May 4, 2017

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

Robust balance sheet to support planned Phase 2 clinical development program for ACH-4471, Achillion's first small molecule factor D inhibitor, for the potential treatment of PNH and C3G

NEW HAVEN, Conn., May 04, 2017 (GLOBE NEWSWIRE) -- **Achillion Pharmaceuticals, Inc.** (Nasdaq:ACHN) today reported financial results for the three months ended March 31, 2017. For the first quarter of 2017, the Company reported a net loss of $20.2 million or $0.15 per share, compared with a net loss of $18.1 million or $0.13 per share for the first quarter of 2016. Cash, cash equivalents, marketable securities and interest receivable as of March 31, 2017 were $386.6 million.

"During the first quarter of 2017, we advanced our first, orally administered factor D inhibitor, ACH-4471, into Phase 2 development for PNH. As we continue to enroll patients into this study, the first two PNH patients have now completed 28 days of dosing. After assessment of safety and efficacy by the investigators both patients continue to receive up to two additional months of therapy. Our plan is to submit interim results from this study for presentation at a major medical conference this quarter," commented Milind Deshpande, Ph.D., President and CEO of Achillion.

Dr. Deshpande further stated, "With Achillion's robust balance sheet, the ongoing worldwide HCV collaboration with Janssen, who has fully enrolled patients into their Phase 2b OMEGA-1 clinical trial, and the emerging results from our complement factor D inhibitor program, I believe Achillion is well positioned to achieve its goal of advancing novel therapies that can address significant unmet needs for patients around the globe."

First Quarter 2017 Results

For the first quarter of 2017, the Company reported a net loss of $20.2 million, or $0.15 per share, compared with a net loss of $18.1 million, or $0.13 per share for the first quarter of 2016. Cash, cash equivalents, marketable securities, and interest receivable as of March 31, 2017 were $386.6 million.

Research and development expenses were $15.5 million in the first quarter of 2017, compared with $13.3 million for the same period of 2016. The increase was primarily due to increased clinical trial and consulting costs related to ACH-4471combined with increased preclinical and manufacturing costs related to the Company's next generation factor D inhibitor compounds.

For the three months ended March 31, 2017, general and administrative expenses totaled $5.7 million, compared to $5.4 million for the same period in 2016, with the increase primarily due to increased corporate legal fees and consulting fees.

Non-cash stock compensation expense totaled $3.2 million for the first quarter of 2017 as compared to $3.0 million for the first quarter of 2016, and is included in research and development and general and administrative expenses.

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

Dr. Deshpande further commented, "As our complement factor D inhibitor platform continues to grow, with nearly 2,000 small molecule compounds synthesized to date, and our broad IP strategy, we have continued to make significant advancements in complement biology and research, all of which supports our ongoing clinical development program evaluating factor D inhibition by ACH-4471 for the potential treatment of PNH and C3G."

Developing ACH-4471 Complement Factor D Inhibitor for Rare Diseases

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

  In April 2017, Achillion announced the initiation of a phase 2 trial with ACH-4471 for patients with untreated PNH. The primary objective of the study is to assess the change-from-baseline in serum lactate dehydrogenase (LDH) levels, a sensitive biomarker for intravascular hemolysis, during 28 days of dosing. The protocol allows for intra-patient dose-escalation with patients initially receiving 100 mg three times daily of ACH-4471 with the ability to increase dosage during the treatment period. Secondary endpoints being assessed include changes in hemoglobin and red blood cell

levels, complement pathway biomarkers, such as Bb and factor D, levels, pharmacokinetics, and safety.

Enrollment of patients into this trial in ongoing. Two patients have completed 28 days of dosing and each continues to receive longer term treatment, for up to an additional two months with ACH-4471, under the protocol. Interim results are anticipated during the second quarter.

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.

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

  In February 2017, Achillion announced that it has entered into an agreement with Imperial College London to conduct a natural history study of C3 glomerulopathy (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 a disease over time. The aim of such studies is to collect data on disease progression which can inform and support product development and approval.

  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 during the second half of 2017.

  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.

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).

Update of HCV Clinical Program

- ***'604 Study' Phase 2 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, with additional results presented in April 2017. This trial supports the evaluation of a triplet regimen, JNJ-4178, consisting of odalasvir, AL-335, and simeprevir, for durations of eight and six weeks Initiated in October 2015, this phase 2a clinical trial demonstrated JNJ-4178 was highly effective in treatment-naïve patients with HCV genotype 1 infection, achieving 100% sustained viral response 24 weeks after the completion of treatment, or SVR24, for treatment durations of both 6 and 8 weeks. Janssen is continuing to evaluate the short-duration triple regimen for the treatment of HCV genotypes 1, 2, 4, 5, or 6.

- ***OMEGA-1 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, 4, 5, and 6 infection, with and without cirrhosis***

  In April 2017, we 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.

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), and geographic atrophy (GA), an advanced form of age-related macular degeneration (AMD).

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). Complement 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 Chronic Hepatitis C Viral Infection

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 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 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 and other diseases; the timing for interim results from the Phase 2 study of ACH-4771 in PNH; and statements concerning Achillion's ability to achieve its strategic goals, and statements concerning its plans, and prospects, including those relating to ACH-4771 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 and planned 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 Annual Report on Form 10-K for the fiscal year ended December 31, 2016, 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 March 31,	
	2017	**2016**
Revenue	$ -	$ -
Operating expenses:		
Research and development	15,495	13,278
General and administrative	5,648	5,440
Total operating expenses	21,143	18,718
Loss from operations	(21,143)	(18,718)
Other income (expense):		
Interest income	1,008	679
Interest expense	(17)	(15)
Net loss	$ (20,152)	$ (18,054)
Net loss per share - basic and diluted	$ (0.15)	$ (0.13)
Weighted average shares outstanding - basic and diluted	136,722	136,640

Balance Sheets
(Unaudited, in thousands)

	March 31, 2017	December 31, 2016
Cash, cash equivalents, marketable securities and interest receivable	$ 386,565	$ 392,486
Working capital	372,795	368,564
Total assets	393,353	413,875
Long-term liabilities	376	450
Total liabilities	11,007	14,421
Total stockholders' (deficit) equity	382,346	399,454

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