November 3, 2016

Achillion Reports Third Quarter 2016 Financial Results And Provides Update On Clinical Programs

- *Janssen HCV triple combination candidate JNJ-4178 reported 100% SVR12 after 6 weeks of therapy; global phase IIB study to begin 4Q 2016*
- *Achillion's phase I MAD study with oral factor D inhibitor ACH-4471 ongoing; strong complement inhibition observed in in vivo and ex vivo assays*
- *Company advancing small molecule candidates for the treatment of dry AMD*

NEW HAVEN, Conn., Nov. 03, 2016 (GLOBE NEWSWIRE) -- **Achillion Pharmaceuticals, Inc.** (Nasdaq:ACHN) today reported financial results for the three months ended September 30, 2016. For the third quarter of 2016, Achillion reported a net loss of $20.7 million or $0.15 per share, compared with net income of $26.3 million or $0.19 per share for the third quarter of 2015. Cash, cash equivalents, marketable securities, and interest receivable as of September 30, 2016 were $409.5 million.

"We continue to advance ACH-4471 and our platform of small molecule factor D inhibitors," commented Milind Deshpande, Ph.D., President and Chief Executive Officer of Achillion. "Our strong balance sheet, together with our collaboration with Janssen who is developing JNJ-4178, a next-generation short-duration treatment regimen for HCV, enables us to focus on the discovery and development of what could be a game-changing approach to modulating diseases of the complement alternative pathway."

Third Quarter Results

For the three months ended September 30, 2016, Achillion reported a net loss of $20.7 million compared with net income of $26.3 million during the same period of 2015.

During the third quarter of 2015, Achillion and Janssen Pharmaceuticals, Inc. (Janssen), one of the Janssen Pharmaceutical Companies of Johnson & Johnson, completed the closing of the collaboration providing Janssen with an exclusive, worldwide license to develop and, upon regulatory approval, commercialize HCV products and regimens containing one or more of Achillion's HCV assets. Upon closing in 2015, Achillion received $225 million from Johnson & Johnson Innovation — JJDC, Inc. following the issuance of 18,367,346 shares of Achillion at a price of $12.25 per share.

Achillion recognized in the third quarter of 2015 revenue of $33.8 million under the Janssen Agreement, representing a portion of the premium paid by JJDC. No revenue was recognized during the three months ended September 30, 2016.

Research and development expenses were $16.7 million for the three months ended September 30, 2016, compared with $12.0 million for the same period of 2015. The increase for the three months ended September 30, 2016 was primarily due to increased manufacturing; clinical trial and consulting costs related to ACH-4471, partially offset by decreased manufacturing, clinical trial and consulting costs related to our HCV compounds which were licensed to Janssen in 2015.

General and administrative expenses were $4.8 million for the three months ended September 30, 2016, compared with $4.9 million incurred during the same period in 2015. The decrease for the three months ended September 30, 2016 was primarily due to decreased business development consulting fees and corporate legal fees related to the Janssen Agreement, partially offset by increased personnel and non-cash stock costs largely related to the addition of personnel.

Nine Month Results

For the nine months ended September 30, 2016, Achillion reported a net loss of $57.3 million, compared to a net loss of $22.0 million in the same period in 2015. During the nine months ended September 30, 2015, Achillion recognized revenue of $34.5 million under the Janssen Agreement, representing a portion of the premium paid by JJDC.

Research and development expenses were $44.1 million and $46.9 million for the nine months ended September 30, 2016 and 2015, respectively. The decrease for the nine months ended September 30, 2016 was primarily due to decreased manufacturing, clinical trial and consulting costs related to our HCV compounds, which were licensed to Janssen in 2015, partially offset by increased manufacturing, clinical trial and consulting costs related to ACH-4471. Personnel costs also increased due to the addition of personnel in our discovery and development groups.

General and administrative expenses in the period ending September 30, 2016 were $15.4 million, compared with $19.2 million incurred during the same period in 2015. The decrease for the nine months ended September 30, 2016 was primarily due to decreased consulting fees and corporate legal fees related to the Janssen Agreement, partially offset by increased personnel and non-cash stock compensation costs largely related to the addition of personnel.

Complement Factor D Platform

"ACH-4471 is the first factor D inhibitor to demonstrate 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 will highlight groundbreaking research with ACH-4471 regarding complement biology and factor D inhibition."

Phase I Clinical Program

In June 2016, Achillion initiated enrollment and dosing in an ongoing phase I multiple ascending dose (MAD) study of ACH-4471 in healthy volunteers. The goal of the MAD study is to evaluate safety and tolerability as well as to optimize the PK/PD profile and dosing regimens for further development in patients.

In the three dose cohorts completed to date, strong complement inhibition using multiple *in vivo* as well as *ex vivo* assays was observed. The plasma trough concentrations of ACH-4471 exceeded the range the Company anticipates for potential treatment of patients. These current projections are based on emerging data including the benchmarking of ACH-4471 with eculizumab for inhibition of lysis of PNH red blood cells and the PK/PD profile of ACH-4471 from the phase I program. In the ongoing MAD study, Achillion is planning to assess additional dosing regimens to maintain the projected effective trough concentrations and safety of ACH-4471.

