May 3, 2016

Achillion Reports First Quarter 2016 Financial Results and Provides Update on Clinical Programs

- Company provides update on HCV collaboration with Janssen; Phase 2b combination study evaluating doublet and triplet regimens for six and eight weeks anticipated to begin in Q3 2016 —

- Strong balance sheet to support planned 2016 clinical expansion of ACH-4471, Achillion's first small molecule factor D inhibitor, for the potential treatment of PNH and C3G -

NEW HAVEN, Conn., May 03, 2016 (GLOBE NEWSWIRE) -- **Achillion Pharmaceuticals, Inc.** (Nasdaq:ACHN) today reported financial results for the three months ended March 31, 2016. For the first quarter of 2016, the Company reported a net loss of $18.1 million or $0.13 per share, compared with a net loss of $19.3 million or $0.17 per share for the first quarter of 2015. Cash, cash equivalents, marketable securities and interest receivable as of March 31, 2016 were $444 million.

"In the first quarter of 2016, we continued to progress our complement pipeline and to work with Janssen as they advance our combined HCV pipeline under our global collaboration," said Milind Deshpande, Ph.D., President and Chief Executive Officer of Achillion. "By the end of 2016, Achillion expects to report a number of data points from both the HCV program and our ongoing phase 1 trial with ACH-4471, our first orally-administered, small-molecule complement factor D inhibitor drug candidate being developed for rare diseases, including PNH and C3G."

First Quarter 2016 Results

For the first quarter of 2016, the Company reported a net loss of $18.1 million, or $0.13 per share, compared with a net loss of $19.3 million, or $0.17 per share for the first quarter of 2015. Cash, cash equivalents, marketable securities, and interest receivable as of March 31, 2016 were $444 million.

Research and development expenses were $13.3 million in the first quarter of 2016, compared with $15.2 million for the same period of 2015. The decrease was primarily due to decreased manufacturing, clinical trial and consulting costs related to our HCV compounds which were licensed to Janssen in 2015, offset by increased manufacturing and clinical costs related to ACH-4471. Additionally, personnel costs increased due to the addition of personnel in our discovery and development groups.

For the three months ended March 31, 2016, general and administrative expenses totaled $5.4 million, compared to $4.2 million for the same period in 2015, with the increase primarily due to increased salaries and non-cash stock compensation charges due to the addition of personnel.

Non-cash stock compensation expense totaled $3.0 million for the first quarter of 2016 as compared with $2.9 million for the first quarter of 2015, and is included in research and development and general and administrative expenses.

Update on world-wide collaboration with Janssen for 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 chronic hepatitis C viral infection (HCV).

"Our collaboration with Janssen creates a combined pipeline that has the potential to provide more effective and shorter duration regimens for the treatment of HCV," said Dr. Deshpande. "In the coming months, we anticipate release of data on both doublet and triplet treatment regimens from Janssen's ongoing phase 2a clinical trial."

Update of HCV Clinical Program

- ***Phase 2a randomized, open-label study to evaluate the safety, pharmacokinetics and efficacy of the combination of AL-335, odalasvir (ACH-3102), and simeprevir in treatment-naïve subjects with genotype 1 chronic hepatitis C***

  The Janssen sponsored phase 2a clinical trial evaluating all-oral regimens for durations of eight weeks and less

remains ongoing. This trial is evaluating a triplet regimen, consisting of odalasvir, AL-335, and simeprevir, for durations of eight and six weeks, as well as a doublet regimen, consisting of odalasvir and AL-335, for eight weeks. Top line results from this study are expected to be released in the third quarter of the year.

- ***Phase 2b, multicenter, randomized, open-label study to investigate the efficacy, safety and pharmacokinetics of different treatment regimens of AL-335, odalasvir, and simeprevir in treatment-naive and treatment-experienced subjects with chronic hepatitis C virus genotype 1, 2, 3, 4, 5, and 6 infection, with and without cirrhosis***

  Janssen's planned global phase 2b clinical trial is expected to begin enrolling patients in the third quarter of 2016. This phase 2b clinical trial is designed to evaluate two regimens, a triplet (odalasvir, AL-335, and simeprevir) regimen and a doublet regimen (odalasvir and AL-335), for treatment durations of six and eight weeks in treatment-naïve and treatment-experienced patients with genotypes 1 - 6 HCV, including those patients with or without cirrhosis This trial is expected to enroll approximately 400 patients.

Status of Complement Factor D Inhibitor Program: Developing ACH-4471 for Rare Diseases

Dr. Deshpande further commented, "We have synthesized more than 1,300 small molecule factor D inhibitor compounds to date and continue to make significant progress with our complement factor D program. This past February, we advanced ACH-4471 into a phase 1 study in healthy volunteers, and are working to advance ACH-4471 as a potential treatment for two serious and rare diseases, PNH and C3G."

Summary of ACH-4471 Clinical Program

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

  During the first quarter of 2016, Achillion initiated a first-in-human phase 1 clinical trial to evaluate the safety, pharmacokinetics and pharmacodynamics of ACH-4471, the Company's first orally-administered, small molecule inhibitor of complement factor D. Interim results from this study are expected to be reported during the second quarter of 2016.

- ***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 anticipates starting a phase 1 multiple ascending dose study with ACH-4471 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 Annual Report on Form 10-K for the fiscal year ended December 31, 2015, 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 March 31,	
	2016	**2015**
Revenue	$ -	$ -
Operating expenses:		
Research and development	13,278	15,156
General and administrative	5,440	4,243
Total operating expenses	18,718	19,399
Loss from operations	(18,718)	(19,399)
Other income (expense):		
Interest income	679	152
Interest expense	(15)	(16)
Net loss	$ (18,054)	$ (19,263)
Net loss per share - basic and diluted	$ (0.13)	$ (0.17)
Weighted average shares outstanding - basic and diluted	136,640	111,202

Balance Sheets
(Unaudited, in thousands)

	March 31, 2016	**December 31, 2015**
Cash, cash equivalents, marketable securities and interest receivable	$ 444,033	$ 460,540
Working capital	429,578	447,930
Total assets	448,656	464,525
Long-term liabilities	325	231
Total liabilities	13,788	14,889
Total stockholders' (deficit) equity	434,868	449,636

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