



## Achillion Reports Third Quarter 2018 Financial Results

November 7, 2018

- Completed enrollment of Phase 2 clinical trial of ACH-4471 in PNH as monotherapy -
- Interim data and strategic update planned for December 17, 2018 –
- September 30, 2018 cash & securities position of \$283 million –
- Mary Kay Fenton to step down as CFO at year-end 2018 -

NEW HAVEN, Conn., Nov. 07, 2018 (GLOBE NEWSWIRE) -- **Achillion Pharmaceuticals, Inc. (Nasdaq: ACHN)**, a clinical-stage biopharmaceutical company focused on advancing its oral factor D inhibitors into late-stage development and commercialization, today reported financial results for the three and nine months ended September 30, 2018.

"In my first full quarter as CEO, our priorities have been to strengthen the leadership team, improve clinical execution and create a data-driven, patient-centric portfolio strategy. This quarter we added several experienced executives including, Paul Firuta as Chief Operating Officer, Dr. Steven Zelenkofske as Chief Medical Officer, Anthony Gibney as Chief Business Officer, and Dr. Kevin Malobisky as Senior Vice President Regulatory Affairs, Quality & Compliance. We continue to build development capabilities and therapeutic area expertise as we advance Achillion from a discovery and early-stage development organization to a patient-focused, late-stage development company. We have made these changes while reducing overall headcount and our projected cash-burn rate for the full year 2018 to less than \$65 million," said Joe Truitt, President and Chief Executive Officer.

"In the third quarter we completed enrollment in our Phase 2 PNH monotherapy trial of ACH-4471, initiated ten additional clinical trial sites to support our C3G development program, and commenced development of a comprehensive portfolio strategy that we plan to communicate to investors in December 2018. We also plan to provide interim data on all open-label ACH-4471 clinical trials as well as Phase 1 safety and pharmacokinetics interim data from the single ascending dose trials with our second-generation factor D compounds, ACH-5228 and ACH-5548," Truitt noted. "We believe our innovative and differentiated factor D inhibitors have the potential to address unmet medical needs in patients with PNH and C3G as well as additional complement mediated diseases, together representing significant value-creation opportunities."

Truitt continued, "Additionally, after 18 years with Achillion, Mary Kay Fenton has decided to step down from Achillion and her role as Chief Financial Officer effective year end 2018. Mary Kay joined Achillion at its inception and during her tenure has played a leadership role in establishing and growing the company through business development deals and a number of capital market transactions including Achillion's IPO and listing on Nasdaq. On behalf of the entire company, I would like to thank Mary Kay for her many significant contributions. Her expertise in finance and operations were instrumental in transforming Achillion from a research organization into a clinical stage development company."

"Achillion has talented employees, a robust balance sheet and exciting prospects in its clinical development programs, and I look forward to its continued success," said Fenton. "I am also excited to explore new opportunities in the next phase of my career."

### Third Quarter Financial Results

For the three months ended September 30, 2018, Achillion reported a net loss of \$15.9 million compared with a net loss of \$19.3 million during the same period of 2017. Cash, cash equivalents, marketable securities, and interest receivable as of September 30, 2018 was \$283.1 million compared with \$295.8 million as of June 30, 2018. Research and development expenses were \$13.1 million for the three months ended September 30, 2018, compared with \$15.6 million for the same period of 2017. The decrease for the three months ended September 30, 2018 was primarily due to decreased manufacturing and formulation costs related to ACH-5228 and decreased discovery research costs related to the Company's intravitreal factor D inhibitors, combined with decreased personnel costs and non-cash stock compensation. These amounts were partially offset by increased clinical trial costs related to ACH-4471 and preclinical costs related to ACH-5548.

For the three months ended September 30, 2018, general and administrative expenses were \$4.2 million, compared with \$4.8 million incurred during the same period in 2017. The decrease for the three months ended September 30, 2018 was primarily due to decreased non-cash stock compensation expense related to the Company's former chief executive officer combined with fewer employees.

Non-cash stock compensation expense totaled \$1.6 million for the three months ended September 30, 2018 as compared with \$2.4 million for the same period in 2017 and is included in research and development expenses and general and administrative expenses.

### Nine Month Financial Results

For the nine months ended September 30, 2018, Achillion reported a net loss of \$53.7 million, compared to a net loss of \$62.0 million in the same period in 2017. For the nine months ended September 30, 2018, research and development expenses totaled \$39.3 million, compared with \$49.4 million during the same period in 2017. The decrease for the nine months ended September 30, 2018 was primarily due to decreased manufacturing and formulation costs related to ACH-5228 and decreased discovery research costs related to the Company's intravitreal factor D inhibitors, combined with decreased personnel costs and non-cash stock compensation due to fewer employees, including fewer executives. These amounts were partially offset by increased clinical trial costs related to ACH-4471 and preclinical costs related to ACH-5548.