To date in the MAD study, ACH-4471 has been generally well tolerated across three dose cohorts (200 mg, 500 mg or 800 mg given every 12 hours) with no treatment-related SAEs reported. Two cases of self-limited, ALT elevations (Grade 3 and 4) were observed post-treatment in the mid- and high dose groups, respectively, with neither subject exhibiting signs or symptoms of hepatic decompensation. Both subjects' ALT levels normalized without intervention during follow up. Further, no treatment-associated fever or infections were observed.
Achillion anticipates announcing interim results from this phase I study in the first half of 2017.

Upcoming Presentations at the 2016 Meeting of the American Society of Hematology

Today, the American Society of Hematology announced the titles of two ACH-4471 related posters to be presented at the 58th annual meeting in San Diego on December 3-6, 2016.

- ‘Effect of complement inhibition by anti-C5 (eculizumab) or a small molecule inhibitor of Factor D (ACH-4471) on survival of meningococci in blood from vaccinated adults.' These data will be presented by Dr. Dan M. Granoff, UCSF Benioff Children's Hospital, a world expert on meningococcal infections.

- ‘Evaluation of bacteria-mediated potential "Bystander" hemolysis of PNH red cells in vitro: No evidence of significant complement classical or lectin pathway-mediated hemolysis induced by microorganisms.' These data will be presented by Dr. Xuan Yuan from the laboratory of Dr. Robert Brodsky, Johns Hopkins University, a leading PNH expert.

In addition, Achillion recently presented a poster at the XXVIth International Complement Workshop in Kanazawa, Ishikawa, Japan, demonstrating that ACH-4471 does not affect bacterial killing via lysis or opsonophagocytosis using *E. coli* as a test pathogen.

These data expand Achillion's understanding of complement biology and demonstrate the potential advantages of inhibition of factor D in treatment of complement-mediated diseases.

Ophthalmology

Achillion's leading research in medicinal chemistry and structural biology has led to the development of factor D inhibitors which also have properties appropriate for ocular delivery. The company is now advancing small molecule candidates with characteristics appropriate for treatment of dry AMD. Preclinical studies have demonstrated ocular safety and desirable tissue distribution. Achillion is working on approaches that could be used to deliver its small molecule factor D inhibitors to the eye less frequently than other treatments in development.

Janssen Collaboration

In September 2016, Achillion announced positive interim results from the Janssen-sponsored 604 phase IIa clinical trial of the triple combination of simeprevir, AL-335 and odalasvir, the combination now referred to as JNJ-4178, which achieved 100 percent SVR12, or sustained viral response 12 weeks after the completion of treatment, for both 8-week and 6-week treatment durations in HCV genotype 1 infected patients . Based on these positive results, Janssen plans to advance the triple combination in a phase IIb clinical study (OMEGA-1) in HCV patients along with the expanded 604 study. Janssen recently announced that enrollment in the phase IIb study is targeted to begin in the fourth quarter of 2016.

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), and dry age-related macular degeneration (dry AMD).

About HCV

Globally, HCV infection is a leading cause of liver disease and liver related mortality. It is currently estimated that more than 150 million people are infected with HCV worldwide including approximately 3 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. Despite available treatments, there remains a significant unmet need for many patients 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 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 HCV development candidates being advanced by Janssen under Achillion's collaboration with Janssen; the planned advancement of Achillion's other small molecule factor D inhibitors, including those for the treatment of dry AMD; 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, 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 planned phase IIb combination trial that include Achillion's 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 June 30, 2016, and its subsequent SEC filings.

-- 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,	
	2016	**2015**	**2016**	**2015**
Revenue	$ -	$ 33,820	$ -	$ 34,531
Operating expenses:				
Research and development	16,701	11,983	44,133	46,912
General and administrative	4,848	4,856	15,443	19,226
Total operating expenses	21,549	16,839	59,576	66,138
Loss from operations	(21,549)	16,981	(59,576)	(31,607)
Other income (expense):				
Interest income	846	346	2,353	723
Interest expense	(27)	(12)	(54)	(42)
Other Income	-	8,944	-	8,944
NetIncome (loss)	$ (20,730)	$ 26,259	$ (57,277)	$ (21,982)
Net income (loss) per share - basic	$ (0.15)	$ 0.19	$ (0.42)	$ (0.18)
Net income (loss) per share - diluted	$ (0.15)	$ 0.19	$ (0.42)	$ (0.18)
Weighted average shares outstanding - basic	136,681	136,439	136,647	121,896
Weighted average shares outstanding - diluted	136,681	140,024	136,647	121,896

Balance Sheets
(Unaudited, in thousands)

	September 30, 2016	December 31, 2015
Cash, cash equivalents, marketable securities and interest and subscriptions receivable	$ 409,550	$ 460,540
Working capital	398,082	447,930
Total assets	415,193	464,525
Long-term liabilities	221	231
Total liabilities	13,982	14,889
Total stockholders' equity	401,211	449,636

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