



February 22, 2018

Achillion Announces Restructuring to Advance Corporate Strategy; Announces 2017 Fourth Quarter and Full Year Financial Results

- | *Corporate restructuring expected to enable Achillion to focus on goal of advancing its clinical and late-stage preclinical portfolio while maintaining a strong balance sheet*
- | *Joseph Truitt, Chief Operating Officer, promoted to President of Achillion*
- | *Achillion seeking to generate additional datasets with ACH-4471 in both C3G and PNH programs in 2018*
- | *Conference call scheduled for today at 8:30 a.m. EST*

NEW HAVEN, Conn., Feb. 22, 2018 (GLOBE NEWSWIRE) -- **Achillion Pharmaceuticals, Inc.** (Nasdaq:ACHN), a clinical stage biopharmaceutical company focused on developing inhibitors of the complement alternative pathway, today announced an operational restructuring plan that aims to focus the organization on advancing Achillion's existing clinical and late-stage preclinical factor D inhibitors and reduce expenses to maintain its strong balance sheet. The plan is expected to deliver approximately \$10 million of savings in 2018 over 2017 expense levels. The restructuring will reduce the Company's workforce by approximately 20% to approximately 70 employees.

In addition, the Company announced that Joseph Truitt has been promoted to President and Chief Operating Officer, continuing to report to Chief Executive Officer, Milind Deshpande, Ph.D.

Dr. Deshpande stated, "We are focused on executing against our 2018 strategic objectives with the goal of delivering transformative therapies to patients. We believe the operational expertise that Joe brings to his new role will strengthen our capabilities to achieve those objectives. While it is difficult to undertake a restructuring, we believe through efficient use of our capital, we will have the potential to build significant value in our Factor D inhibitor portfolio."

Mr. Truitt commented, "At Achillion, we have established ourselves as a leader in complement alternative pathway (AP) research, drug development and intellectual property with the depth and breadth of our patent portfolio. Our lead compound, ACH-4471, is in phase 2 studies in both C3 glomerulopathy (C3G) and paroxysmal nocturnal hemoglobinuria (PNH) and I look forward to delivering on our goal of additional clinical data in these indications in 2018. I am particularly enthusiastic about our work in C3G where our market research has shed light on the underserved needs of these patients who have a debilitating disease that is believed to be caused by an overactive AP. C3G affects at least 8,000 people in the United States and the major European markets, specifically France, Germany, Italy, Spain and the United Kingdom, and there are no approved treatments."

2018 Areas of Strategic Focus

- | ***ACH-4471, oral factor D inhibitor, for the treatment of C3G & IC-MPGN***
 - Completion of a Phase 2 14-day study in patients with C3G or IC-MPGN with interim data targeted for third quarter 2018;
 - Initiation of a 6-month double-blind, placebo-controlled therapeutic study in patients with C3G with data available in 2019; and
 - Initiation of a 12-month, open-label study in patients with C3G with interim data targeted for fourth quarter 2018.
- | ***ACH-4471, oral factor D inhibitor, for the treatment of PNH***
 - Continuation of on-going Phase 2 monotherapy study with interim data targeted for fourth quarter 2018; and
 - Initiation of add-on study with eculizumab with interim data targeted for fourth quarter 2018.
- | ***ACH-5228 and ACH-5548, next-generation factor D inhibitors***
 - Completion of a Phase 1, single-ascending dose clinical study of ACH-5228 in healthy volunteers, which was initiated in December 2017; and
 - Initiation of a phase 1, single-ascending dose clinical study of ACH-5548 in healthy volunteers targeted to be initiated in the second quarter 2018; and
 - Results for both next-generation compounds (ACH-5228 and ACH-5548) targeted for fourth quarter 2018.

Financial Results

Achillion also reported earnings for the three months and year ended December 31, 2017.

