



November 4, 2014

Achillion Reports Third Quarter and Nine Month 2014 Financial Results

- Hepatitis C development program remains on track to initiate all-oral ribavirin-free regimens with ACH-3422, ACH-3102 and sovalprevir for HCV in 2015 -

- Advancement of novel platform for complement factor D inhibitors for the oral treatment of immune-related rare diseases -

NEW HAVEN, Conn., Nov. 4, 2014 (GLOBE NEWSWIRE) -- **Achillion Pharmaceuticals, Inc.** (Nasdaq:ACHN) today reported financial results for the three and nine months ended September 30, 2014, outlined upcoming milestones in its development programs for chronic hepatitis C viral infection (HCV), and introduced a novel platform for advancing oral complement factor D inhibitors.

For the third quarter of 2014, Achillion reported a net loss of \$15.7 million or \$0.16 per share, compared with a net loss of \$13.9 million or \$0.14 per share for the third quarter of 2013. Cash, cash equivalents, marketable securities, and interest receivable as of September 30, 2014 were \$127.1 million.

Hepatitis C Development Program

During the remainder of 2014, Achillion expects to achieve the following milestones in its HCV development program:

- 1 Present Phase 2 SVR12 results following 8-weeks of treatment with the interferon-free, ribavirin-free regimen of ACH-3102, a second-generation NS5A inhibitor, and sofosbuvir in patients with treatment-naïve genotype 1 HCV. These results will be presented in a late breaker poster presentation and made available in a related press release along with development updates at The Liver Meeting 2014 (AASLD) which begins Saturday, November 8, 2014 in Boston, MA
- 1 Present three posters on ACH-3422, a uridine-analog nucleotide NS5B polymerase inhibitor prodrug, at AASLD that will detail the preclinical profile of this Phase 1 direct-acting antiviral agent for HCV; and
- 1 Report Phase 1 proof-of-concept results with ACH-3422 including safety following 14-day exposure in healthy volunteers and antiviral activity on treatment-naïve genotype 1 HCV patients.

"Over the course of 2014, we executed on our global HCV development plan and achieved several important milestones including the advancement of ACH-3422 into a Phase 1 trial, working with the FDA to remove the clinical hold on sovalprevir, our Phase 2 protease inhibitor, and continuing to demonstrate that, based upon results from three Phase 2 trials, ACH-3102 is a clearly differentiated NS5A inhibitor," commented David Apelian, M.D., Ph.D., Executive Vice President and Chief Medical Officer at Achillion. "As previously announced, we expect to report top-line results from our ongoing Phase 1 trial of ACH-3422 later this quarter and look forward to initiating a proprietary combination program evaluating ACH-3422, ACH-3102 and sovalprevir during 2015."

Complement Factor D Inhibitor Platform

Achillion also announced today that the Company has leveraged its internal discovery capabilities and a novel complement-related platform to develop 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. The new complement platform is focused on advancing compounds that inhibit factor D, can be orally-administered, and potentially can 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), atypical hemolytic uremic syndrome (aHUS), myasthenia gravis, and age-related macular degeneration (AMD). Achillion anticipates that its platform could play a role to addressing the needs of all PNH patients, including patients who have suboptimal response to, or fail to respond to, the currently available treatment, as well as for patients suffering from other complement-mediated diseases.

Milind Deshpande, Ph.D., President and Chief Executive Officer of Achillion commented, "I am very proud of our accomplishments and we remain focused on transforming innovation into treatments that improve patients' lives. With our HCV compounds progressing through clinical development and on track to deliver additional results during the remainder of 2014, the Achillion discovery team has been focused on identifying and advancing exciting new candidates into our pipeline.

We have now generated a portfolio of small molecule compounds that will be evaluated as orally administered inhibitors of complement factor D, potentially offering novel treatment options for patients with complement-related disease."

Dr. Deshpande continued, "Initial in vitro and in vivo data that we have generated with complement factor D inhibitors have been accepted for presentation at the 2014 American Society of Hematology (ASH) annual meeting next month and the abstracts will be released later this week. We currently expect that the first compound from this platform will enter clinical development before the 2015 ASH annual meeting and look forward to providing additional updates on this platform shortly."

Third Quarter Results

For the three months ended September 30, 2014, Achillion reported a net loss of \$15.7 million compared with a net loss of \$13.9 million during the same period of 2013. The Company recognized no revenues for the three months ended September 30, 2014 nor any during the same period in 2013.

Research and development expenses were \$12.1 million for the three months ended September 30, 2014, compared with \$11.3 million for the same period of 2013. Research and development expenses increased primarily due to increased clinical and manufacturing costs related to ACH-3422 and ACH-3102, as well as increased costs related to our complement inhibitor program. These amounts were partially offset by decreased clinical trial and manufacturing costs related to combination and drug interaction studies of sofosbuvir and ACH-3102. Personnel costs and non-cash stock compensation also increased primarily due to the addition of personnel in the Company's development group.

