



November 8, 2017

Aquinox Pharmaceuticals Announces Third Quarter 2017 Financial Results

VANCOUVER, British Columbia, Nov. 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 reported financial results for the third quarter ending September 30, 2017 and provided a corporate update.

"Our LEADERSHIP 301 clinical trial in Interstitial Cystitis/Bladder Pain Syndrome (IC/BPS) continues to enroll on pace to meet our guidance of top-line data in the third quarter of 2018," said David Main, President & CEO of Aquinox. "In addition, November marks Bladder Health Month in the U.S. and we are pleased to be supporting greater awareness of IC/BPS with our continued support for the Interstitial Cystitis Association (ICA) and their patient advocacy efforts. We are committed to increasing awareness of this debilitating disease and the ongoing pursuit of effective treatments."

November was established as Bladder Health Month in 2016 as one of the US Department of Health and Human Services (HHS) National Health Observances. Bladder Health Month aims to raise awareness about conditions that affect the bladder and other parts of the urinary tract, in both men and women, and includes a week dedicated to interstitial cystitis & neurogenic bladder, to take place November 1-4. Aquinox is sponsoring a disease awareness initiative with the ICA to draw attention to the needs of patients with IC/BPS and provide tools to enhance the dialogue between health care providers and patients. The video and associated resources for patients and health care providers is available at: <http://icbpstips.com>

Business Highlights

FDA Advisory Committee Meeting on IC/BPS. On August 28th Aquinox welcomed the announcement by the FDA Division of Bone, Reproductive and Urology Products that it will hold an Advisory Committee meeting to discuss IC/BPS disease definition and appropriate trial endpoints. Details of the Advisory Committee meeting, which is scheduled for December 7th, 2017, can be found at: <https://www.federalregister.gov/documents/2017/08/28/2017-18131/bone-reproductive-and-urologic-drugs-advisory-committee-notice-of-meeting-establishment-of-a-public>

Summary of Financial Results

Cash Position. Cash, cash equivalents, short-term and long-term investments totaled \$119.7 million as of September 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, 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 top-line data from the LEADERSHIP 301 trial and at least to mid-2019.

R&D Expenses. Research and development expenses for the third quarter of 2017 increased to \$8.5 million from \$6.1 million in the third 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 third quarter of 2017 increased to \$3.6 million from \$2.1 million in the third 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 third quarter of 2017 was \$11.8 million compared to a net loss of \$8.1 million in the third 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.

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 [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 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 September 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)

	SEPTEMBER 30, 2017	DECEMBER 31, 2016
Assets		
Cash, cash equivalents, short-term and long-term investments	\$ 119,695	\$ 153,105
Other current assets	878	426
Other long-term assets	1,029	849
Total assets	<u>\$ 121,602</u>	<u>\$ 154,380</u>
Liabilities		
Current liabilities	\$ 6,997	\$ 9,519
Non-current liabilities	513	197
Total liabilities	<u>7,510</u>	<u>9,716</u>
Stockholders' equity	<u>114,092</u>	<u>144,664</u>
Total liabilities and stockholders' equity	<u>\$ 121,602</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 SEPTEMBER 30,		NINE MONTHS ENDED SEPTEMBER 30,	
	2017	2016	2017	2016
Operating expenses				
Research and development	\$ 8,456	\$ 6,134	\$ 24,708	\$ 20,250
General and administrative	3,614	2,149	9,879	5,932
Total operating expenses	<u>12,070</u>	<u>8,283</u>	<u>34,587</u>	<u>26,182</u>
Other income, net	237	137	672	424
Net loss	<u>\$ (11,833)</u>	<u>\$ (8,146)</u>	<u>\$ (33,915)</u>	<u>\$ (25,758)</u>
Net loss per common stock - basic and diluted	\$ (0.50)	\$ (0.46)	\$ (1.45)	\$ (1.48)
Basic and diluted weighted average number of common stock outstanding	23,464,785	17,690,362	23,444,181	17,372,616

