Achillion Reports Second Quarter 2019 Financial Results and Provides Corporate Update

August 8, 2019

– Danicopan (ACH-4471) Phase 2 combination therapy interim data showed clinically meaningful improvements in laboratory parameters of PNH; End-of-Phase 2 meeting activities initiated with FDA –

– ACH-5228's near complete and sustained inhibition of the alternative pathway and its tolerability support US IND submission Q4 2019 –

– Cash and securities of $241.3 million as of June 30, 2019 –

BLUE BELL, Pa., Aug. 08, 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 and six months ended June 30, 2019 and provided a corporate update.

"We continued to advance our complement factor D inhibitors in the second quarter of 2019 by efficiently executing on our global development and regulatory strategies, while continuing to strengthen our intellectual property position. As we maintain discipline on our spending, we continue to increase our chemistry, manufacturing, and controls (CMC) capabilities in preparation for our expanding portfolio," said Joe Truitt, President and Chief Executive Officer at Achillion. "We believe our portfolio of oral factor D inhibitors has potential clinical benefits in multiple indications, and we look forward to initiating a clinical trial in a new indication in 2020, in addition to our Phase 3 clinical trial of danicopan for paroxysmal nocturnal hemoglobinuria (PNH) in early 2020."

Key Highlights and Development Updates:

Danicopan

- The ongoing Phase 2 PNH clinical trial assessing the safety and effectiveness of danicopan (ACH-4471) in combination with intravenous eculizumab is expected to be completed in the third quarter of 2019. Interim data, presented at The New Era of Aplastic Anemia and PNH Meeting in May 2019, demonstrated clinically meaningful improvements in laboratory parameters of PNH including hemoglobin, reticulocyte count, and bilirubin, as well as meaningful improvements in health-related quality of life FACIT-Fatigue scores relative to baseline scores on eculizumab. The addition of danicopan nearly eliminated the patients' needs for blood transfusions in this study. The full data set is expected in the fourth quarter of 2019. Dosing with ULTOMIRIS™ (ravulizumab) has been initiated in the extension study in combination with danicopan.
- The Company completed enrollment in the danicopan Phase 2 clinical trials for C3 glomerulopathy (C3G) and C3G/immune complex-mediated membrane glomerulonephritis (IC-MPGN). A total of 32 patients have been enrolled in the two trials; 13 C3G patients in the 6-month double-blind, placebo-controlled trial and 19 C3G/IC-MPGN patients in the 12-month single-arm open-label trial. Pending an analysis of the C3G data, the Company plans to meet with the U.S. Food and Drug Administration (FDA) for an End-of-Phase 2 meeting and the European Medicines Agency (EMA) for scientific advice, in 2020.

Next-generation Factor D Inhibitors

- Results from the completed ACH-5228 Phase 1 multiple ascending dose study demonstrated that ACH-5228, when dosed 120 mg twice a day (BID) or higher, achieved near complete and sustained alternative pathway (AP) inhibition with a mean value of >95% at steady state concentrations as measured by AP Hemolysis and AP Wieslab assays. In the study, ACH-5228 was generally well-tolerated over the dose ranges tested, which included the doses expected to be evaluated in Phase 2 clinical trials. The Company expects to submit an investigational new drug (IND) application to the FDA for ACH-5228 in the fourth quarter of 2019.
- The Company has identified a series of third-generation factor D inhibitors, with unique pharmacokinetic attributes, which are believed to provide additional optionality and durability for our factor D development program. The Company plans to nominate one of the third-generation factor D inhibitors for clinical development in 2020.

Second Quarter 2019 Financial Results

For the three months ended June 30, 2019, Achillion reported a net loss of $19.4 million compared with a net loss of $17.2 million during the same period of 2018. Research and development expenses were $15.9 million for the three months ended June 30, 2019, compared with $11.0 million for the same period of 2018. The increase was primarily due to increased clinical trial costs related to danicopan and ACH-5228, combined with increased manufacturing and formulation costs related to ACH-5228 and ACH-5548. These amounts were partially offset by decreased discovery research costs.

For the three months ended June 30, 2019, general and administrative expenses were $5.1 million, compared with $7.5 million incurred during the same period in 2018. The decrease was primarily due to decreased personnel costs and non-cash stock-based compensation charges related to the transition of our former chief executive officer in the second quarter of 2018.

