



May 2, 2018

Achillion Reports First Quarter 2018 Financial Results and Management Transition

- Plans for clinical data in C3 glomerulopathy (C3G) and for paroxysmal nocturnal hemoglobinuria (PNH) remain on track for 2018 -

- Joseph Truitt appointed Chief Executive Officer -

NEW HAVEN, Conn., May 02, 2018 (GLOBE NEWSWIRE) -- **Achillion Pharmaceuticals, Inc.** (Nasdaq:ACHN), a biopharmaceutical company focused on advancing oral small-molecule factor D inhibitors to modulate the complement alternative pathway, today reported financial results for the three months ended March 31, 2018. For the first quarter of 2018, the Company reported a net loss of \$20.6 million or \$0.15 per share, compared with a net loss of \$20.2 million or \$0.15 per share for the first quarter of 2017. Cash, cash equivalents, marketable securities and interest receivable as of March 31, 2018 were \$308.4 million. The Company also reported that Joseph Truitt has been named Chief Executive Officer and that Milind Deshpande, Ph.D. will be stepping down.

Over the past several months, Achillion has been focused on expanding its global clinical development program for ACH-4471 which includes five Phase 2 studies, three in patients with C3G and two in patients with PNH. The Company anticipates reporting interim data from these studies during the second half of this year.

First Quarter 2018 Results

For the first quarter of 2018, the Company reported a net loss of \$20.6 million, or \$0.15 per share, compared with a net loss of \$20.2 million, or \$0.15 per share for the first quarter of 2017. Cash, cash equivalents, marketable securities, and interest receivable as of March 31, 2018 were \$308.4 million.

Research and development expenses were \$14.8 million in the first quarter of 2018, compared with \$15.5 million for the same period of 2017. The decrease was primarily due to decreased non-cash stock compensation and personnel costs due to our restructuring in February 2018, which resulted in fewer employees, combined with decreased manufacturing and formulation costs related to ACH-5228. These amounts were partially offset by increased clinical and clinical trial costs related to ACH-4471 and ACH-5228, combined with increased preclinical costs related to ACH-5548.

For the three months ended March 31, 2018, general and administrative expenses totaled \$5.3 million, compared to \$5.7 million for the same period in 2017, with the decrease primarily due to decreased non-cash stock compensation combined with decreased legal and consulting fees.

Non-cash stock compensation expense totaled \$2.3 million for the first quarter of 2018 as compared to \$3.2 million for the first quarter of 2017, and is included in research and development, general and administrative and restructuring expenses.

During the three months ended March 31, 2018, we incurred \$1.8 million of restructuring charges. These charges consist primarily of employee severance payments, continuation of benefits and outplacement services resulting from the implementation of our restructuring plan in February 2018 which reduced employee headcount by approximately 20%.

CEO Transition

Achillion today announced that Joseph Truitt, the Company's President and Chief Operating Officer, has been named Chief Executive Officer and will be appointed to the Company's Board of Directors. Milind S. Deshpande, Ph.D. will be stepping down from his role as Chief Executive Officer and resigning from the Board of Directors, and is expected to provide continued guidance and scientific support under a consulting agreement.

Dr. Deshpande has been at Achillion for 17 years, the last five as CEO, and under his leadership, the Company transformed from its previous focus on anti-infectives to discovering and developing its first-in-class complement Factor D platform. David Scheer, Chairman of the Achillion Board of Directors, remarked, "Milind has done an outstanding job through a critical transition to a new therapeutic area, and I believe he leaves the Company well positioned to move successfully to late-stage clinical development and commercialization. We are grateful for his contributions."

"I am quite proud of the innovation we have demonstrated over the last five years," said Dr. Deshpande. "The Company has

an exciting future and I am proud to have had a foundational role in positioning Achillion to be a leader in complement biology and developing investigational products that have the potential to improve the lives of patients who have no or few options," added Dr. Deshpande.

