



## Achillion Reports Fourth Quarter and Full Year 2018 Financial Results

March 7, 2019

- Phase 2 trial enrollment on schedule in PNH combo, interim data expected in Q2:2019 -
- Phase 2 trial enrollment on schedule in C3G, 24 sites recruiting -
- Next-generation factor D inhibitor ACH-5228 US IND anticipated in Q4:2019 -
- Reduced 2018 net cash spend to \$60 million, projecting \$80 million in 2019 -
- Well funded with cash and securities of \$271 million at December 31, 2018 -

BLUE BELL, Pa., March 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 financial results for the three and twelve months ended December 31, 2018 and provided a corporate update.

"We made significant progress advancing our portfolio of oral factor D inhibitors in the fourth quarter of 2018," said Joe Truitt, President and Chief Executive Officer at Achillion. "ACH-4471 has demonstrated preliminary proof-of-concept in patients with paroxysmal nocturnal hemoglobinuria (PNH) and C3 glomerulopathy (C3G). We look forward to reporting updated interim results for our Phase 2 trial of ACH-4471 for PNH, in combination with a C5 inhibitor, in the second quarter of 2019. We plan to present data for the PNH and C3G trials to the U.S. Food and Drug Administration (FDA) in the fourth quarter of 2019. We also remain on track to complete the ongoing ex-US Phase 1 multiple ascending dose (MAD) study for our more potent next-generation oral factor D inhibitor, ACH-5228. We anticipate submitting an Investigational New Drug (IND) Application in the US for ACH-5228 in the fourth quarter of 2019."

Mr. Truitt continued, "We took several important steps to strengthen our leadership team and expand the Company's capabilities from a discovery engine to a clinical-stage organization in 2018. Additionally, we were pleased to announce that the Board of Directors recently elected Nicole Vitullo as Chair and we added Brian Di Donato as Senior Vice President and Chief Financial Officer to our executive management team. We also expanded our operations into the Philadelphia area which adds access to a strong talent pool for future growth. We remain well positioned to advance our first- and next-generation oral factor D inhibitors in patients with serious medical conditions. In 2019, we will continue to focus on expanding our clinical operations for PNH and C3G. We now have 24 trial sites recruiting patients for C3G. We also plan to evaluate the potential of our oral small molecule complement inhibitors in additional indications to deliver on the promise of complement inhibition across a wide spectrum of diseases."

### Key 2019 Planned Milestones

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

- The Company expects to report updated interim results for its Phase 2 open-label combination trial of ACH-4471 for PNH, with C5 inhibitor (eculizumab), in the second quarter of 2019 and is targeting an FDA meeting in the fourth quarter of 2019.
- The Company plans to present data from its Phase 2 clinical trials of ACH-4471 for C3G and immune complex membranoproliferative glomerulonephritis (IC-MPGN) to the FDA in the fourth quarter of 2019.

- **ACH-5228, Next Generation Complement Factor D Inhibitor**

- The Company recently initiated a Phase 1 MAD study of ACH-5228 and expects to complete the study and submit a US IND Application in the fourth quarter of 2019.

### Fourth Quarter 2018 Financial Results

For the three months ended December 31, 2018, the Company reported a net loss of \$16.6 million, compared to a net loss of \$23.2 million in the three months ended December 31, 2017.

Research and development expenses were \$12.2 million for the three months ended December 31, 2018, compared to \$15.7 million for the same period of 2017. The decrease was primarily due to decreased personnel costs due to fewer employees combined with decreased discovery research costs related to ACH-5228. These amounts were partially offset by increased manufacturing expenses for ACH-5228.

For the three months ended December 31, 2018, general and administrative expenses totaled \$6.0 million, compared to \$8.7 million for the same period of 2017. The decrease was primarily the result of the Company's payment of the underwriting fees in connection with the public resale by the Johnson and Johnson Development Corporation (JJDC) in November 2017 of all the shares of common stock it acquired from the Company in 2015.

### Year-end 2018 Financial Results

For the year ended December 31, 2018, the Company's net loss was \$70.3 million, or \$0.51 per share, compared to a net loss of \$85.2 million, or \$0.62 per share, for the year ended December 31, 2017.

For the year ended December 31, 2018, research and development expenses totaled \$50.1 million, compared to \$63.6 million for the year ended December 31, 2017. The decrease was primarily due to manufacturing and formulation costs related to ACH-5228, discovery research costs, and personnel costs and non-cash stock compensation due to fewer employees. These amounts were partially offset by increased clinical trial costs related to ACH-4471 and increased pre-clinical costs related to ACH-5548.

General and administrative expenses were \$23.9 million for the year ended December 31, 2018, compared to \$26.0 million for the year ended

December 31, 2017. The decrease was primarily due to decreased corporate legal fees and consulting fees combined with the Company's payment of \$2.9 million in underwriting fees in connection with the sale by JJDC in November 2017 of all of the shares of the Company's common stock it acquired pursuant to the JJDC Stock Purchase Agreement. These amounts were partially offset by an increase in personnel and non-cash stock-based compensation charges related to the transition of the Company's former chief executive officer and former chief financial officer.

Cash, cash equivalents, and marketable securities at December 31, 2018 were \$271.0 million.

## Financial Guidance

The company expects net cash usage for 2019 will be approximately \$80 million based on current operating plans, anticipated timelines and the estimated costs of clinical trials and product development programs.

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

Achillion's first-generation oral complement factor D inhibitor, ACH-4471, is being evaluated for safety and efficacy with Phase 2 clinical programs in both PNH and C3G and has demonstrated preliminary proof-of-concept in both indications. The PNH program consists of a Phase 2 clinical trial evaluating ACH-4471 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 currently recruiting, 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 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 system inhibitors into late-stage development and commercialization. Each of the product candidates in the Company's portfolio was discovered in its laboratories and is wholly owned. 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. Initial indications being evaluated for its compounds include paroxysmal nocturnal hemoglobinuria (PNH), C3 glomerulopathy (C3G), and immune complex membranoproliferative glomerulonephritis (IC-MPGN). To advance its investigational drugs into phase 3 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," "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 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; 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; 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 September 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**  
(in thousands, except per share amounts)

	Three Months Ended December 31,		Year Ended December 31,	
	2018	2017	2018	2017
Revenue	\$ -	\$ -	\$ -	\$ -
Operating expenses:				
Research and development	12,202	15,411	50,118	63,607
General and administrative	5,972	8,944	23,896	25,969
Restructuring charges	-	-	1,900	-
Total operating expenses	18,174	24,355	75,914	89,576
Loss from operations	(18,174 )	(24,355 )	(75,914 )	(89,576 )
Other income (expense):				
Interest income	1,585	1,165	5,678	4,390
Interest expense	(12 )	(13 )	(36 )	(50 )
Net loss	\$ (16,601 )	\$ (23,203 )	\$ (70,272 )	\$ (85,236 )
Net loss per share - basic and diluted	\$ (0.12 )	\$ (0.17 )	\$ (0.51 )	\$ (0.62 )
Wtd avg shares outstanding - basic and diluted	138,638	137,870	138,418	137,180

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

	December 31, 2018	December 31, 2017
Cash, cash equivalents, and marketable securities	\$ 270,977	\$ 330,585
Working capital	263,551	291,054
Total assets	277,858	337,613
Long-term liabilities	17	214
Total liabilities	11,846	13,098
Total stockholders' equity	266,012	324,515



Source: Achillion Pharmaceuticals, Inc.