Non-cash stock compensation expense totaled $1.9 million for the second quarter of 2019 as compared with $3.5 million for the second quarter of

2018 and is included in research and development expenses and general and administrative expenses.

Cash, cash equivalents and marketable securities as of June 30, 2019 was $241.3 million. The company expects net-cash spend for the full year of 2019 to be approximately $85 million, resulting in year-end 2019 cash, cash equivalents and marketable securities of approximately $185 million.

Six Month 2019 Financial Results

For the six months ended June 30, 2019, Achillion reported a net loss of $38.4 million, compared to a net loss of $37.8 million in the same period in 2018. For the six months ended June 30, 2019, research and development expenses totaled $30.8 million, compared with $25.1 million during the same period in 2018. The increase was primarily due to increased clinical trial costs related to danicopan and ACH-5228, combined with increased manufacturing and formulation costs related to ACH-5228 and ACH-5548. These amounts were partially offset by decreased discovery research costs.

General and administrative expenses were $10.3 million for the six months ended June 30, 2019, compared to $13.5 million in the same period in 2018. The decrease was primarily due to decreased personnel costs and non-cash stock-based compensation charges related to the transition of our former chief executive officer.

Non-cash stock compensation expense totaled $3.2 million for the six months ended June 30, 2019 as compared with $5.9 million for the same period in 2018 and is included in both research and development and general and administrative expenses.

About the Achillion Complement Factor D Portfolio
Achillion has leveraged its internal discovery capabilities and a novel complement-related platform to develop oral small molecule drug candidates that are inhibitors of complement factor D. Factor D is an essential serine protease involved in the alternative pathway (AP) of the complement system, a part of the innate immune system. Achillion's complement platform is focused on seeking to advance oral small molecules that inhibit the AP and can potentially be used in the treatment of immune-related diseases in which complement AP plays a critical role. Potential indications currently being evaluated for these compounds include paroxysmal nocturnal hemoglobinuria (PNH), C3 glomerulopathy (C3G), and immune complex-mediated membranoproliferative glomerulonephritis (IC-MPGN).

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 significant unmet medical needs persist despite existing therapies. 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. In its efforts to advance its investigational product candidates into registrational clinical trials and commercialization, the Company plans to work closely with key stakeholders including patients, payors, regulators, and healthcare providers.

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 include statements about: the potential benefits of factor D inhibition as a treatment for complement-mediated diseases, including danicopan (ACH-4471) for PNH; the potential benefits of, and indications for, Achillion's compounds that inhibit factor D, including danicopan and ACH-5228; 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 (including danicopan and ACH-5228) as well as its ability to advance additional compounds; Achillion's expectations regarding the timing of regulatory interactions and filings; 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, including danicopan and ACH-5228; 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; whether interim results from a clinical trial will be predictive of the final results of that trial or whether results of early clinical trials or preclinical studies will be indicative of the results of later clinical trials; enroll patients in its clinical trials on its projected timelines; 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 Quarterly Report on Form 10-Q for the quarterly period ended March 31, 2019, 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 June 30,		Six Months Ended June 30,	
	2019	**2018**	**2019**	**2018**
Revenue	$ -	$ -	$ -	$ -
Operating expenses:				
Research and development	15,932	11,023	30,750	25,072
General and administrative	5,109	7,463	10,266	13,478
Restructuring charges	-	75	655	1,825
Total operating expenses	21,041	18,561	41,671	40,375
Loss from operations	(21,041)	(18,561)	(41,671)	(40,375)
Other income (expense):				
Interest income	1,621	1,370	3,302	2,609
Interest expense	(6)	(8)	(16)	(21)
Net loss	(19,426)	(17,199)	(38,385)	(37,787)
Net loss per share - basic and diluted	$ (0.14)	$ (0.12)	$ (0.28)	$ (0.27)
Weighted average shares outstanding - basic and diluted	138,761	138,426	138,739	138,221

Balance Sheets
(Unaudited, in thousands)

	June 30, 2019	December 31, 2018
Cash, cash equivalents and marketable securities	$ 241,305	$ 270,977
Working capital	221,286	263,551
Total assets	248,375	277,858
Long-term liabilities	510	17
Total liabilities	16,732	11,846
Total stockholders' equity	231,643	266,012

Source: Achillion Pharmaceuticals, Inc.

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