



## Achillion Reports Second Quarter 2018 Financial Results

August 8, 2018

- Global phase 2 clinical programs for ACH-4471 continue to expand in PNH and C3G -
- Completed dosing phase 1 clinical study of ACH-5228 and initiated phase 1 study of ACH-5548 -
- June 30, 2018 cash position \$295.8 million -

NEW HAVEN, Conn., Aug. 08, 2018 (GLOBE NEWSWIRE) -- **Achillion Pharmaceuticals, Inc. (NASDAQ: ACHN)**, a clinical-stage biopharmaceutical company focused on advancing its orally administered complement system inhibitors into late-stage development and commercialization, today reported financial results for the three and six months ended June 30, 2018.

"We believe our highly innovative and differentiated factor D inhibitors have the potential to address not only PNH and C3G, but multiple diseases of the alternative pathway, and represent a significant value-creation opportunity. In order to build this value, in my first quarter as CEO, we have focused on beginning to transition Achillion from a discovery and early-stage clinical organization to a patient-focused, late-stage development biopharmaceutical company," said Joseph Truitt, President and Chief Executive Officer.

"My immediate priorities have been in three important areas: strengthening the team, clinical execution, and creating a cohesive, data-driven and patient-centric strategy. Over the next few weeks, we will be announcing important additions to the Achillion management team and we believe this team will position us to drive clinical execution and our portfolio strategy."

Truitt continued, "I am pleased that we've recently opened clinical trial sites in the US, EU, UK, Australia, New Zealand and South Korea and are enrolling patients in our ACH-4471 clinical trials. Based on our updated clinical plans, we now expect to provide data and a strategic update in December 2018 which will include all four open-label clinical trials with ACH-4471, including the 14-day and 12-month C3G trials, the PNH monotherapy and PNH add-on therapy trials, as well as phase 1 single ascending dose trials with ACH-5228 and ACH-5548."

### Second Quarter Financial Results

For the three months ended June 30, 2018, Achillion reported a net loss of \$17.2 million compared with a net loss of \$22.5 million during the same period of 2017. Cash, cash equivalents, marketable securities, and interest receivable as of June 30, 2018 was \$295.8 million compared with \$308.4 million as of March 31<sup>st</sup>, 2018. Research and development expenses, exclusive of restructuring, were \$11.4 million for the three months ended June 30, 2018, compared with \$18.3 million for the same period of 2017. The decrease was primarily due to decreased personnel and non-cash stock compensation expenses, due to fewer employees, combined with decreased manufacturing and formulation costs related to ACH-5228 and decreased discovery research costs related to our intravitreal factor D inhibitors. 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 June 30, 2018, general and administrative expenses were \$7.1 million, compared with \$5.4 million incurred during the same period in 2017. The increase was primarily due to increased personnel and non-cash stock-based compensation charges related to the transition of our former chief executive officer, partially offset by decreased consulting fees.

Non-cash stock compensation expense totaled \$3.5 million for the second quarter of 2018 as compared with \$2.8 million for the second quarter of 2017 and is included in research and development expenses and general and administrative expenses.

### Six Month Financial Results

For the six months ended June 30, 2018, Achillion reported a net loss of \$37.8 million, compared to a net loss of \$42.7 million in the same period in 2017. For the six months ended June 30, 2018, research and development expenses totaled \$26.1 million, compared with \$33.7 million during the same period in 2017. The decrease was primarily due to decreased personnel and non-cash stock compensation expenses, due to fewer employees, combined with decreased manufacturing and formulation costs related to ACH-5228 and decreased discovery research costs related to our intravitreal factor D inhibitors. 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 \$12.4 million for the six months ended June 30, 2018, increased from \$11.0 million in the same period in 2017. The increase was primarily due to increased personnel and non-cash stock-based compensation charges related to the transition of our former chief executive officer, partially offset by decreased consulting fees.

Non-cash stock compensation expense totaled \$5.9 million for the six months ended June 30, 2018 as compared with \$6.0 million for the same period in 2017, and is included in both research and development and general and administrative expenses.

