	$ (48,241)
Net loss per share - basic and diluted	$ (0.14)	$ (0.25)	$ (0.27)	$ (0.42)
Weighted average shares outstanding - basic and diluted	136,680	117,770	136,647	114,504

Balance Sheets
(Unaudited, in thousands)

	June 30, 2016	December 31, 2015
Cash, cash equivalents, marketable securities and interest receivable	$ 426,474	$ 460,540
Working capital	416,264	447,930
Total assets	432,355	464,525
Long-term liabilities	114	231
Total liabilities	13,100	14,889
Total stockholders' equity	419,255	449,636

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

Media:
Liz Power
Senior Director, Public Relations
Achillion Pharmaceuticals, Inc.
Tel: (203) 752-5509
lpower@achillion.com