Achillion Reports First Quarter 2019 Financial Results

May 9, 2019

- PNH combo Phase 2 trial interim data to be presented May 17, 2019 -
- C3G Phase 2 trials 24 patients enrolled as of May 2019 -
- Next-generation ACH-5228 planned U.S. IND submission Q4:2019 -
- Cash and securities of $254 million as of March 31, 2019 -

BLUE BELL, Pa., May 09, 2019 (GLOBE NEWSWIRE) -- Achillion Pharmaceuticals, Inc. (Nasdaq: ACHN), a clinical-stage biopharmaceutical company dedicated to transforming the lives of patients and families affected by complement-mediated diseases, today reported its financial results for the three months ended March 31, 2019 and provided a portfolio update.

"We made significant progress advancing our portfolio of oral factor D inhibitors in the first quarter of 2019," said Joe Truitt, President and Chief Executive Officer at Achillion. "Our team completed enrollment in our Phase 2 clinical trial of ACH-4471 for paroxysmal nocturnal hemoglobinuria (PNH) in combination with eculizumab. Interim data from this trial has been accepted for presentation at the New Era of Aplastic Anemia and PNH meeting in Napoli, Italy, on May 17, 2019. Our hypothesis for combination therapy is that if the alternative pathway is adequately inhibited then meaningful patient benefit can be achieved in fundamentally different ways than has been seen with C5 inhibitors alone. In addition, we expect to present the data from the recently completed Phase 2 PNH monotherapy trial at a medical meeting later this year."

"In C3G, we have now enrolled 24 patients in our 6-month and 12-month proof of concept trials of ACH-4471," continued Mr. Truitt. "Our goal is to assess interim efficacy and safety data from these two trials, along with a sizeable data set from real-world data derived from patients with C3G. If this data supports advancing into a Phase 3 program, we plan to meet with the U.S. Food and Drug Administration in an end-of-Phase 2 meeting during the fourth quarter of 2019. In addition, we expect to complete our ongoing ex-U.S. Phase 1 multiple ascending dose study for our more potent next-generation oral factor D inhibitor, ACH-5228, and anticipate submitting an Investigational New Drug (IND) Application in the U.S. in the fourth quarter of 2019. We believe successful development of our portfolio of factor D compounds has the potential to be transformative for patients and to deliver on the promise of alternative pathway inhibition."

First Quarter 2019 Financial Results

For the three months ended March 31, 2019, the Company reported a net loss of $19.0 million, compared to a net loss of $20.6 million in the three months ended March 31, 2018.

Research and development expenses were $14.8 million for the three months ended March 31, 2019, compared to $14.0 million for the same period of 2018. The increase for the three months ended March 31, 2019 was primarily due to increased clinical trial costs related to ACH-4471 and ACH-5228. These amounts were partially offset by decreased non-cash stock-based compensation and personnel costs due to fewer employees as compared to the prior period.

For the three months ended March 31, 2019, general and administrative expenses totaled $5.2 million, compared to $6.0 million for the same period of 2018. The decrease for the three months ended March 31, 2019 was primarily due to decreased non-cash stock-based compensation combined with decreased intellectual property related legal fees.

Cash, cash equivalents, and marketable securities at March 31, 2019 were $254.1 million.

About ACH-4471, Complement Factor D Inhibitor for PNH and C3G
Achillion's first-generation oral complement factor D inhibitor, ACH-4471, continues to be evaluated for safety and efficacy in multiple, global Phase 2 clinical programs in patients with PNH and C3G. Preliminary proof-of-concept has been demonstrated in both indications. The PNH program consists of a Phase 2 clinical trial evaluating ACH-4471 in combination with eculizumab in patients who are inadequately controlled or sub-optimally responding to eculizumab. Additionally, the Company continues to dose patients in its PNH monotherapy extension trial. The C3G program consists of two Phase 2 clinical trials which are a six-month blinded, placebo-controlled study and a 12-month open-label study. More information is available at http://www.achillion.com/patients-and-clinicians/.