Mr. Scheer continued, "We are very pleased that Joe has stepped up to lead Achillion through its next stage of growth, and that he has the experience of successfully navigating many of the challenges that come with launching a rare disease drug. We currently have potentially transformative therapies discovered and developed at Achillion in phase 2 global clinical trials, and we know Joe, the management team and the board are committed to further advancing the Company's drug portfolio."

Mr. Truitt has led Achillion's business development and commercial strategy since joining the Company in 2009. He joined Achillion from Viropharma, after its acquisition of Lev Pharmaceuticals, where he led the build-out of commercial infrastructure and the strategy for the launch of Cinryze[®]. He was previously responsible for sales and commercial operations at Johnson and Johnson's OraPharma subsidiary.

Goals for Complement Factor D Inhibitor Program for Rare Diseases

- | ***ACH-4471, oral factor D inhibitor, for the treatment of C3G***
 - | Completion of a phase 2 14-day study in patients with C3G with interim data targeted for third quarter 2018;
 - | Continue patient screening and enrollment into a phase 2 six-month double-blind, placebo-controlled therapeutic study in patients with C3G with data projected to be available in 2019; and
 - | Initiate patient screening and enrollment into a phase 2 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 the on-going phase 2 monotherapy study with interim data targeted for fourth quarter 2018; and
 - | Continue patient screening and enrollment into a phase 2 add-on study with eculizumab with interim data targeted for fourth quarter 2018.

- | ***ACH-5228 and ACH-5548, next-generation factor D inhibitors***
 - | Completion in the second quarter of a phase 1, single-ascending dose clinical study of ACH-5228 in healthy volunteers;
 - | Initiation of a phase 1, single-ascending dose clinical study of ACH-5548 in healthy volunteers targeted for the second quarter 2018; and
 - | Results for both next-generation compounds (ACH-5228 and ACH-5548) targeted for fourth quarter 2018.

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), and paroxysmal nocturnal hemoglobinuria (PNH).

About C3G

C3G is a devastating disease affecting the kidneys for which there is no approved therapy. C3G affects men and women equally. There are estimated to be approximately 4,000 C3G patients in the United States, more than 4,000 in Europe, and more than 1,000 patients with this disease in Japan. C3G describes a rare renal disease characterized by the presence of predominantly C3 protein fragments in the filtering units (glomeruli) of the kidney. These C3 fragment deposits are thought by experts to be the result of overactivation of the complement alternative pathway. The chronic deposition of C3 fragments results in inflammation in the glomeruli (glomerulonephritis) and subsequent permanent renal damage. 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 Achillion Pharmaceuticals

Achillion Pharmaceuticals, Inc. (NASDAQ:ACHN) is a science-driven, patient-focused company seeking to leverage its capabilities 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 build a platform of potent and specific complement factor D inhibitors for AP-mediated diseases. 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," 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 expectations regarding the CEO transition; and other 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 Annual Report on Form 10-K for the fiscal year ended December 31, 2017, and any 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 March 31,	
	2018	2017
Revenue	\$ -	\$ -
Operating expenses:		
Research and development	14,750	15,495
General and administrative	5,315	5,648
Restructuring charges	1,750	-
Total operating expenses	21,815	21,143

Loss from operations	<u>(21,815)</u>	<u>(21,143)</u>
Other income (expense):		
Interest income	1,239	1,008
Interest expense	<u>(12)</u>	<u>(17)</u>
Net loss	<u>\$ (20,588)</u>	<u>\$ (20,152)</u>
Net loss per share - basic and diluted	<u>\$ (0.15)</u>	<u>\$ (0.15)</u>
Weighted average shares outstanding - basic and diluted	<u>138,014</u>	<u>136,722</u>

Balance Sheets
(Unaudited, in thousands)

	March 31, 2018	December 31, 2017
Cash, cash equivalents, marketable securities and interest receivable	\$ 308,400	\$ 331,845
Working capital	301,362	291,054
Total assets	315,566	337,613
Long-term liabilities	158	214
Total liabilities	8,296	13,098
Total stockholders' (deficit) equity	307,270	324,515

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