The Company expects that research and development expense during the second half of 2018 will increase somewhat, consistent with previous guidance, and that general and administrative expenses will be consistent with the first half of the year. Annual total research and development expense is expected to be in the range of \$55-60 million and annual total general and administrative expense in the range of \$22-24 million. Annual cash utilization, which excludes non-cash expenses, is expected to be approximately \$62-65 million.

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

The first of Achillion's oral factor D inhibitors, ACH-4471, has active phase 2 clinical programs in both paroxysmal nocturnal hemoglobinuria (PNH) and C3 glomerulopathy (C3G). In PNH, the Company is advancing ACH-4471 in both naïve patients as monotherapy, and in patients treated with, but sub-optimally responding to, eculizumab. Achillion anticipates reporting interim data on these clinical trials in December 2018.

In C3G, the Company is advancing three clinical trials – a 14-day biomarker study, a six-month blinded, placebo-controlled study, and a 12-month open label study. Achillion anticipates reporting interim data on the 14-day and 12-month clinical trials in December 2018. More information is available

at <http://www.achillion.com/for-patients>.

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

Both ACH-5228 and ACH-5548 are next-generation factor D inhibitors being developed for oral administration. These compounds have demonstrated enhanced potency as well as optimized pharmacokinetic properties that may allow for reduced frequency of dosing. Both compounds are in phase 1 clinical assessment. Achillion anticipates reporting data on these compounds in December 2018.

### **About the Achillion Complement Factor D Platform**

Achillion has leveraged its internal discovery capabilities and a novel complement-related platform to develop small molecule drug candidates that are oral inhibitors of complement factor D. Factor D is an essential serine protease involved in the complement pathway, a part of the innate immune system. Achillion's complement platform is focused on seeking to advance small molecule compounds that inhibit factor D and can potentially be used in the treatment of immune-related diseases in which complement alternative pathway plays a critical role. Potential indications 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 factor D inhibitors into late-stage development and commercialization. Each of the drug candidates in the Company's oral factor D portfolio was discovered in its laboratories and is wholly owned. Achillion is focusing its drug development activities on alternative pathway-mediated, rare diseases where there are no approved therapies or where existing therapies are inadequate for patients. 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," "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 or provide strategic optionality; 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 drug 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 drug 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 March 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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-- Financial Tables Attached --

## **ACHILLION PHARMACEUTICALS INC. (ACHN)**

### **Statements of Operations**

**(Unaudited, in thousands, except per share amounts)**

<b>Three Months Ended</b>		<b>Six Months Ended</b>	
<b>June 30,</b>		<b>June 30,</b>	
<b>2018</b>	<b>2017</b>	<b>2018</b>	<b>2017</b>

Revenue	\$ -	\$ -	\$ -	\$ -
Operating expenses:				
Research and development	11,392	18,253	26,142	33,747
General and administrative	7,094	5,363	12,408	11,011
Restructuring charges	75	-	1,825	-
Total operating expenses	18,561	23,616	40,375	44,758
Loss from operations	(18,561 )	(23,616 )	(40,375 )	(44,758 )
Other income (expense):				
Interest income	1,370	1,085	2,609	2,092
Interest expense	(8 )	(12 )	(21 )	(29 )
Net loss	(17,199 )	(22,543 )	(37,787 )	(42,695 )
Net loss per share - basic and diluted	\$ (0.12 )	\$ (0.16 )	\$ (0.27 )	\$ (0.31 )
Weighted average shares outstanding - basic and diluted	138,426	136,736	138,221	136,729

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

	<b>June 30, 2018</b>	<b>December 31, 2017</b>
Cash, cash equivalents, marketable securities and interest receivable	\$ 295,827	\$ 331,845
Working capital	283,778	291,054
Total assets	302,789	337,613
Long-term liabilities	103	214
Total liabilities	8,230	13,098
Total stockholders' equity	294,559	324,515

 [Primary Logo](#)

Source: Achillion Pharmaceuticals, Inc.