Achillion Reports Third Quarter 2019 Financial Results and Provides Corporate Update

November 7, 2019

– Reached an agreement to be acquired by Alexion;
transaction expected to close in first half of 2020 –

– Danicopan (ACH-4471) received Breakthrough Therapy designation for treatment of PNH;
Phase 2 combination trial top-line results to be presented at 61st ASH Annual Meeting –

– ACH-5228 Phase 1 MAD study completed;
Phase 2 trial initiation planned for first half of 2020 –

– Cash and securities of $229.0 million as of September 30, 2019;
projected Year-End Cash and securities of $200 million –

BLUE BELL, Pa., Nov. 07, 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 nine months ended September 30, 2019 and provided a corporate update.

"In the third quarter of 2019 we reached a number of important milestones as part of our mission to bring oral complement factor D inhibitors to patients in need," said Joe Truitt, President and Chief Executive Officer at Achillion. "We look forward to sharing the top-line results from the Phase 2 PNH combination study at the upcoming American Society of Hematology (ASH) Annual Meeting and initiating our Phase 3 program for danicopan in early 2020. Additionally, we continue to advance our second-generation oral factor D inhibitor, ACH-5228, with a planned Phase 2 PNH clinical trial initiation in the first half of 2020."

As previously announced on October 16, 2019, Achillion entered into a definitive agreement to be acquired by Alexion Pharmaceuticals, Inc. (NASDAQ: ALXN). The initial consideration comprises $6.30 per share in cash for each share of Achillion common stock, and the transaction includes the potential for additional consideration in the form of non-tradeable contingent value rights, which will be paid to Achillion stockholders if certain clinical and regulatory milestones are achieved within specified periods.

Mr. Truitt continued, "We are making progress on the pending acquisition of Achillion by Alexion Pharmaceuticals, which is on track to close in the first half of 2020. Looking ahead to our future with Alexion, we expect the full potential for our portfolio of small-molecule complement inhibitors will be leveraged to accelerate our objective of bringing novel therapies to market faster to improve the lives of patients."

Key Highlights and Development Updates:

Danicopan

- The U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy designation for danicopan (ACH-4471) for treatment in combination with a C5 monoclonal antibody for patients with paroxysmal nocturnal hemoglobinuria (PNH) who are sub-optimal responders to a C5 inhibitor alone. The FDA's decision was based on interim data from the Company's Phase 2 clinical trial assessing the safety and effectiveness of danicopan in combination with intravenous eculizumab. The top-line results from the Phase 2 clinical trial of danicopan in combination with eculizumab will be presented at the 61st ASH Annual Meeting. Global regulatory discussions are ongoing with initiation of a Phase 3 program for danicopan planned for early 2020.
- The Company completed enrollment in the danicopan Phase 2 clinical trials for C3 glomerulopathy (C3G) and C3G/immune complex-mediated membrane glomerulonephritis (IC-MPGN). In the third quarter of 2019, three adolescent patients were enrolled in the 12-month single-arm open-label trial. A total of 35 patients have been enrolled in the two trials. Pending an analysis of the C3G data, the Company plans to meet with the FDA for an End-of-Phase 2 meeting and the European Medicines Agency (EMA) for scientific advice in 2020.
- Further evaluation of pharmacokinetic and pharmacodynamic biomarker data from the Phase 2 PNH monotherapy study for untreated PNH patients was accepted for poster presentation at the 61st ASH Annual Meeting.

ACH-5228

- 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 plans to initiate Phase 2 development of ACH-5228 in PNH in early 2020.

Third-generation Factor D Inhibitors

- The Company continues to evaluate 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 its third-generation factor D inhibitors for clinical development in 2020.