About ACH-5228 and ACH-5548, Next Generation Complement Factor D Inhibitors
ACH-5228 and ACH-5548 are the Company's next-generation oral factor D inhibitors currently in Phase 1 clinical trials. These compounds have demonstrated enhanced potency as well as improved pharmacokinetic properties that should allow for higher alternative pathway inhibition along with a reduced dosing frequency.

About Achillion Pharmaceuticals
Achillion Pharmaceuticals, Inc. (Nasdaq: ACHN) is a clinical-stage biopharmaceutical company focused on advancing its oral small molecule complement inhibitors into late-stage development and commercialization. Research has shown that an overactive complement system plays a critical role in multiple disease conditions including the therapeutic areas of nephrology, hematology, ophthalmology and neurology. Achillion is initially focusing its drug development activities on complement-mediated diseases where there are no approved therapies or where existing therapies are inadequate for patients. Potential indications being evaluated for its compounds include paroxysmal nocturnal hemoglobinuria (PNH), C3 glomerulopathy (C3G), and immune complex membranoproliferative glomerulonephritis (IC-MPGN). Each of the product candidates in the Company's oral small molecule portfolio was discovered in its laboratories and is wholly owned. To advance its investigational product candidates into Phase 3 clinical trials and commercialization, the Company plans to work closely with key stakeholders including healthcare professionals, patients, regulators and payors. 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,” “continue,” “goal,” “strategy,” “objective,” “may,” “potential,” and similar expressions to identify such forward-looking statements. These forward-looking statements also include statements about: the potential benefits of factor D inhibition as a treatment for complement-mediated diseases; the potential benefits of, and indications for, Achillion’s compounds that inhibit factor D, including ACH-4471, ACH-5228 and ACH-5548; Achillion’s belief that its portfolio of compounds could expand factor D portfolio opportunities, provide strategic optionality or create significant value; the status of enrollment in Achillion’s ongoing clinical trials; Achillion’s expectations regarding the advancement of, and timeline for reporting results from, clinical trials of its product candidates as well as its ability to advance additional compounds; Achillion’s expectations regarding the timing of regulatory interactions and submissions; Achillion’s anticipated cash expenditures for 2019 and the sufficiency of its existing cash resources; and other statements concerning Achillion’s strategic goals, efforts, 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: demonstrate in any current and future clinical trials the requisite safety, efficacy and combinability of its product candidates; 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; enroll patients in its clinical trials on its projected timelines; replicate in later stage clinical trials favorable data demonstrated in preclinical and early-stage clinical trials; obtain and maintain patent protection for its product candidates and the freedom to operate under third party intellectual property; obtain and maintain necessary regulatory approvals, and the granting of orphan designation does not alter the standard regulatory requirements and process for obtaining such approval; establish commercial manufacturing arrangements; identify, enter into and maintain collaboration and other commercial 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; 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 year ended December 31, 2018, and any other SEC filings that Achillion makes from time to time.

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.

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ACHILLION PHARMACEUTICALS INC. (ACHN)
Statements of Operations
(Unaudited, in thousands, except per share amounts)

	Three Months Ended March 31,	
	2019	**2018**
Revenue	$ -	$ -
Operating expenses:		
Research and development	14,817	14,049
General and administrative	5,157	6,016
Restructuring charges	655	1,750
Total operating expenses	20,629	21,815
Loss from operations	(20,629)	(21,815)
Other income (expense):		
Interest income	1,681	1,239
Interest expense	(11)	(12)
Net loss	$ (18,959)	$ (20,588)

Net loss per share - basic and diluted	$ (0.14)	$ (0.15)
Weighted average shares outstanding - basic and diluted	138,716	138,014

Balance Sheets
(Unaudited, in thousands)

	March 31, 2019	December 31, 2018
Cash, cash equivalents and marketable securities	$ 254,068	$ 270,977
Working capital	245,911	263,551
Total assets	262,677	277,858
Long-term liabilities	559	17
Total liabilities	13,738	11,846
Total stockholders' (deficit) equity	248,939	266,012

Source: Achillion Pharmaceuticals, Inc.

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