



For Immediate Release

Aquinox Pharmaceuticals Announces Year End 2017 Financial Results

VANCOUVER, British Columbia, March 12, 2018 -- [Aquinox Pharmaceuticals](#), Inc. ("Aquinox") (NASDAQ:AQXP), a clinical-stage pharmaceutical company discovering and developing novel drug candidates to treat inflammation, inflammatory pain, and blood cancers, today provided a corporate update and reported financial results for the year ending December 31, 2017.

"We are excited to have completed enrollment in our LEADERSHIP 301 clinical trial of rosiptor in interstitial cystitis/bladder pain syndrome (IC/BPS), with the hope of showing that rosiptor demonstrates efficacy and tolerability in these patients," said David Main, President & CEO of Aquinox. "We recognize that there is a great need for new treatment options in IC/BPS. We remain on track and anticipate releasing topline data in the third quarter of 2018."

Recent Business Highlights & Upcoming Milestones

LEADERSHIP 301 Enrollment. On February 15, 2018, Aquinox completed enrollment in its LEADERSHIP 301 clinical trial of rosiptor (AQX-1125) in IC/BPS, with a total of 433 enrolled subjects, including 341 females and 92 males. Previously, on February 9, 2018, Aquinox announced that it had met its enrollment threshold of 300 females enrolled in LEADERSHIP 301.

Planned Initiation of Phase 2 Trial with Rosiptor in CP/CPPS. Aquinox is on track to initiate a Phase 2 proof-of-concept clinical trial with rosiptor in subjects with chronic prostatitis/chronic pelvic pain syndrome (CP/CPPS) in the second quarter of 2018. This 12-week, randomized, double-blind, placebo-controlled clinical trial will evaluate the efficacy and safety of once-daily rosiptor in approximately 100 male subjects with CP/CPPS. Study sites will be located in the US and Canada. CP/CPPS is characterized by pelvic pain, unrelated to urinary bladder filling or emptying, and lower urinary tract symptoms present for at least three months without evidence of urinary tract infection.

FDA BRUDAC Advisory Committee Meeting on IC/BPS. On December 7, 2018 the U.S. Food and Drug Administration (FDA) division of Bone, Reproductive, and Urologic Drugs (BRUDAC) held an Advisory Committee meeting specific to the topic of IC/BPS. At the meeting, the panel voted 15 to 0 that patients with interstitial cystitis and patients with bladder pain syndrome should be combined in clinical trials. This is aligned with the current American Urological Association (AUA) guideline and Aquinox's position that IC/BPS is a single symptom complex defined by bladder pain and urinary symptoms. Minutes from the meeting have since been published and are available online at <https://www.fda.gov/downloads/AdvisoryCommittees/CommitteesMeetingMaterials/Drugs/ReproductiveHealthDrugsAdvisoryCommittee/UCM594730.pdf>

Analyst and Investor Event. Aquinox hosted an analyst and investor event on February 9, 2018 in New York City, including a presentation from Dr. Philip Hanno, MD, MPH, clinical professor of urology at the Stanford University School of Medicine and co-chair of the Medical Advisory Board of the Interstitial Cystitis Association (ICA). Dr. Hanno shared his expert perspective on the current diagnosis and treatment landscape for patients with IC/BPS, and the need for effective treatment options for this patient population. Members of Aquinox management, Dr. Barbara Troupin and Ms. Abigail Jenkins, provided an update on Aquinox's clinical program in IC/BPS with rosiptor and the prospective commercial opportunity, respectively.

Summary of Financial Results

Cash Position. Cash, cash equivalents, short-term and long-term investments totaled \$108.1 million as of December 31, 2017, compared to \$153.1 million as of December 31, 2016. The decrease was primarily the result of the ongoing expenditures related to our LEADERSHIP 301 clinical trial in IC/BPS. Aquinox expects its cash, cash equivalents, and short-term investments to be sufficient for additional clinical development, manufacturing, preclinical, and pre-commercial and market assessment activities. Aquinox continues to expect that its cash-on-hand will carry it beyond topline data from the LEADERSHIP 301 trial and at least to mid-2019.

R&D Expenses. Research and development expenses for the year ended December 31, 2017 increased to \$36.3 million from \$28.4 million for the year ended December 31, 2016. This increase was primarily driven by increased clinical activities as Aquinox continued its LEADERSHIP 301 clinical trial with rosiptor in IC/BPS.

G&A Expenses. General and administrative expenses for the year ended December 31, 2017 increased to \$14.9 million from \$9.3 million for the year ended December 31, 2016. This increase was primarily driven by higher personnel related costs, professional fees, and pre-commercial and market assessment activities.

Net Loss. Net loss for the year ended December 31, 2017 was \$50.2 million compared to a net loss of \$37.0 million for the year ended December 31, 2016. This increase was primarily driven by increased operating expenditures as Aquinox continued its LEADERSHIP 301 clinical trial of rosiptor in IC/BPS.

Aquinox will host a conference call and live audio webcast on Monday, March 12, 2018 at 4:30 PM (ET)/ 1:30 PM (PT).

Conference Call and Webcast Details:

Date: Monday, March 12, 2018

Time: 4:30 PM (ET) / 1:30 PM (PT)

Toll-free: (866) 357-7878

International: (315) 625-3088

Audience Passcode 4665267

Webcast URL: <https://edge.media-server.com/m6/p/9vvkbdes>

The live webcast may be accessed through the "[Events & Presentations](#)" page in the "[Investor Relations](#)" section of the company's website at www.aqxpharma.com. The archived webcast will

also be available on Aquinox's website approximately two hours after the event and will be available for replay for at least 30 days after the event.

