



August 8, 2017

Aquinox Pharmaceuticals Announces Second Quarter 2017 Financial Results

- Enrollment in LEADERSHIP 301 with Rosiptor in IC/BPS Passes 50% -

- Selection of CP/CPPS as Second Clinical Indication -

VANCOUVER, British Columbia, Aug. 08, 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 second quarter ending June 30, 2017.

"With more than one hundred trial sites initiated, enrollment in LEADERSHIP 301 is now on pace with expectations and we anticipate reporting top-line data in the third quarter of 2018," said David Main, President & CEO of Aquinox. "After a thorough examination of numerous potential disease states, we also are pleased to announce the expansion of our rosiptor (AQX-1125) clinical development program with the planned initiation of a Phase 2 clinical trial in Chronic Prostatitis/Chronic Pelvic Pain Syndrome (CP/CPPS) and expect patient enrollment to commence in the first quarter of 2018."

Recent Business Highlights

Progress in Leadership 301. As of August 7, 2017, 195 patients have been enrolled in Aquinox's LEADERSHIP 301 Phase 3 clinical trial with rosiptor (AQX-1125) in Interstitial Cystitis/Bladder Pain Syndrome (IC/BPS) across 108 trial sites. With 154 women enrolled to date, we have surpassed 50% of our female enrollment target. Based on the number of active sites and current enrollment rates, Aquinox anticipates reporting top-line data from the LEADERSHIP 301 trial in the third quarter of 2018. Aquinox will announce once enrollment is complete in the LEADERSHIP 301 trial.

Initiation of Phase 2 Trial with Rosiptor in CP/CPPS. Aquinox is today announcing its plans to initiate a proof-of-concept Phase 2 trial with rosiptor (AQX-1125) in patients with CP/CPPS. 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.

USAN Assigns Generic Name "Rosiptor" to AQX-1125. On April 26, 2017, the United States Adopted Name Council (USAN) adopted the generic name "rosiptor" (pronounced "roe sip' tor") for AQX-1125. This name was published by USAN on July 7, 2017. Aquinox will adopt the reference of rosiptor for appropriate use going forward while phasing out reference to AQX-1125.

Summary of Financial Results

Cash Position. Cash, cash equivalents, short-term and long-term investments totaled \$131.3 million as of June 30, 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. Aquinox continues to expect that its cash-on-hand will carry it beyond top-line data from the LEADERSHIP 301 trial and into 2019.

R&D Expenses. Research and development expenses for the second quarter of 2017 increased to \$10.5 million from \$9.2 million in the second quarter of 2016. This increase was primarily driven by increased clinical activities as Aquinox continued its LEADERSHIP 301 clinical trial with rosiptor (AQX-1125) in IC/BPS.

G&A Expenses. General and administrative expenses for the second quarter of 2017 increased to \$3.5 million from \$1.8 million in the second 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 second quarter of 2017 was \$13.8 million compared to a net loss of \$10.9 million in the second

quarter of 2016. This increase was primarily driven by increased operating expenditures as Aquinox continued its LEADERSHIP 301 clinical trial of rosiptor (AQX-1125) in IC/BPS.

Aquinox will host a conference call and live audio webcast on **Tuesday, August 8, 2017 at 8:30AM (ET)/ 5:30AM (PT)**.

Conference Call and Webcast Details:

Date: Tuesday, August 8, 2017

Time: 8:30 AM (ET) / 5:30 AM (PT)

Toll-free: (866) 357-7878

International: (315) 625-3088

Audience Passcode 5911876

Conference ID: 59711876

Webcast URL: <http://edge.media-server.com/m/p/mqa4bkna>

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 [Rosiptor \(AQX-1125\)](#)

Rosiptor, 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, rosiptor 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. Rosiptor has demonstrated preliminary safety and favorable drug properties for once daily oral administration in multiple preclinical studies and eight completed clinical trials.

About [Interstitial Cystitis/Bladder Pain Syndrome \(IC/BPS\)](#)

IC/BPS is a chronic inflammatory bladder disease characterized by pelvic pain and increased urinary frequency and/or urgency. 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 the [LEADERSHIP 301 Trial](#)

The LEADERSHIP 301 trial, which commenced enrollment in September 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 rosiptor (AQX-1125) to reduce bladder pain in patients with IC/BPS. The primary endpoint of the LEADERSHIP 301 trial is 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. Additional 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 rosiptor. 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 third quarter of 2018.

About [Chronic Prostatitis/Chronic Pelvic Pain Syndrome \(CP/CPPS\)](#)

Chronic prostatitis/chronic pelvic pain syndrome (CP/CPPS) is a chronic pain disorder characterized by the presence of noninfectious pelvic pain, unrelated to urinary bladder filling or emptying, lasting longer than 3 months. CP/CPPS is a complex condition of uncertain etiology affecting men of all ages, which can significantly impair the quality of life and the social functioning of patients. In the US, the number of patients diagnosed with and receiving treatment for CP/CPPS is similar to that of IC/BPS - at approximately 1 million. There are no FDA approved products for the treatment of CP/CPPS and effective management of this condition is challenging and often inadequate, supporting the need for new and effective therapies to treat CP/CPPS.

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, rosiptor (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 rosiptor 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 rosiptor (AQX-1125), LEADERSHIP 301, availability of top-line data, initiation of 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 Quarterly Report on Form 10-Q for the quarter ended June 30, 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>JUNE 30,</u> <u>2017</u>	<u>DECEMBER 31,</u> <u>2016</u>
Assets		
Cash, cash equivalents, short-term and long-term investments	\$ 131,277	\$ 153,105
Other current assets	915	426
Other long-term assets	1,092	849
Total assets	<u>\$ 133,284</u>	<u>\$ 154,380</u>
Liabilities		
Current liabilities	\$ 8,128	\$ 9,519
Non-current liabilities	548	197
Total liabilities	<u>8,676</u>	<u>9,716</u>
Stockholders' equity	124,608	144,664
Total liabilities and stockholders' equity	<u>\$ 133,284</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)

	THREE MONTHS ENDED JUNE 30,		SIX MONTHS ENDED JUNE 30,	
	2017	2016	2017	2016
Operating expenses				
Research and development	\$ 10,475	\$ 9,235	\$ 16,252	\$ 14,116
General and administrative	3,520	1,830	6,265	3,783
Total operating expenses	13,995	11,065	22,517	17,899
Other income, net	229	164	435	287
Net loss	<u>\$ (13,766)</u>	<u>\$ (10,901)</u>	<u>\$ (22,082)</u>	<u>\$ (17,612)</u>
Net loss per common stock - basic and diluted	\$ (0.59)	\$ (0.63)	\$ (0.94)	\$ (1.02)
Basic and diluted weighted average number of common stock outstanding	23,444,150	17,212,007	23,433,708	17,211,997

 [Primary Logo](#)

Source: Aquinox Pharmaceuticals, Inc.

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