General and administrative expenses were \$16.6 million for the nine months ended September 30, 2018, compared to \$15.9 million in the same period in 2017. The increase for the nine months ended September 30, 2018 was primarily due to increased personnel and non-cash stock-based compensation charges related to the transition of the Company's former chief executive officer, partially offset by decreased consulting fees.

Non-cash stock compensation expense totaled \$7.5 million for the nine months ended September 30, 2018 as compared with \$8.4 million for the same period in 2017 and is included in research and development, general and administrative, and restructuring expenses.

The Company expects that research and development expense during the fourth quarter of 2018 will increase slightly as a result of increased clinical

trial activity, and that general and administrative expenses during the fourth quarter of 2018 will be consistent with prior 2018 quarters. Annual total research and development expense is expected to be in the range of \$55 to \$58 million and annual total general and administrative expense is expected to be in the range of \$22 to \$24 million. Annual cash utilization, which excludes non-cash expenses, is expected to be between approximately \$62 to \$65 million.

#### **ACH-4471, Complement Factor D Inhibitor for PNH and C3G**

Achillion's first generation oral factor D inhibitor, ACH-4471 is being evaluated for safety and efficacy with Phase 2 clinical programs in both paroxysmal nocturnal hemoglobinuria (PNH) and C3 glomerulopathy (C3G).

The PNH program consists of two trials: A Phase 2 clinical trial in untreated PNH patients where ACH-4471 is being assessed as a monotherapy; the second trial is Phase 2 clinical trial evaluating ACH-4471 in patients who are inadequately controlled or sub-optimally responding to eculizumab, which is a therapy for patients with PNH.

The C3G program consists of three currently recruiting Phase 2 clinical trials: a 14-day biomarker study, 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/>

#### **ACH-5228 and ACH-5548, Complement Factor D Inhibitors**

ACH-5228 and ACH-5548 are next-generation oral factor D inhibitors currently in Phase 1 clinical trials. In preclinical studies, these compounds demonstrated enhanced potency as well as improved pharmacokinetic properties that may allow for a reduced dosing frequency.

Achillion plans to provide interim data on all open-label ACH-4471 clinical trials as well as Phase 1 safety and pharmacokinetics interim data from the single ascending dose trials of its next-generation factor D compounds, ACH-5228 and ACH-5548, on December 17, 2018, more details to follow.

#### **About Achillion Pharmaceuticals**

Achillion Pharmaceuticals, Inc. (Nasdaq: ACHN) is a clinical-stage biopharmaceutical company focused on advancing its oral factor D inhibitors into late-stage development and commercialization. Factor D is an essential serine protease involved in the alternative pathway of the complement system, a part of the innate immune system. Achillion is initially focusing its drug development activities on alternative pathway-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 mediated membranoproliferative glomerulonephritis (IC-MPGN). Each of the product candidates in the Company's oral factor D 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 patients, payors, regulators and healthcare professionals. 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," "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; 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; 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; 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 fiscal quarter ended June 30, 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)**

	<b>Three Months Ended September 30,</b>		<b>Nine Months Ended September 30,</b>	
	<b>2018</b>	<b>2017</b>	<b>2018</b>	<b>2017</b>
Revenue	\$ -	\$ -	\$ -	\$ -
Operating expenses:				
Research and development	13,137	15,620	39,279	49,368
General and administrative	4,152	4,843	16,560	15,853
Restructuring charges	75	-	1,900	-
Total operating expenses	17,364	20,463	57,739	65,221
Loss from operations	(17,364 )	(20,463 )	(57,739 )	(65,221 )
Other income (expense):				
Interest income	1,484	1,133	4,093	3,225
Interest expense	(4 )	(8 )	(25 )	(37 )
Net loss	(15,884 )	(19,338 )	(53,671 )	(62,033 )
Net loss per share - basic and diluted	\$ (0.12 )	\$ (0.14 )	\$ (0.39 )	\$ (0.45 )
Weighted average shares outstanding - basic and diluted	138,586	137,375	138,344	136,947

**Balance Sheets****(Unaudited, in thousands)**

	<b>September 30, 2018</b>	<b>December 31, 2017</b>
Cash, cash equivalents, marketable securities and interest receivable	\$ 283,138	\$ 331,845
Working capital	277,810	291,054
Total assets	289,177	337,613
Long-term liabilities	47	214
Total liabilities	8,671	13,098
Total stockholders' equity	280,506	324,515



Source: Achillion Pharmaceuticals, Inc.