Pending Acquisition of Achillion by Alexion

On October 15, 2019, Achillion entered into an Agreement and Plan of Merger, or Merger Agreement with Alexion Pharmaceuticals, Inc., a Delaware corporation, or Alexion, and Beagle Merger Sub, Inc., a Delaware corporation and wholly owned subsidiary of Alexion, or Merger Sub. Pursuant to the Merger Agreement, on the terms and subject to the conditions thereof, Merger Sub will merge with and into Achillion, which we refer to herein as the Merger, with Achillion surviving the Merger as a wholly owned subsidiary of Alexion. The initial consideration of approximately $930 million, or $6.30 per share of our common stock, will be funded by Alexion from cash on hand. The transaction includes the potential for additional consideration in the form of non-tradeable contingent value rights, or CVRs, which will pay our shareholders up to $2.00 per share if certain clinical and regulatory milestones are achieved within specified periods. These include $1.00 per share for FDA approval of danicopan within fifty-four months of the closing date of the transaction and $1.00 per share for ACH-5228 Phase 3 initiation within four years of the closing date of the transaction. Alexion's acquisition of the Company is subject to the approval of Company shareholders and satisfaction of customary closing conditions and approval from relevant regulatory agencies, including clearance under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended. Subject to the satisfaction of these conditions, the parties expect the transaction to close in the first half of 2020. Additional information related to the Merger Agreement is set forth in our Current Report on Form 8-K filed with the SEC on October 16, 2019, which includes the full text of the Merger Agreement as Exhibit 2.1.

Third Quarter Financial Results

For the three months ended September 30, 2019, Achillion reported a net loss of $19.6 million compared with a net loss of $15.9 million during the same period of 2018. Research and development expenses were $15.0 million for the three months ended September 30, 2019, compared with $12.8 million for the same period of 2018. The increase for the three months ended September 30, 2019 was primarily due to increased manufacturing and formulation costs related to ACH-5228, partially offset by decreased clinical trial costs for danicopan.

For the three months ended September 30, 2019, general and administrative expenses were $6.1 million, compared with $4.4 million incurred during the same period in 2018. The increase for the three months ended September 30, 2019 was primarily due to increased legal fees combined with market research related professional fees.

Non-cash stock compensation expense totaled $1.7 million for the third quarter of 2019 as compared with $1.6 million for the third quarter of 2018 and is included in research and development expenses and general and administrative expenses.

Cash, cash equivalents and marketable securities as of September 30, 2019 was $229.0 million. The company expects year-end 2019 cash, cash equivalents and marketable securities of approximately $200 million.

Nine Month Financial Results

For the nine months ended September 30, 2019, Achillion reported a net loss of $58.0 million, compared to a net loss of $53.7 million in the same period in 2018. For the nine months ended September 30, 2019, research and development expenses totaled $45.7 million, compared with $37.9 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 danicopan and ACH-5228.

General and administrative expenses were $16.3 million for the nine months ended September 30, 2019, compared to $17.9 million in the same period in 2018. The decrease for the nine months ended September 30, 2019 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 $4.9 million for the nine months ended September 30, 2019 as compared with $7.5 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 potential benefits of Achillion's third generation factor D inhibitors and its plans to nominate a third generation inhibitor for clinical development; 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; the proposed acquisition of Achillion by Alexion, including the potential contractual contingent value right payment by Alexion in connection with the acquisition; 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. Risks and uncertainties related to the proposed acquisition of Achillion by Alexion include, among other things, risks related to the fact that the proposed acquisition may not be completed due to Achillion's or Alexion's failure to satisfy or waive the conditions to closing the proposed acquisition (including the failure to obtain necessary regulatory approvals) in the anticipated timeframe or at all, including uncertainties as to whether Achillion's stockholders will approve the Merger and the possibility that the acquisition does not close; the possibility that competing offers may be made; risks related to obtaining the requisite consents to the acquisition, including, without limitation, the timing (including possible delays) and receipt of regulatory approvals from various governmental entities (including any conditions, limitations or restrictions placed on these approvals and the risk that one or more governmental entities may deny approval); the risk that the businesses will not be integrated successfully; disruption from the transaction making it more difficult to maintain business and operational relationships; negative effects of the announcement or the consummation of the proposed acquisition on the market price of Alexion's common stock, Alexion's credit ratings and/or Alexion's operating results; significant transaction costs or unexpected expenses; unknown liabilities; the risk of litigation and/or regulatory actions related to the proposed acquisition.