About Interstitial Cystitis/Bladder Pain Syndrome

Interstitial cystitis/bladder pain syndrome (IC/BPS) is a debilitating condition marked by chronic bladder pain and urinary symptoms. Patients may experience recurring pain, pressure, and/or discomfort perceived to be related to the urinary bladder as well as urinary frequency, urgency, and/or nocturia. The pain often worsens upon bladder filling and may be relieved upon bladder emptying. Many patients living with IC/BPS report that it takes a physical, emotional, and psychological toll, greatly impacting employment and social and intimate relationships. There are currently few FDA approved and/or effective treatment options for IC/BPS. Only about 1M of the 5.5M adults in the United States with symptoms of IC/BPS have been diagnosed or are receiving treatment.

About the LEADERSHIP 301 Trial

The LEADERSHIP 301 trial is a three-arm, multicenter, randomized, double-blind, placebo-controlled Phase 3 clinical trial assessing the effect of once-daily rosiptor (AQX-1125) 100 mg and 200 mg on bladder pain and urinary symptoms in female and male subjects with IC/BPS. The primary endpoint of the LEADERSHIP 301 trial is the change from baseline at week 12 in maximum daily bladder pain based on an 11-point numeric rating scale (NRS) compared to placebo in female subjects, recorded by electronic diary. Additional endpoints include urinary symptoms as well as an evaluation of overall improvement with therapy. The LEADERSHIP 301 trial has a 52-week extension period, affording all participating subjects the opportunity for treatment with rosiptor. Subjects have been enrolled at clinical research centers in the United States, Canada, and Europe. Topline data are anticipated in the third quarter of 2018.

About Rosiptor

Rosiptor (AQX-1125), Aquinox's lead drug candidate, is a first-in-class, once-daily, oral treatment being studied for its effects on inflammation and inflammatory pain. Rosiptor has a novel mechanism of action, activating SHIP1 (SH2-containing inositol-5'-phosphatase 1), an enzyme that serves to down-regulate inflammation through its role in the PI3K signaling pathway. Rosiptor has been generally well tolerated in multiple completed clinical studies, with more than 395 subjects dosed.

About Aquinox Pharmaceuticals, Inc.

Aquinox Pharmaceuticals, Inc. is a clinical-stage pharmaceutical company developing novel therapeutics for chronic urological conditions marked by inflammation and pain. Aquinox's lead drug candidate, rosiptor (AQX-1125), is in Phase 3 development for the treatment of patients with interstitial cystitis/bladder pain syndrome (IC/BPS), a condition for which there are currently few FDA approved and/or effective treatment options. Aquinox is focused on leveraging its library of novel compounds that activate SHIP1 to develop therapeutics for application in inflammation, inflammatory pain, and blood cancers. For more information, please visit www.aqxpharma.com.

Cautionary Note on Forward-Looking Statements

Certain of the statements made in this press release are forward looking, such as those, among others, relating to: development of rosiptor (AQX-1125), LEADERSHIP 301, availability of top-line data, initiation of additional clinical trials and expected sufficiency of cash-on-hand. These

statements are subject to risks and uncertainties that could cause actual results and events to differ materially from those anticipated, including, but not limited to, risks and uncertainties related to: our ability to enroll patients in our clinical trials at the pace that we project; as an organization, we have never conducted a pivotal clinical trial before; the size and growth of the potential markets for rosiptor (AQX-1125) or any future product candidates and our ability to serve those markets; our ability to obtain and maintain regulatory approval of rosiptor (AQX-1125) or any future product candidates; reaching agreement on design of pivotal trials with regulatory authorities and our expectations regarding the potential safety, efficacy or clinical utility of rosiptor or any future product candidates. Actual results or developments may differ materially from those projected or implied in these forward-looking statements. More information about the risks and uncertainties faced by Aquinox is contained in the company's Annual Report on Form 10-K for the year ended December 31, 2017 filed with the Securities and Exchange Commission. Aquinox disclaims any intention or obligation to update or revise any forward-looking statements, whether as a result of new information, future events or otherwise.

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AQUINOX PHARMACEUTICALS, INC.

Condensed consolidated balance sheets

(In thousands of U.S. dollars)

	<u>DECEMBER 31,</u> <u>2017</u>	<u>DECEMBER 31,</u> <u>2016</u>
Assets		
Cash, cash equivalents, short-term and long-term investments	\$ 108,085	\$ 153,105
Other current assets	740	426
Other long-term assets	1,504	849
Total assets	<u>\$ 110,329</u>	<u>\$ 154,380</u>
Liabilities		
Current liabilities	\$ 10,956	\$ 9,519
Non-current liabilities	486	197
Total liabilities	<u>\$ 11,442</u>	<u>\$ 9,716</u>
Stockholders' equity	<u>98,887</u>	<u>144,664</u>
Total liabilities and stockholders' equity	<u>\$ 110,329</u>	<u>\$ 154,380</u>



AQUINOX PHARMACEUTICALS, INC.

Condensed consolidated statements of operations

(In thousands of U.S. dollars, except per share and share amounts)

	YEARS ENDED DECEMBER 31,		
	2017	2016	2015
Operating expenses			
Research and development	\$ 36,267	\$ 28,382	\$ 15,799
General and administrative	14,852	9,263	5,541
Total operating expenses	<u>51,119</u>	<u>37,645</u>	<u>21,340</u>
Other income (expenses)			
Interest expense	(4)	(1)	-
Other income (expenses)	940	644	(520)
	<u>936</u>	<u>643</u>	<u>(520)</u>
Net loss	<u>\$ (50,183)</u>	<u>\$ (37,002)</u>	<u>\$ (21,860)</u>
Net loss per common stock - basic and diluted	\$ (2.14)	\$ (1.96)	\$ (1.73)
Basic and diluted weighted average number of common stock outstanding	23,450,315	18,893,515	12,637,839