Fourth Quarter 2017 Financial Results

For the three months ended December 31, 2017, the Company reported a net loss of \$23.2 million, compared to a net loss of \$4.4 million in the three months ended December 31, 2016. During the three months ended December 31, 2017, the Company did not recognize any revenue compared to the three months ended December 31, 2016 in which it recognized \$15.0 million of revenue under the Janssen Agreement that was terminated effective November 8, 2017.

Research and development expenses were \$15.7 million in the fourth quarter of 2017, compared to \$15.0 million for the same period of 2016. The increase was primarily due to increased clinical trial expenses for ACH-4471 and manufacturing and formulation expenses for ACH-5228.

For the three months ended December 31, 2017, general and administrative expenses totaled \$8.7 million, compared to \$5.3 million in the same period in 2016. The increase was primarily the result of the Company's payment of the underwriting fees in connection with the public resale by the Johnson and Johnson Development Corporation in November 2017 of all the shares of common stock it acquired from the Company in 2015.

Year-end 2017 Financial Results

For the year ended December 31, 2017, the Company's net loss was \$85.2 million, or \$0.62 per share, compared to a net loss of \$61.7 million for the year ended December 31, 2016, or \$0.45 per share. During the year ended December 31, 2017, Achillion did not recognize any revenue compared to the year ended December 31, 2016 in which Achillion recognized \$15.0 million of revenue under the Janssen Agreement that was terminated effective November 8, 2017.

For the year ended December 31, 2017, research and development expenses totaled \$65.1 million, compared to \$59.2 million for the year ended December 31, 2016. The increase primarily related to clinical trial costs for ACH-4471 combined with increased preclinical and manufacturing costs for ACH-5228. Discovery research costs related to our next-generation factor D inhibitors also increased, and were partially offset by decreased preclinical and manufacturing costs for ACH-4471.

General and administrative expenses were \$24.5 million for the year ended December 31, 2017, compared to \$20.7 million for the year ended December 31, 2016. The increase was primarily due to legal and consulting fees, as well as the Company's payment of underwriting fees, in connection with the public resale by the Johnson and Johnson Development Corporation in November 2017 of all the shares of common stock it acquired from the Company in 2015.

Cash, cash equivalents, marketable securities, and interest receivable at December 31, 2017 were \$331.8 million.

Restructuring Details and 2018 Financial Guidance

In February, the Company implemented a plan of restructuring to focus on the Company's clinical stage and late-stage preclinical portfolio while retaining the biology and chemistry core strengths necessary to support future clinical development and continue research with its complement factor D portfolio. Under the restructuring, the Company expects to reduce annual projected operating expenses by approximately \$10 million, or 12%, from 2017 levels and reduced headcount by approximately 20% over 2017 levels.

As a result of its restructuring, the Company expects that research and development expenses during 2018 will be approximately \$58-60 million, that general and administrative expenses will be approximately \$19 - 20 million, and that net cash used in operating activities in 2018 will be approximately \$68 - 70 million based on current operating plans, anticipated timelines and the estimated cost of clinical trials and product development programs. Year-end 2018 cash, cash equivalents and marketable securities are anticipated to total approximately \$260 million. The net loss per share for fiscal 2018 is anticipated to approximate \$0.55 - 0.58 per share.

Webcast and Dial-in Information

Achillion will host a conference call and simultaneous webcast on Thursday, February 22, 2018 at 8:30 a.m. EST. To participate in the conference call, please dial (866) 205-4820 in the U.S. or (419) 386-0004 for international callers. A live audio webcast of the call will be accessible at <http://www.achillion.com> or <http://ir.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 for 30 days on <http://www.achillion.com>. Alternatively, a telephonic replay of the conference call will be available starting at 11:30 a.m. EST on February 22, 2018, through 11:30 a.m. EST on February 26, 2018 by dialing (855) 859-2056 or (404) 537-3406. The replay passcode is 1079527.

