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Achillion Reports 2015 Fourth Quarter and Year-End Financial Results

Strong Balance Sheet to Support Planned 2016 Clinical Expansion of Complement Factor D Platform

NEW HAVEN, Conn., Feb. 25, 2016 (GLOBE NEWSWIRE) -- **Achillion Pharmaceuticals, Inc.** (Nasdaq:ACHN) today reported financial results for the three and twelve month periods ending December 31, 2015.

For the three months ended December 31, 2015, the Company reported net income of \$17.0 million, compared to a net loss of \$21.6 million in the three months ended December 31, 2014. For the full year ended December 31, 2015, the Company's net loss was \$5.0 million, or \$0.04 per share, compared to a net loss of \$69.0 million for the year ended December 31, 2014, or \$0.70 per share. Cash, cash equivalents, marketable securities, and interest receivable at December 31, 2015 were \$460.5 million.

"2015 was a transformational year for Achillion that we believe positions us for future success. We established a world-wide collaboration with Janssen, who is currently evaluating a combination therapy with odalasvir, which we believe has the potential to change the HCV treatment paradigm. Our strong balance sheet enables us to advance innovative therapies, discovered by our scientists, that could significantly improve the lives of patients with complement-mediated diseases," commented Milind Deshpande, Ph.D., President and Chief Executive Officer of Achillion.

Dr. Deshpande further commented, "As a leader in complement biology, we look forward to achieving a number of milestones throughout 2016 with ACH-4471, our first, orally-administered, small molecule complement factor D inhibitor being developed as a potential treatment for PNH and other complement-mediated rare diseases. We plan to report interim results from the ongoing phase 1 trial in healthy volunteers that aims to generate insights into the safety, tolerability, pharmacokinetics and pharmacodynamics of ACH-4471 in the second quarter. And by the end of this year, we plan to report results from a multiple ascending dose study of ACH-4471 in healthy volunteers, as well as initiate a phase 2 trial for patients with PNH."

Highlights of 2015

- 1 Achillion and Janssen Pharmaceuticals, Inc. (Janssen), one of the Janssen Pharmaceutical Companies of Johnson & Johnson, established a 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. Assuming successful development and commercialization, Achillion is eligible to receive up to \$905 million in clinical, regulatory and commercialization milestone payments. Achillion is also eligible to receive tiered royalty percentages between mid-teens and low-twenties based upon future worldwide sales, if any. Janssen is responsible for all development and commercialization costs within the collaboration. Achillion received \$225 million from Johnson & Johnson Innovation — JJDC, Inc. relating to the issuance and sale of 18,367,346 shares of Achillion's common stock at a price of \$12.25 per share.
- 1 Achillion nominated and made a regulatory submission for ACH-4471, the Company's first orally-administered small molecule complement factor D inhibitor, as a potential treatment for PNH and other complement-mediated diseases.

Key 2016 Planned Milestones

ACH-4471, small-molecule factor D inhibitor

- 1 In February 2016, Achillion initiated a phase 1 healthy-volunteer single-ascending dose (SAD) trial to explore the safety, pharmacokinetics and pharmacodynamics of ACH-4471. Interim results from this trial are anticipated in the second quarter of 2016.
- 1 During the second quarter of 2016, Achillion anticipates initiation of a phase 1a healthy-volunteer multiple-ascending dose (MAD) trial to explore the safety, pharmacokinetics and pharmacodynamics of ACH-4471. Interim results from this trial are anticipated in the third quarter of 2016.
- 1 During the third quarter of 2016, Achillion anticipates initiation of a phase 2 trial with ACH-4471 for patients with PNH. Interim results from this trial are anticipated during the fourth quarter of 2016.

Dr. Joel Barrish, Chief Scientific Officer at Achillion, commented, "Achillion has established a robust and differentiated platform to evaluate and advance potential complement factor D inhibitors tailored to specific indications. Throughout 2016,

we plan to continue to optimize distinct chemical candidates with attributes suitable for alternative delivery routes for potential use in ophthalmic indications, including dry AMD, and for respiratory indications such as COPD."

World-wide collaboration for HCV with Janssen

- ▮ Achillion anticipates that interim top-line results from Janssen's ongoing phase 2a trial of odalasvir, AL-335, and simeprevir in patients with treatment-naïve genotype 1 HCV will be reported during the first half of 2016.

Fourth Quarter 2015 Financial Results

The Company reported net income of \$17.0 million for the three months ended December 31, 2015, compared to a net loss of \$21.6 million for the three months ended December 31, 2014.

Achillion recognized in the fourth quarter of 2015 revenue of \$31.6 million under the Janssen Agreement, representing a portion of the premium paid by JJDC associated with its equity purchase of Achillion common stock which was being recognized over the 180-day technology transfer period. No revenue was recognized during the three months ended December 31, 2014.

