



March 9, 2017

## Aquinox Pharmaceuticals Announces Year End 2016 Financial Results

### Conference call update today at 4:30PM ET

VANCOUVER, British Columbia, March 09, 2017 (GLOBE NEWSWIRE) -- [Aquinox Pharmaceuticals, Inc.](#) ("[Aquinox](#)") (NASDAQ:AQXP), a clinical-stage pharmaceutical company discovering and developing targeted therapeutics in disease areas of inflammation and immuno-oncology, today provided a corporate update and reported financial results for the year ending December 31, 2016.

"We are actively enrolling patients in the LEADERSHIP 301 Phase 3 clinical trial with AQX-1125 in interstitial cystitis/bladder pain syndrome (IC/BPS) and are encouraged by initiated sites enrolling patients at the expected pace," said David Main, President & CEO of Aquinox. "However, the rate of initiating sites has been considerably slower than planned such that we now expect top-line data in 2018. With enrollment accelerating, sites continuing to be added and recruitment optimization plans underway for initiated sites, we anticipate having a clearer outlook on timing of top-line data at our mid-year financial call in August."

The LEADERSHIP 301 trial is enrolling both male and female patients, with a minimum of 300 female patients, at clinical research centers in Canada, the United States and Europe. The trial is being conducted as a three arm, multicenter, randomized, double-blind, placebo-controlled, Phase 3 clinical trial investigating the ability of 200 mg and 100 mg oral, once daily AQX-1125 to reduce bladder pain in patients with IC/BPS. The primary endpoint of the LEADERSHIP 301 trial will be to measure the difference in the change from baseline in the maximum daily bladder pain score based on an 11-point numeric rating scale (NRS) at twelve weeks recorded by electronic diary. The trial will also include a 40-week extension period affording all participating patients the opportunity for treatment with AQX-1125.

### Recent Business Highlights

**Completion of \$75.4 Million Financing.** On September 23, 2016, we announced the closing of a public offering of 6,152,500 shares of common stock at a price to the public of \$12.25 per share. Gross proceeds of the financing were \$75.4 million, inclusive of the fully exercised underwriters' option to purchase an additional 802,500 shares and before underwriting discounts and commissions and offering costs of approximately \$4.7 million. Aquinox intends to use the net proceeds from the offering to fund additional clinical development of AQX-1125 and to fund pre-commercial and market assessment activities, research and development costs to advance its pipeline of preclinical product candidates and for working capital and other general corporate purposes.

**Leadership 301 initiation and enrollment.** The Company now has 65 sites initiated and continues to plan for the initiation of a minimum of 100 total sites. As at March 8, 2017, a total of 64 patients had been enrolled, with the majority enrolled in the past two months and patient screening on the rise. Given the trial is now entering an acceleration phase, several more months of execution will be valuable for the Company to provide an enrollment update with additional guidance on expected timing of top-line data at its mid-year financial conference call in early August.

**Appointment of Barbara Troupin.** On October 3, 2016, we announced the appointment of Dr. Barbara Troupin, M.D. as Chief Medical Officer and Vice President, Clinical Development. Dr. Troupin brings to Aquinox experience in leading the development and execution of clinical strategy for drug development programs ranging from proof-of-concept to NDA filing as well as in building relationships with key opinion leaders (KOLs). Dr. Troupin is leading Aquinox's overall clinical and medical affairs strategies for ongoing programs as well as future development and potential commercialization plans.

**Presentation of Data at ASH.** On December 3, 2016, Prof. Graham Packham BSc (Hons), PhD, Professor of Molecular Oncology within Medicine at the University of Southampton presented at the 58th American Society of Hematology (ASH) Annual Meeting an abstract describing the in vitro response of malignant human B cells to a novel chemical activator of SHIP1. Dr. Packham's work supports the hypothesis that chemical SHIP1 activation is sufficient to suppress BCR-mediated signaling in chronic lymphocytic leukemia cells, acting, at least in part, by decreasing activation of the AKT pathway. Titled "Chemical Activation of the SHIP1 Inositol Lipid Phosphatase: A Novel Therapeutic Strategy to Suppress B-cell Receptor Signaling and CXCR4 Expression in Malignant Human B Cells", the abstract can be found on the ASH website at

## Summary of Financial Results

**Cash Position.** Cash, cash equivalents, short-term and long-term investments totaled \$153.1 million as of December 31, 2016, compared to \$112.9 million as of December 31, 2015. The increase was primarily the result of the public offering of common stock in September 2016 partly offset by the ongoing expenditures related to our LEADERSHIP 301 clinical trial in IC/BPS. Aquinox expects its cash, cash equivalents, short-term and long-term investments to be sufficient for additional clinical development, manufacturing, preclinical, and pre-commercial and market assessment activities. We continue to expect that our cash-on-hand will carry us beyond top-line data from the 301 trial and into 2019.

**R&D Expenses.** Research and development expenses for the year ended December 31, 2016 increased to \$28.4 million from \$15.8 million for 2015. This increase was primarily driven by increased clinical activities related to the initiation of our LEADERSHIP 301 clinical trial.

