February 23, 2017

Achillion Reports 2016 Fourth Quarter and Year-End Financial Results

- *Complement factor D inhibitor program on track for initiation of ACH-4471 phase II trial for treatment-naïve PNH patients during the first half of 2017*
- *Expanding complement platform includes internally discovered next-generation oral factor D inhibitors with the goal of advancing at least one into clinical development by year-end*
- *Company advancing multiple small molecule candidates for ophthalmic administration to potentially treat dry AMD/ geographic atrophy*
- *Company reports $392.5 million in cash, cash equivalents, marketable securities, and interest receivable*

NEW HAVEN, Conn., Feb. 23, 2017 (GLOBE NEWSWIRE) -- **Achillion Pharmaceuticals, Inc.** (Nasdaq:ACHN) today reported financial results for the three and twelve month periods ending December 31, 2016.

For the three months ended December 31, 2016, the Company reported a net loss of $4.4 million, compared to net income of $17.0 million in the three months ended December 31, 2015. For the full year ended December 31, 2016, the Company's net loss was $61.7 million, or $0.45 per share, compared to a net loss of $5.0 million for the year ended December 31, 2015, or $0.04 per share. Cash, cash equivalents, marketable securities, and interest receivable at December 31, 2016 were $392.5 million, not including an additional $15.0 million milestone payment earned under the Janssen collaboration in December 2016 and received in January 2017.

"Achillion has established itself as a leader in complement alternative pathway (AP) research. With the advancement of ACH-4471 into the clinic, we were first to report inhibition of AP activity in humans after oral dosing, including robust suppression of Bb levels, an *in vivo* marker of biologic activity of Factor D," commented Milind Deshpande, Ph.D., President and Chief Executive Officer of Achillion. "I am excited about the progress we have achieved in our portfolio of alternative pathway inhibitors and we remain focused on developing medicines for underserved C3G, PNH and dry AMD patients."

Dr. Deshpande further stated, "Over the course of 2017, we look forward to making progress against our goal to advance ACH-4471 into phase II studies, first for treatment-naive PNH patients during the first half of 2017, followed by treatment of C3G patients during the second half of 2017. In addition, multiple next-generation oral inhibitors are being assessed in IND-enabling studies and we intend to bring at least one into clinical development by the end of 2017. Lastly, our ophthalmology portfolio now includes several internally discovered factor D inhibitors that are being optimized in parallel with ocular delivery technologies with the goal of identifying potential treatment regimens that can be administered in a minimum of three month intervals for dry AMD/geographic atrophy."

Key 2017 Planned Milestones

- ***ACH-4471, oral factor D inhibitor***
  - Achillion recently initiated patient screening in a phase II clinical trial for patients with PNH. The Company anticipates providing an update on this PNH study and the phase I MAD study during the first half of 2017.
  - During the second half of 2017, Achillion anticipates initiating a phase II trial with ACH-4471 for patients with C3G, which includes dense deposit disease (DDD) and C3 glomerulonephritis (C3GN).
- ***Next generation oral factor D inhibitors***
  - By the end of 2017, Achillion expects to initiate phase I clinical development with an oral next-generation, alternative pathway factor D inhibitor compound.
- ***Factor D inhibitors for intraocular administration***
  - Excessive activation of the complement alternative pathway has been implicated in multiple therapeutic areas outside of rare diseases including dry AMD/geographic atrophy (GA). Achillion has discovered and initiated preclinical testing of multiple compounds for potential clinical development for GA. The Company anticipates selecting a lead compound for clinical development by the end of 2017.

Janssen World-wide Collaboration for HCV

In September 2016, Achillion announced positive interim results from the Janssen-sponsored 604 phase IIa clinical trial of the triple combination of odalasvir, simeprevir, and AL-335, the triple combination now referred to as JNJ-4178, which achieved 100% SVR12, or sustained viral response 12 weeks after the completion of treatment, for both 8-week and 6-week treatment durations in HCV genotype 1 infected patients. Based on these positive results, Janssen initiated in November 2016 a phase IIb clinical study (OMEGA-1) in HCV patients evaluating both six- and eight-week treatment durations.

David Apelian, M.D., Ph.D., Chief Medical Officer of Achillion commented, "Our worldwide collaboration for HCV with Janssen has continued to make significant progress since its establishment in mid-2015. In November 2016, JNJ-4178, a once-daily regimen containing odalasvir, AL-335, and simeprevir, was advanced into a global phase IIb clinical trial, for which we earned a $15 million clinical milestone. SVR12 results from this phase IIb trial, in cohorts of 150 subjects receiving either 6 weeks or 8 weeks of JNJ-4178, are expected to position the program for potential advancement in phase III in 2018."

