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Achillion Reports Third Quarter 2017 Financial Results and Provides Update on Clinical Programs

Robust balance sheet to support global expansion of ACH-4471 clinical development program in PNH and C3G

NEW HAVEN, Conn., Nov. 01, 2017 (GLOBE NEWSWIRE) -- **Achillion Pharmaceuticals, Inc.** (Nasdaq:ACHN) today reported financial results for the three months ended September 30, 2017. For the third quarter of 2017, Achillion reported a net loss of \$19.3 million or \$0.14 per share, compared with a net loss of \$20.7 million or \$0.15 per share for the third quarter of 2016. Cash, cash equivalents, marketable securities, and interest receivable as of September 30, 2017 were \$353.5 million.

"Throughout 2017, our focus has been on executing on an aggressive global development program aimed at establishing biologic activity and evidence of potential efficacy of factor D inhibition as a novel approach to treating complement-mediated diseases. With the positive interim data reported in August from our phase II clinical trial of ACH-4471 for patients with untreated PNH, we now look forward to reporting initial data from our phase II trial for C3G before the end of the year," commented Milind Deshpande, Ph.D., President and Chief Executive Officer of Achillion. "Over the coming months, the Achillion team plans to launch additional phase II trials of ACH-4471 for PNH, C3G, and IC-MPGN, as well as to evaluate potential extended release formulations of ACH-4471 in phase I by year-end. In parallel, we look forward to advancing ACH-5228, our next-generation factor D inhibitor, into a phase I clinical trial by the end of 2017."

Third Quarter Results

For the three months ended September 30, 2017, Achillion reported a net loss of \$19.3 million compared with a net loss of \$20.7 million during the same period of 2016.

Research and development expenses were \$15.6 million for the three months ended September 30, 2017, compared with \$16.7 million for the same period of 2016. The decrease for the three months ended September 30, 2017 was primarily due to decreased preclinical and manufacturing costs related to ACH-4471, as well as decreased legal fees. These amounts were partially offset by increased clinical trial costs related to ACH-4471 and preclinical costs related to ACH-5228.

General and administrative expenses were \$4.8 million for the three months ended September 30, 2017, and \$4.8 million during the same period in 2016.

Non-cash stock compensation expense totaled \$2.4 million for the third quarter of 2017 as compared with \$2.7 million for the third quarter of 2016 and is included in research and development and general and administrative expenses.

Dr. Deshpande further commented, "In October, Janssen reported that 98.9% of non-cirrhotic patients with HCV genotypes 1, 2, 4, 5 and 6 achieved SVR12 after receiving just six weeks of once daily treatment JNJ-4178, the triple combination of odalasvir, simeprevir, and AL-335, in the OMEGA-1 phase IIb clinical trial. In light of the efficacy demonstrated in OMEGA-1, we remain disappointed that the JNJ-4178 regimen is no longer being advanced and we continue to believe that odalasvir is a best-in-class NS5A inhibitor that demonstrates the strength of Achillion's internal discovery capabilities."

Nine Month Results

For the nine months ended September 30, 2017, Achillion reported a net loss of \$62.0 million, compared to a net loss of \$57.3 million in the same period in 2016.

Research and development expenses were \$49.4 million and \$44.1 million for the nine months ended September 30, 2017 and 2016, respectively. The increase for the nine months ended September 30, 2017 was primarily due to increased clinical trial costs related to ACH-4471 combined with increased preclinical and manufacturing costs for ACH-5228.

General and administrative expenses in the period ending September 30, 2017 were \$15.9 million, compared with \$15.4 million incurred during the same period in 2016. The increase for the nine months ended September 30, 2017 was primarily due to increased marketing-related consulting fees, corporate legal fees, and software license fees, partially offset by a

decrease in corporate taxes.

Non-cash stock compensation expense totaled \$8.4 million for the nine months ended September 30, 2017 as compared with \$8.3 million for the same period in 2016, and is included in research and development and general and administrative expenses.