About C3G

C3G is a devastating disease affecting the kidney, believed to be caused by an overactive AP and for which there is no FDA approved therapy. C3G affects persons of all ages, with an incidence estimated to be at 1-2 cases per year per 1,000,000 people in both the U.S and EU. There are estimated to be at least 8,000 people with C3G in the United States and the major European markets, specifically France, Germany, Italy, Spain and the United Kingdom, and there are no approved treatments. C3G describes a rare kidney disease characterized by the presence of C3 protein fragments in the filtering units (glomeruli) of the kidney. C3 fragment deposition is reported to result from over-activation of the complement alternative pathway. The chronic deposition of C3 fragments results in inflammation in the glomeruli (glomerulonephritis) and often subsequent permanent renal damage. It is reported that an estimated 30-50% of C3G patients will require dialysis or a transplant within 10 years of diagnosis.

About 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 the Complement Factor D Platform

Achillion has leveraged its internal discovery capabilities and a novel complement-related drug development 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 C3G, immune complex-mediated membranoproliferative glomerulonephritis (IC-MPGN), paroxysmal nocturnal hemoglobinuria (PNH).

About Achillion Pharmaceuticals

Achillion Pharmaceuticals, Inc. (NASDAQ:ACHN) is a science-driven, patient-focused biopharmaceutical company seeking to leverage its capabilities across the continuum from discovery through commercialization in its goal of meeting the needs of people with complement-mediated diseases. The company has employed a highly-disciplined discovery and development approach that has allowed it to develop potent and specific complement factor D inhibitors for AP-mediated diseases. Achillion is rapidly advancing its efforts to bring potentially 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," and "may" 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 ACH-4471, ACH-5228 and ACH-5548; the potential advancement of Achillion's other small molecule factor D inhibitors; the anticipated costs and benefits of Achillion's restructuring plans; Achillion's guidance regarding its financial results for 2018; 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; realize the planned cost savings benefits of its restructuring plan; 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 and enter into collaboration agreements with third-parties; compete successfully in the

markets in which it seeks to develop and commercialize its product candidates and future products; manage expenses and achieve the levels of research and development expense, cash burn, and net loss it has projected for fiscal 2018; 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 fiscal quarter ended September 30, 2017, 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

(in thousands, except per share amounts)

	Three Months Ended December 31,		Year Ended December 31,	
	2017	2016	2017	2016
Revenue	\$ -	\$ 15,000	\$ -	\$ 15,000
Operating expenses:				
Research and development	15,684	15,029	65,052	59,162
General and administrative	8,671	5,260	24,524	20,703
Total operating expenses	24,355	20,289	89,576	79,865
Loss from operations	(24,355)	(5,289)	(89,576)	(64,865)
Other income (expense):				
Interest income	1,165	874	4,390	3,227
Interest expense	(13)	(14)	(50)	(68)
Net loss	<u>\$ (23,203)</u>	<u>\$ (4,429)</u>	<u>\$ (85,236)</u>	<u>\$ (61,706)</u>
Net loss per share - basic and diluted	<u>\$ (0.17)</u>	<u>\$ (0.03)</u>	<u>\$ (0.62)</u>	<u>\$ (0.45)</u>
Weighted average shares outstanding - basic and diluted	<u>137,870</u>	<u>136,693</u>	<u>137,180</u>	<u>136,667</u>

Balance Sheets

(Unaudited, in thousands)

	December 31, 2017	December 31, 2016
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Cash, cash equivalents, marketable securities, and interest

receivable	\$	331,845	\$	392,486
Working capital		291,054		368,564
Total assets		337,613		413,875
Long-term liabilities		214		450
Total liabilities		13,098		14,421
Total stockholders' equity		324,515		399,454

Investors & Media:

Glenn Schulman, PharmD, MPH

Executive Director, Investor Relations

Achillion Pharmaceuticals, Inc.

Tel. (203) 752-5510

gschulman@achillion.com