Research and development expenses were \$9.6 million in the fourth quarter of 2015, compared to \$16.4 million for the same period of 2014, the change primarily resulting from increased preclinical and manufacturing costs related to Achillion's complement inhibitor program that were offset by decreased clinical trial costs related to its ACH-3422 clinical trials, its odalasvir and sofosbuvir combination trial and ACH-3422 clinical and manufacturing costs. Personnel costs and non-cash stock-based compensation also increased.

For the three months ended December 31, 2015, general and administrative expenses totaled \$5.5 million, compared to \$5.2 million in the same period in 2014, the increase primarily due to increased personnel and facilities costs due to the addition of personnel, partially offset by decreased corporate legal fees.

Year-end 2015 Financial Results

For the year ended December 31, 2015, the Company reported a net loss of \$5.0 million, compared to a net loss of \$69.0 million in 2014. For the year ended December 31, 2015, research and development expenses totaled \$56.6 million, compared to \$53.5 million in 2014. The increase in research and development costs from 2014 to 2015 was primarily due to increased preclinical and manufacturing costs related to the Company's complement inhibitor program. These amounts were partially offset by decreased clinical trial costs related to its ACH-3422 clinical trials, odalasvir and sofosbuvir combination trial and ACH-2684 clinical and manufacturing costs. Personnel and non-cash stock-based compensation costs also increased due to the addition of personnel in the drug development group.

General and administrative expenses were \$24.7 million for the year ended December 31, 2015, compared to \$15.9 million for the year ended December 31, 2014, the increase primarily due to increased business consulting and corporate legal fees related to the Janssen Agreement, increased corporate fees and taxes, and increased personnel and non-cash stock-based compensation costs due to the addition of personnel.

2016 Financial Guidance

At December 31, 2015, Achillion had cash, cash equivalents, marketable securities and interest receivable of approximately \$460.5 million.

The Company expects that research and development expenses during 2016 will be approximately \$65-70 million and that net cash used in operating activities in 2016 will be approximately \$80 million based on current operating plans, anticipated timelines and the estimated cost of clinical trials and product development programs. The net loss per share for fiscal 2016 is anticipated to approximate \$0.65-0.70 per share.

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 compounds that inhibit factor D, can be orally-administered, 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), atypical hemolytic uremic syndrome (aHUS), 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 positioning for success in 2016 and beyond; the potential benefits of the collaboration with Janssen for treatment of HCV and the Company's expectation that interim top-line results from the ongoing phase 2a trial of odalasvir, AL-335, and simeprevir will be reported during the first half of 2016; the potential for the Company's complement factor D inhibitor program to significantly improve the lives of patients with complement-mediated diseases; the Company's plans to rapidly advance its complement factor D inhibitor platform, including ACH-4471; the expected plans, timing, data readouts and results from ongoing and planned clinical trials of ACH-4471; the Company's estimates with respect to research and development expenses and net cash used in operating activities in 2016 and net loss per share for fiscal 2016; 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 drug candidates, including 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. 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 quarter ended September 30, 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.

ACHILLION PHARMACEUTICALS INC. (ACHN)

Statements of Operations

(Unaudited, in thousands, except per share amounts)

	Three Months Ended December 31,		Year Ended December 31,	
	2015	2014	2015	2014
Revenue	\$ 31,591	\$ -	\$ 66,122	\$ -
Operating expenses:				
Research and development	9,642	16,426	56,553	53,515
General and administrative	5,450	5,235	24,676	15,911
Total operating expenses	15,092	21,661	81,229	69,426

Loss from operations	16,499	(21,661)	(15,107)	(69,426)
Other income (expense):				
Interest income	466	78	1,188	455
Interest expense	(13)	(13)	(55)	(37)
Other income	-	-	8,944	-
Net loss	<u>\$ 16,952</u>	<u>\$ (21,596)</u>	<u>\$ (5,030)</u>	<u>\$ (69,008)</u>
Net loss per share - basic and diluted	<u>\$ 0.12</u>	<u>\$ (0.21)</u>	<u>\$ (0.04)</u>	<u>\$ (0.70)</u>
Weighted average shares outstanding - basic and diluted	<u>136,558</u>	<u>100,579</u>	<u>125,592</u>	<u>98,367</u>

Balance Sheets
(Unaudited, in thousands)

	December 31, 2015	December 31, 2014
Cash, cash equivalents, marketable securities, interest and subscriptions receivable	\$ 460,540	\$ 159,167
Working capital	447,930	141,816
Total assets	464,525	156,807
Long-term liabilities	231	279
Total liabilities	14,889	13,338
Total stockholders' equity	449,636	143,469

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