



For Immediate Release

## **Aquinox Pharmaceuticals Initiates Patient Dosing in LEADERSHIP 301 Phase 3 Clinical Trial of AQX-1125 in Interstitial Cystitis/Bladder Pain Syndrome**

**Vancouver, British Columbia – September 6, 2016** - (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, announced today that it has initiated dosing in a Phase 3 clinical trial of AQX-1125 for the treatment of interstitial cystitis/bladder pain syndrome (IC/BPS).

The LEADERSHIP 301 trial will enroll a minimum of 300 female patients and up to 300 male subjects at clinical research centers in Canada, the United States and Europe and will be 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 moderate to severe IC/BPS.

“Building upon the positive results from the LEADERSHIP Phase 2 trial where AQX-1125 demonstrated an ability to improve both pain and urinary symptoms in patients with IC/BPS, we are excited to begin this next phase of development towards potential commercialization.” said David Main, President & CEO of Aquinox. “We are eager to continue to explore the potential of AQX-1125 to meet this unmet medical need, as it is a severely debilitating disease where no new oral therapy has been approved in the last twenty years.”

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 an open-label extension of up to 40 weeks affording all participating patients the opportunity for treatment with AQX-1125. Secondary endpoints will include urinary symptoms, including frequency and nighttime awakenings, as well as measures of quality of life. Top-line data from the LEADERSHIP 301 trial is anticipated in the fourth quarter of 2017. For more information on the LEADERSHIP 301 trial, please visit [www.clinicaltrials.gov](http://www.clinicaltrials.gov).

### **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 in order to reduce the chronic pain and urinary symptoms are needed.

### **About the LEADERSHIP 201 Trial**

The LEADERSHIP 201 trial was a multicenter, randomized, double-blind, placebo-controlled, Phase 2 clinical trial investigating the ability of 200 mg oral, once daily AQX-1125 to reduce pain in female patients with IC/BPS. The primary endpoint was to measure the difference in the change from baseline in the average daily bladder pain score based on an 11-point numeric rating scale (NRS) at six weeks recorded by electronic diary. Results demonstrated a positive trend in the primary endpoint and statistically significant changes on secondary endpoints including both pain and urinary symptoms. A total of 69 subjects were enrolled. For more information on the LEADERSHIP 201 trial, please visit [www.clinicaltrials.gov](http://www.clinicaltrials.gov).

## 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 signalling 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 clinical-stage pharmaceutical company discovering and developing targeted therapeutics in disease areas of inflammation and immuno-oncology. Aquinox's lead drug candidate, AQX-1125, is a small molecule activator of SHIP1 suitable for oral, once daily dosing. With a successful Phase 2 clinical trial completed in 2015, Aquinox initiated a Phase 3 trial in 2016 (LEADERSHIP 301) with AQX-1125 for treatment of IC/BPS. 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: enrollment in and results from our LEADERSHIP 301 trial in IC/BPS; and potential commercialization of, and market opportunities for, AQX-1125. 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 June 30, 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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