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Achillion Announces Positive Proof-of-Concept Data With ACH-3102

-Second-Generation Pan-Genotypic NS5A Inhibitor Achieves Potent Antiviral Activity of Mean Maximum 3.74 Log₁₀ Reduction Following a Single Dose -

- Initiated Enrollment in a Phase 2 Clinical Trial Evaluating ACH-3102 Plus Ribavirin for the Treatment of HCV Genotype 1b-

- Hosting Analyst Day Today With Live Webcast Beginning at 1:00 p.m. ET -

NEW HAVEN, Conn., Sept. 27, 2012 (GLOBE NEWSWIRE) -- **Achillion Pharmaceuticals, Inc.** (Nasdaq:ACHN) today announced positive proof-of-concept results with ACH-3102, a second-generation pan-genotypic NS5A inhibitor being developed for the treatment of chronic hepatitis C viral infections (HCV). Administration of a single-dose of ACH-3102 to genotype (GT) 1a HCV-infected subjects resulted in a mean maximum 3.74 log₁₀ reduction in HCV RNA (range 2.9 — 4.6 log₁₀). Significant reductions in HCV RNA were achieved in subjects with resistant variants at baseline, including L31M and Y93C variants.

Based on these data, combined with safety and tolerability results from the Phase 1a trial in healthy subjects evaluating up to 14 days of ACH-3102, Achillion has initiated a pilot Phase 2 clinical trial evaluating ACH-3102 in combination with ribavirin for the treatment of patients with chronic GT 1b HCV.

"We believe these proof-of-concept results demonstrate the differentiation of ACH-3102 from first-generation NS5A inhibitors. The potency of ACH-3102 was successfully shown against genotype 1a, historically the hardest to treat HCV subtype," commented Michael Kishbauch, President and Chief Executive Officer of Achillion. "Furthermore, we believe the enhanced resistance profile of ACH-3102 observed *in vitro* has been validated in the clinic with robust antiviral activity against baseline mutations such as L31M. These results support our belief that this second-generation pan-genotypic NS5A inhibitor has the potential to become a cornerstone compound."

ACH-3102: Phase 1 Program

In May 2012, Achillion initiated a Phase 1a clinical trial evaluating the safety and tolerability of single and multiple ascending doses of ACH-3102 in healthy volunteers. To date, 42 healthy volunteers have received a single dose of ACH-3102, ranging from 25 mg to 1,000 mg. An additional 32 healthy volunteers have received 14 days of ACH-3102 once-daily evaluating various dosing regimens. Preliminary data from the single and multiple ascending dose groups demonstrated that ACH-3102 was well tolerated at all doses evaluated. There were no serious adverse events and no clinically significant changes in vital signs, electrocardiograms (ECGs), or laboratory evaluations. All reported adverse events were classified as mild or moderate and were transient in nature.

In August 2012, Achillion initiated a Phase 1b clinical trial enrolling a total of 14 patients infected with GT 1a chronic HCV, of which 2 received placebo and 12 received a single dose of 50 mg, 150 mg or 300 mg ACH-3102. No serious adverse events were reported and there were no patient discontinuations.

The mean maximum HCV RNA decline for each dose group is provided below:

Genotype	Dose (mg)	N	Mean maximal	Range decline
			decline HCV RNA (log ₁₀)	HCV RNA (log ₁₀)
1a	50	4	3.78	3.35 — 4.16
	150	4	3.52	2.91 — 3.98
	300	4	3.93	3.40 — 4.60
	Placebo	2	0.72	--

An assessment of clinical virology was conducted on baseline samples from all 12 patients receiving a single-dose of ACH-3102. Sequencing revealed one patient had a baseline L31M mutation (300 mg dose group, maximum HCV RNA decline of 3.4 log₁₀) and another patient had a baseline Y93C mutation (300 dose group, maximum HCV RNA decline of 4.6 log₁₀). These mutations have been previously reported to convey a high level of resistance to first-generation NS5A inhibitors which was not observed following exposure to ACH-3102.

ACH-3102: All-oral, interferon-free pilot Phase 2 12-week trial of ACH-3102 and ribavirin for the treatment of HCV GT 1b

Achillion has initiated patient enrollment in an open-label Phase 2 pilot trial evaluating 12-weeks of once-daily ACH-3102 in combination with ribavirin for the treatment of HCV GT 1b. This study will initially enroll up to 16 treatment-naïve patients with GT 1b IL28B CC HCV. Patients will receive 225 mg of ACH-3102 on day 1 followed by 75 mg of ACH-3102 once daily on subsequent days in combination with twice daily ribavirin. The primary objective of the trial is to determine the safety and sustained virologic response 12 weeks after the completion of treatment (SVR12) with secondary endpoints assessing safety, pharmacokinetics, pharmacodynamics, and virologic endpoints including rapid virologic response (RVR) and extended RVR (eRVR). Achillion expects to report initial RVR results from this study during the fourth quarter of 2012.

Mr. Kishbauch further commented, "With the initiation of this all-oral 12-week study evaluating ACH-3102 and ribavirin for the treatment of HCV genotype 1b, we have rapidly advanced our portfolio and believe the attributes of ACH-3102, as well as sovalprevir, our Phase 2 protease inhibitor, have the potential to provide optimized compounds for the broad treatment of HCV."

Analyst Day Webcast

Achillion is hosting its inaugural Analyst Day and simultaneous webcast on Thursday, September 27, 2012 at 1:00 p.m. Eastern Time. To access a copy of the presentation and the live audio webcast of the event, please visit www.achillion.com. Please connect to Achillion's website several minutes prior to the start of the broadcast to ensure adequate time for any software download that may be necessary. A replay of the webcast will be available on www.achillion.com beginning approximately 2 hours after the conclusion of the event.

About HCV

The hepatitis C virus is the most common cause of viral hepatitis, which is an inflammation of the liver. It is currently estimated that more than 170 million people are infected with HCV worldwide including more than 5 million people in the United States, more than twice as widespread as HIV. Three-fourths 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. The current standard of care is limited by its specificity for certain types of HCV, significant side-effect profile, and injectable route of administration.

About Achillion Pharmaceuticals

Achillion is an innovative pharmaceutical company dedicated to bringing important new treatments to patients with infectious disease. Achillion's proven discovery and development teams have advanced multiple product candidates with novel mechanisms of action. Achillion is focused on solutions for the most challenging problems in infectious disease including HCV and resistant bacterial infections. For more information on Achillion Pharmaceuticals, please visit www.achillion.com or call 1-203-624-7000.

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 favorable activity and potential benefits of ACH-3102 and sovalprevir, and expectations about milestone achievement including the potential to report RVR results during the fourth quarter of 2012. Among the 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: replicate in later clinical trials the positive results found in nonclinical studies and earlier stage clinical studies of sovalprevir, ACH-2684, and ACH-3102; advance the development of its drug candidates under the timelines it anticipates in current and future clinical trials; obtain necessary regulatory approvals; obtain 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 other companies that are seeking to develop improved therapies for the treatment of HCV; manage expenses; and raise the substantial additional capital needed to achieve its business objectives. 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, 2011 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 obligation to update any forward-looking statement, except as required by applicable law.

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