For the three months ended September 30, 2014, general and administrative expenses were \$3.7 million, compared with \$2.7 million incurred during the same period in 2013. The increase for the three months ended September 30, 2014 was primarily due to increased corporate legal, consulting and insurance fees, combined with increased personnel costs.

Non-cash stock compensation expense totaled \$1.6 million for the third quarter of 2014 as compared with \$1.2 million for the third quarter of 2013 and is included in research and development and general and administrative expenses.

Nine Month Results

For the nine months ended September 30, 2014, Achillion reported a net loss of \$47.4 million, compared to a net loss of \$45.6 million in the same period in 2013. There were no revenues during the first nine months of 2014, and no revenues in the prior year period.

For the nine months ended September 30, 2014, research and development expenses totaled \$37.1 million, compared with \$36.6 million during the same period in 2013. Research and development expenses increased primarily due to increased consulting fees and manufacturing and clinical costs related to ACH-3422 and ACH-3102 combination trials with ACH-2684 and sofosbuvir, as well as increased costs related to our complement inhibitor program. Personnel costs and non-cash stock compensation also increased primarily due to the addition of personnel in the Company's development group.

General and administrative expenses were \$10.7 million for the nine months ended September 30, 2014, increased from \$9.4 million in the same period in 2013. The increase for the nine months ended September 30, 2014 was primarily due to increased corporate legal and insurance fees, combined with increased personnel costs.

Non-cash stock compensation expense totaled \$4.8 million for the nine months ended September 30, 2014 as compared with \$4.3 million for the same period in 2013, and is included in research and development and general and administrative expenses.

About Achillion Pharmaceuticals

Achillion is seeking to apply its expertise in biology and structure-guided design and a deep understanding of patient and clinician needs to develop innovative treatment solutions aimed at improving patients' lives. The company's scientific excellence, integrated capabilities and experienced team position it to successfully achieve its goal of advancing new products along the entire continuum from the bench to the patient. Achillion's pipeline is currently focused on small molecule therapeutics for infectious disease and complement-related diseases. 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, including statements with respect to: the potential benefits and

prospects for Achillion's portfolio of HCV and complement factor D inhibitor compounds; the expected efficiency and benefits of Achillion's clinical trial design approaches; and the Company's goals and plans with respect to advancing compounds into and through clinical development and obtaining and releasing data readouts from trials of its compounds. Achillion may use words such as "expect," "anticipate," "project," "intend," "plan," "aim," "believe," "seek," "estimate," "can," "may," "will," "would," and "should" and similar expressions to identify such forward-looking statements. 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: demonstrate in any current and future clinical trials the requisite safety, efficacy and combinability of its drug candidates; advance the preclinical and clinical development of its drug candidates, including ACH-3422, ACH-3102, sovalprevir, and complement factor D inhibitors, under the timelines it projects in current and future clinical trials; obtain and maintain necessary regulatory approvals; obtain and maintain patent protection for its drug candidates and the freedom to operate under third party intellectual property; establish commercial manufacturing arrangements; identify, enter into and maintain collaboration agreements with appropriate third-parties; compete successfully with numerous other companies that are seeking to develop improved therapies for the treatment of HCV; manage expenses; 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 Annual Report on Form 10-K for the year-ended December 31, 2013 and its Quarterly Report on Form 10-Q for the quarter ended June 30, 2014.

In addition, any forward-looking statements in this press release represent 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		Nine Months Ended	
	September 30,		September 30,	
	2014	2013	2014	2013
Revenue	\$ --	\$ --	\$ --	\$ --
Operating expenses:				
Research and development	12,070	11,342	37,089	36,629
General and administrative	3,694	2,734	10,676	9,353
Total operating expenses	15,764	14,076	47,765	45,982
Loss from operations	(15,764)	(14,076)	(47,765)	(45,982)
Other income (expense):				
Interest income	101	166	376	429
Interest expense	(4)	(9)	(23)	(44)
Net loss	<u>\$ (15,667)</u>	<u>\$ (13,919)</u>	<u>\$ (47,412)</u>	<u>\$ (45,597)</u>
Net loss per share - basic and diluted	<u>\$ (0.16)</u>	<u>\$ (0.14)</u>	<u>\$ (0.49)</u>	<u>\$ (0.49)</u>
Weighted average shares outstanding - basic and diluted	<u>99,031</u>	<u>96,648</u>	<u>97,622</u>	<u>93,066</u>

Balance Sheets**(Unaudited, in thousands)**

	September 30,	December 31,
	2014	2013
Cash, cash equivalents, marketable securities and interest receivable	\$ 127,071	\$ 159,104
Working capital	117,709	115,379
Total assets	130,571	162,417
Long-term liabilities	--	56
Total liabilities	10,968	9,459
Total stockholders' equity	119,603	152,958

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