Fourth Quarter 2016 Financial Results

The Company reported net loss of $4.4 million for the three months ended December 31, 2016, compared to a net income of $17.0 million for the three months ended December 31, 2015.

Achillion recognized $15.0 million of revenue during the three months ended December 31, 2016 related to achievement of the first clinical milestone in the development of JNJ-4178 under the Janssen Agreement. In the fourth quarter of 2015, Achillion recognized $31.6 million of revenue under the Janssen Agreement, representing a portion of the premium paid by JJDC associated with its equity purchase of Achillion common stock.

Research and development expenses were $15.0 million in the fourth quarter of 2016, compared to $9.6 million for the same period of 2015, the change primarily due to increased manufacturing, clinical trial and consulting costs related to the advancement of ACH-4471, partially offset by decreased manufacturing, clinical trial and consulting costs related to our HCV compounds, which were licensed to Janssen in 2015.

For the three months ended December 31, 2016, general and administrative expenses totaled $5.3 million, compared to $5.5 million in the same period in 2015, the decrease primarily due to decreased business development consulting fees and corporate legal fees that were incurred in 2015 related to the Janssen Agreement, partially offset by increased personnel and non-cash stock compensation largely related to the addition of personnel.

Year-end 2016 Financial Results

For the year ended December 31, 2016, the Company reported a net loss of $61.7 million, compared to a net loss of $5.0 million in 2015. For the year ended December 31, 2016, research and development expenses totaled $59.2 million, compared to $56.6 million in 2015. The increase was primarily due to increased manufacturing, clinical trial and consulting costs related to the advancement of the Company's factor D portfolio, including ACH-4471, partially offset by decreased manufacturing, clinical trial and consulting costs related to our HCV compounds, which were licensed to Janssen in 2015. Personnel costs also increased due to the addition of personnel in our discovery and development groups.

General and administrative expenses were $20.7 million for the year ended December 31, 2016, compared to $24.7 million for the year ended December 31, 2015, the decrease primarily due to decreased business development consulting fees and corporate legal fees that were incurred in 2015 related to the Janssen Agreement, partially offset by increased personnel and non-cash stock compensation largely related to the addition of personnel.

2017 Financial Guidance

The Company expects that research and development expenses during 2017 will be approximately $75 - 80 million and that net cash used in operating activities in 2017 will be approximately $70 - 75 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 2017 is anticipated to approximate $0.70 - 0.75 per share.

About the Achillion Alternative Pathway Complement Factor D Platform

Achillion has leveraged its internal discovery capabilities and a novel complement-related platform to develop small molecule drug candidates that includes oral inhibitors of the complement alternative pathway. Factor D is an essential serine protease involved in the complement alternative 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 the alternative pathway plays a critical role. Potential indications currently being evaluated for these compounds include paroxysmal nocturnal hemoglobinuria (PNH), C3 glomerulopathy (C3G), and

dry age-related macular degeneration (dry AMD)/geographic atrophy (GA).

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," "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: Achillion'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 Achillion's collaboration with Janssen; the planned advancement of Achillion's other small molecule factor D inhibitors, including those for the treatment of dry AMD and GA; and statements concerning Achillion'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 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 planned phase IIb combination trial that include Achillion's 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 Quarterly Report on Form 10-Q for the fiscal quarter ended September 30, 2016, 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 December 31,		Year Ended December 31,	
	2016	**2015**	**2016**	**2015**
Revenue	$ 15,000	$ 31,591	$ 15,000	$ 66,122
Operating expenses:				
Research and development	15,029	9,642	59,162	56,553
General and administrative	5,260	5,450	20,703	24,676

Total operating expenses	20,289	15,092	79,865	81,229
Loss from operations	(5,289)	16,499	(64,865)	(15,107)
Other income (expense):				
Interest income	874	466	3,227	1,188
Interest expense	(14)	(13)	(68)	(55)
Other income	-	-	-	8,944
Net loss	$ (4,429)	$ 16,952	$ (61,706)	$ (5,030)
Net loss per share - basic and diluted	$ (0.03)	$ 0.12	$ (0.45)	$ (0.04)
Weighted average shares outstanding - basic and diluted	136,693	136,558	136,667	125,592

Balance Sheets
(Unaudited, in thousands)

	December 31, 2016	December 31, 2015
Cash, cash equivalents, marketable securities, and interest receivable	$ 392,486	$ 460,540
Working capital	368,564	447,930
Total assets	413,875	464,525
Long-term liabilities	450	231
Total liabilities	14,421	14,889
Total stockholders' equity	399,454	449,636

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