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 June 30, 2019, the Current Report on Form 8-K filed with the SEC on October 16, 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.

Note to Investors and Security Holders

This document does not constitute a solicitation of any vote or approval. In connection with the proposed acquisition of Achillion by Alexion, Achillion intends to file with the SEC a definitive proxy statement, as well as other relevant documents concerning the proposed transaction. A preliminary proxy statement was filed with the SEC on November 5, 2019. INVESTORS AND SECURITY HOLDERS OF ACHILLION ARE URGED TO READ THE DEFINITIVE PROXY STATEMENT REGARDING THE PROPOSED TRANSACTION WHEN IT BECOMES AVAILABLE AND ANY OTHER RELEVANT DOCUMENTS FILED WITH THE SEC, AS WELL AS ANY AMENDMENTS OR SUPPLEMENTS TO THOSE DOCUMENTS, BECAUSE THEY WILL CONTAIN IMPORTANT INFORMATION. You may obtain these documents (when they become available) free of charge through the website maintained by the SEC at http://www.sec.gov. Copies of the documents filed with the SEC by Alexion will be available free of charge on Alexion's internet website at http://www.alexion.com under the tab, "Investors" and under the heading "SEC Filings" or by contacting Alexion's Investor Relations Department at investorrelations@alexion.com. Copies of the documents filed with the SEC by Achillion will be available free of charge on Achillion's internet website at http://www.achillion.com under the tab "Investors and News" and under the heading "SEC Filings" or by contacting Achillion's Investor Relations Department through http://ir.achillion.com/contact-us.

Certain Information Regarding Participants

Alexion, Achillion and their respective directors and executive officers may be considered participants in the solicitation of proxies in connection with the proposed transaction. Information about the directors and executive officers of Alexion is set forth in its Annual Report on Form 10-K for the year ended December 31, 2018, which was filed with the SEC on February 6, 2019, and its proxy statement for its May 14, 2019 annual meeting of stockholders, which was filed with the SEC on March 26, 2019. Information about the directors and executive officers of Achillion is set forth in its Annual Report on Form 10-K for the year ended December 31, 2018, which was filed with the SEC on March 7, 2019, and its proxy statement for its May 30, 2019 annual meeting of stockholders, which was filed with the SEC on April 15, 2019. Other information regarding the participants in the proxy solicitations and a description of their direct and indirect interests, by security holdings or otherwise, will be contained in the proxy statement and other relevant materials to be filed with the SEC regarding the proposed transaction when they become available.

Investor Relations:
Clayton Robertson
Achillion Pharmaceuticals, Inc.
Tel. 215-709-3078
crobertson@achillion.com

Media:
Susanne Heinzinger
Senior VP, Corporate Communications

Achillion Pharmaceuticals, Inc.
Tel. 215-709-3032
sheinzinger@achillion.com

Source: Achillion Pharmaceuticals, Inc.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	**2018**	**2019**	**2018**
Revenue	$ -	$ -	$ -	$ -
Operating expenses:				
Research and development	14,993	12,842	45,744	37,915
General and administrative	6,061	4,447	16,326	17,924
Restructuring charges	-	75	655	1,900
Total operating expenses	21,054	17,364	62,725	57,739
Loss from operations	(21,054)	(17,364)	(62,725)	(57,739)
Other income (expense):				
Interest income	1,473	1,484	4,775	4,093
Interest expense	(2)	(4)	(18)	(25)
Net loss	(19,583)	(15,884)	(57,968)	(53,671)
Net loss per share - basic and diluted	$ (0.14)	$ (0.12)	$ (0.42)	$ (0.39)
Weighted average shares outstanding - basic and diluted	139,589	138,586	139,025	138,344

Balance Sheets
(Unaudited, in thousands)

	September 30, 2019	**December 31, 2018**
Cash, cash equivalents and marketable securities	$ 228,962	$ 270,977
Working capital	140,232	263,551
Total assets	236,342	277,858
Long-term liabilities	1,518	17
Total liabilities	19,126	11,846
Total stockholders' equity	217,216	266,012

Source: Achillion Pharmaceuticals, Inc.