Complement Factor D Platform

"ACH-4471 is the first factor D inhibitor to show evidence of complement alternative pathway inhibition in humans after oral dosing," said Dr. Deshpande. "Through pioneering research in complement biology, and data emerging from the ACH-4471 clinical program, we are gaining important insights into PK/PD relationships as well as *in vivo* biomarkers to guide development in patients. Upcoming presentations at ASH, we believe, will highlight important research with ACH-4471, complement biology, and the potential of factor D inhibition."

ACH-4471: Expanding Clinical Development Program

"In the United States, we currently have open INDs for PNH and C3G, and are actively working to expand our clinical trial footprint beyond our ongoing trials in Australia and New Zealand into Europe," stated Dr. David Apelian, M.D., Ph.D., M.B.A., Executive Vice President and Chief Medical Officer at Achillion. "As a first-in-class oral factor D inhibitor, we believe we are making great strides in understanding the role of AP modulation by ACH-4471 and the potential for this approach in patients with PNH and for patients with either C3G or IC-MPGN."

PNH: Phase II trials of ACH-4471 monotherapy for untreated PNH

A phase II clinical trial evaluating ACH-4471 monotherapy for patients with untreated PNH remains open with a target enrollment of 12 patients anticipated. To date, four PNH patients have been treated, three of whom have completed the initial three-month trial (-100 study) and have entered the long-term extension trial (-103 study). As previously reported in August 2017, a fourth patient voluntarily withdrew from the trial on day 41 for reasons unrelated to safety or efficacy. Interim data from this first-in-patient trial indicated that ACH-4471 mechanistically inhibited factor D, its intended target, and meaningfully improved LDH, hemoglobin, fatigue score, and other measures of response, including PNH clone size. Updated interim results are expected to be reported before the end of the year.

(Clintrials.gov: NCT03053102/EudraCT: 2016-002652-25; and Clintrials.gov: NCT03181633/EudraCT: 2017-000665-79)

PNH: Phase II trial add-on trial of ACH-4471 for partial responders

During the first half of 2018, Achillion expects to begin enrolling up to 12 PNH patients in a phase II trial evaluating ACH-4471 in PNH patients currently receiving eculizumab, a therapy for PNH that is marketed by a third-party, and who remain transfusion-dependent. This trial is designed to evaluate 6 months of treatment with ACH-4471 plus eculizumab with the potential for patients to transition to a long-term treatment extension.

C3G / IC-MPGN: Phase II 14-day proof-of-mechanism trial of ACH-4471

In September 2017, Achillion initiated patient enrollment in a phase II clinical trial of ACH-4471 for patients with either C3G or IC-MPGN. This trial is designed to evaluate the effect of factor D inhibition on the complement pathway and will enroll up to 10 patients. Measurement of complement proteins, including C3, a complement protein in blood plasma that is typically low in C3G and IC-MPGN patients, and C3 fragments, which are typically high in these patients, will be evaluated. Other measures of kidney function, safety, and tolerability are also being assessed. Per the protocol design, two patients have been enrolled into Group 1 and treated with 100 mg three times daily (TID) for 14 days. Up to eight additional patients are targeted for enrollment into Group 2. Interim results from Group 1 are expected to be reported by year-end 2017.

(Clintrials.gov: NCT03124368 /EudraCT: 2016-003525-42)

C3G / IC-MPGN: Phase II 12-month open-label trial of ACH-4471

Achillion also plans to conduct a phase II open-label, 12-month treatment trial for patients with biopsy-confirmed C3G or IC-MPGN. All patients enrolled will receive treatment with ACH-4471 with periodic assessment of clinical endpoints including proteinuria and estimated glomerular filtration rate (eGFR). Up to 20 patients are expected to be enrolled. Enrollment is expected to be initiated in the first half of 2018.