**G&A Expenses.** General and administrative expenses for the year ended December 31, 2016 increased to \$9.3 million from \$5.5 million for 2015. This increase was primarily driven by higher personnel related costs and the establishment of an office in San Bruno, California.

**Net Loss.** Net loss for the year ended December 31, 2016 was \$37.0 million compared to a net loss of \$21.9 million for 2015. This increase was primarily driven by increased operating expenditures related to the initiation of our LEADERSHIP 301 clinical trial.

Aquinox will host a conference call and live audio webcast on Thursday, March 9, 2017 at 4:30 PM (ET)/ 1:30 PM (PT).

### Conference Call and Webcast Details:

Date: Thursday, March 9, 2017

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

Toll-free: (866) 357-7878

International: (315) 625-3088

Audience Passcode 72144360

Conference ID: 72144360

Webcast URL: <http://edge.media-server.com/m/p/askid88w>

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](http://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 (IC/BPS)

IC/BPS is a chronic inflammatory bladder disease characterized by pelvic pain and increased urinary urgency and/or frequency. For many sufferers, these symptoms are severe and adversely affect all major aspects of their lives, including overall physical and emotional health, employment, social and intimate relationships, and leisure activities. While the cause of the disease remains largely unknown, erosion of the bladder lining is thought to be a significant contributor. IC/BPS is estimated to affect millions of people in the United States and around the world. Most IC/BPS patients continue to suffer this debilitating condition, despite treatment with existing therapies. Most current therapies and those in development are focused solely on symptomatic relief of IC/BPS. Aquinox believes new and innovative therapies that target the underlying disease to reduce the chronic pain and urinary symptoms are needed.

## About [AQX-1125](#)

AQX-1125, Aquinox's lead drug candidate, is a small molecule activator of SHIP1, which is a regulating component of the PI3K cellular signaling pathway. By increasing SHIP1 activity, AQX-1125 accelerates a natural mechanism that has evolved to maintain homeostasis of the immune system and reduce immune cell activation and migration to sites of inflammation. AQX-1125 has demonstrated preliminary safety and favorable drug properties for once daily oral administration in multiple preclinical studies and seven completed clinical trials.

## About [Aquinox Pharmaceuticals, Inc.](#)

Aquinox Pharmaceuticals, Inc. is a late clinical-stage pharmaceutical company discovering and developing targeted therapeutics in disease areas of inflammation and immuno-oncology. Our primary focus is anti-inflammatory product candidates targeting SH2-containing inositol-5'-phosphatase 1, or SHIP1, which is a key regulator of an important cellular

signaling pathway in immune cells, known as the PI3K pathway. Aquinox's lead drug candidate, AQX-1125, is a small molecule activator of SHIP1 suitable for oral, once daily dosing. In September 2016, we began enrolling patients in a Phase 3 clinical trial of AQX-1125 in our lead indication, Interstitial Cystitis/Bladder Pain Syndrome (IC/BPS). Other indications are under consideration for future investigation. Aquinox has a broad intellectual property portfolio and pipeline of preclinical drug candidates that activate SHIP1. For more information, please visit [www.aqxpharma.com](http://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: LEADERSHIP 301, availability of top-line data and our projected cash position. 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 AQX-1125 or any future product candidates and our ability to serve those markets; our ability to obtain and maintain regulatory approval of 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 AQX-1125 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, 2016 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)

	DECEMBER 31, 2016	DECEMBER 31, 2015
<b>Assets</b>		
Cash, cash equivalents, short-term and long-term investments	\$ 153,105	\$ 112,940
Other current assets	426	314
Other long-term assets	849	89
Total assets	<u>\$ 154,380</u>	<u>\$ 113,343</u>
<b>Liabilities</b>		
Current liabilities	\$ 9,519	\$ 4,792
Non-current liabilities	197	131
Total liabilities	<u>\$ 9,716</u>	<u>\$ 4,923</u>
Stockholders' equity	144,664	108,420
Total liabilities and stockholders' equity	<u>\$ 154,380</u>	<u>\$ 113,343</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,		
	2016	2015	2014
<b>Operating expenses</b>			
Research and development	\$ 28,382	\$ 15,799	\$ 18,079
General and administrative	9,263	5,541	4,296
Total operating expenses	<u>37,645</u>	<u>21,340</u>	<u>22,375</u>
<b>Other income (expenses)</b>			
Amortization and extinguishment of remaining discount on preferred shares upon conversion of preferred shares	-	-	(1,884)
Interest expense	(1)	-	(451)
Other income (expenses)	644	(520)	683
	<u>643</u>	<u>(520)</u>	<u>(1,652)</u>
<b>Net loss</b>	<u>\$ (37,002)</u>	<u>\$ (21,860)</u>	<u>\$ (24,027)</u>
Net loss per common stock - basic and diluted	\$ (1.96)	\$ (1.73)	\$ (2.75)
Basic and diluted weighted average number of common stock outstanding	18,893,515	12,637,839	8,667,387

 [Primary Logo](#)

Source: Aquinox Pharmaceuticals, Inc.

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