



May 9, 2017

Aquinox Pharmaceuticals Announces First Quarter 2017 Financial Results

VANCOUVER, British Columbia, May 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 first quarter ending March 31, 2017.

"Highlights of the quarter included continued progress on our LEADERSHIP 301 trial and the addition of two new Directors to our Board," said David Main, President & CEO of Aquinox. "We plan to provide guidance on top line data availability from 301 on our mid-year call in early August as well as provide an update on other pipeline activities."

Recent Business Highlights

Addition of Dr. Kelvin Neu and Dr. Richard Levy to the Board of Directors. On March 23, 2017, Aquinox welcomed the addition of Baker Brothers Investments' representatives Dr. Kelvin Neu and Dr. Richard Levy to the Company's Board of Directors. Baker Brothers Investments, Aquinox's largest shareholder, manages long-term investment funds, focused on publicly traded life sciences companies, for major university endowments and foundations.

Leadership 301 initiation and enrollment. On March 9th the Company provided an update on the progress of the LEADERSHIP 301 trial. Aquinox plans 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. Top line data from the LEADERSHIP 301 trial is anticipated in 2018.

Summary of Financial Results

Cash Position. Cash, cash equivalents, short-term and long-term investments totaled \$141.6 million as of March 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, 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 first quarter of 2017 increased to \$5.8 million from \$4.9 million in the first quarter of 2016. This increase was primarily driven by increased clinical activities as we continued our LEADERSHIP 301 clinical trial of AQX-1125 in IC/BPS.

G&A Expenses. General and administrative expenses for the first quarter of 2017 increased to \$2.7 million from \$2.0 million in the first quarter of 2016. This increase was primarily driven by higher personnel related costs and pre-commercial and market assessment activities.

Net Loss. Net loss for the first quarter of 2017 was \$8.3 million compared to a net loss of \$6.7 million in the first quarter of 2016. This increase was primarily driven by increased operating expenditures as we continued our LEADERSHIP 301 clinical trial of AQX-1125 in IC/BPS.

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 the LEADERSHIP 301 Trial

The LEADERSHIP 301 trial, which commenced enrollment in September of 2016, is 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 is 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. Secondary endpoints include urinary symptoms, including frequency and nighttime awakenings to void, as well as measures of quality of life. The LEADERSHIP 301 trial also has an additional 52-week extension period, affording all participating patients the opportunity for treatment with AQX-1125. An anticipated minimum of 300 female patients, up to a maximum of 600 patients including males, are currently being enrolled at clinical research centers in the United States, Canada and Europe. Top line data from the LEADERSHIP 301 trial is anticipated in 2018.

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.

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 AQX-1125, LEADERSHIP 301 and availability of top-line data. 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 Quarterly Report on Form 10-Q for the quarter ended March 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

(Unaudited)
(In thousands of U.S. dollars)

	<u>MARCH 31,</u> <u>2017</u>	<u>DECEMBER 31,</u> <u>2016</u>
Assets		
Cash, cash equivalents, short-term and long-term investments	\$ 141,589	\$ 153,105
Other current assets	855	426
Other long-term assets	885	849
Total assets	<u>\$ 143,329</u>	<u>\$ 154,380</u>
Liabilities		
Current liabilities	\$ 5,824	\$ 9,519
Non-current liabilities	562	197
Total liabilities	<u>\$ 6,386</u>	<u>\$ 9,716</u>
Stockholders' equity	136,943	144,664
Total liabilities and stockholders' equity	<u>\$ 143,329</u>	<u>\$ 154,380</u>

AQUINOX PHARMACEUTICALS, INC.

Condensed consolidated statements of operations

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

	<u>THREE MONTHS ENDED</u> <u>MARCH 31,</u>	
	<u>2017</u>	<u>2016</u>
Operating expenses		
Research and development	\$ 5,777	\$ 4,881
General and administrative	2,745	1,953
Total operating expenses	8,522	6,834
Other income	206	123
Net loss before income taxes	<u>(8,316)</u>	<u>(6,711)</u>
Net loss	<u>\$ (8,316)</u>	<u>\$ (6,711)</u>
Net loss per common stock - basic and diluted	\$ (0.36)	\$ (0.39)
Basic and diluted weighted average number of common stock outstanding	23,423,150	17,211,986

 [Primary Logo](#)

Source: Aquinox Pharmaceuticals, Inc.

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