C3G: Phase II 6-month randomized, placebo-controlled trial of ACH-4471

During the first half of 2018, Achillion plans to initiate a randomized, placebo-controlled, double-blinded phase II trial evaluating the efficacy and safety of ACH-4471 in patients with C3G. The trial is expected to assess renal biopsy findings and clinical endpoints such as proteinuria and eGFR. Up to twenty patients are expected to be enrolled.

Extended Release: Phase I bioavailability trial

By the end of 2017, Achillion plans to initiate a phase I trial evaluating potential extended release formulations of ACH-4471 in healthy volunteers.

ACH-5228: Next-generation Oral Factor D Inhibitor

ACH-5228 is a next-generation oral factor D inhibitor that is expected to enter phase I clinical development by the end of 2017. In preclinical studies, ACH-5228 had a two- to three-fold increase in potency and improved pharmacokinetic characteristics relative to ACH-4471.

Next-generation Factor D Inhibitors for Ophthalmology

Achillion has selected several compounds from its discovery library for properties that may be advantageous for delivery of a factor D inhibitor to the back of the eye for the potential treatment of geographic atrophy (GA) with the goal of achieving treatment duration of three months or longer. Achillion is advancing several of these compounds in preclinical studies, as well as several delivery technologies, to optimize treatment duration, and anticipates selecting one or more lead compounds and delivery technologies during the first half of 2018.

About the Achillion Complement Factor D Platform

Achillion has leveraged its internal discovery capabilities and a novel complement-related platform to develop small molecule 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 alternative pathway plays a critical role. Potential indications being evaluated for these compounds include paroxysmal nocturnal hemoglobinuria (PNH), C3 glomerulopathy (C3G), immune complex-mediated membranoproliferative glomerulonephritis (IC-MPGN), and geographic atrophy (GA).

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," "target," "intend," "plan," "aim," "believe," "seek," "estimate," "can," "could" "focus," "will," "look forward," "goal," and "may" and similar expressions to identify such forward-looking statements. These forward-looking statements also include statements about: Achillion's expected plans, timing, data readouts and results from ongoing and planned clinical trials of both ACH-4471 and ACH-5228; the planned advancement of Achillion's other small molecule factor D inhibitors, including those for the treatment of geographic atrophy; and statements concerning Achillion'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; 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 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.

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-- Financial Tables Attached --

ACHILLION PHARMACEUTICALS INC. (ACHN)

Statements of Operations

(Unaudited, in thousands, except per share amounts)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	<u>2017</u>	<u>2016</u>	<u>2017</u>	<u>2016</u>
Revenue	\$ -	\$ -	\$ -	\$ -
Operating expenses:				
Research and development	15,620	16,701	49,368	44,133
General and administrative	4,843	4,848	15,853	15,443
Total operating expenses	20,463	21,549	65,221	59,576
Loss from operations	(20,463)	(21,549)	(65,221)	(59,576)
Other income (expense):				
Interest income	1,133	846	3,225	2,353
Interest expense	(8)	(27)	(37)	(54)
Other Income	-	-	-	-
Net loss	<u>\$ (19,338)</u>	<u>\$ (20,730)</u>	<u>\$ (62,033)</u>	<u>\$ (57,277)</u>
Net loss per share - basic and diluted	<u>\$ (0.14)</u>	<u>\$ (0.15)</u>	<u>\$ (0.45)</u>	<u>\$ (0.42)</u>
Weighted average shares outstanding - basic and diluted	<u>137,375</u>	<u>136,681</u>	<u>136,947</u>	<u>136,647</u>

Balance Sheets

(Unaudited, in thousands)

September 30, 2017	December 31, 2016
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Cash, cash equivalents, marketable securities and interest receivable	\$ 353,499	\$ 392,486
Working capital	312,570	368,564
Total assets	360,442	413,875
Long-term liabilities	269	450
Total liabilities	14,705	14,421
Total stockholders' equity	345,737	399,454