



Liquidia's LIQ861 Meets Primary Endpoint in Pivotal Phase 3 INSPIRE Study in Patients with Pulmonary Arterial Hypertension

March 11, 2019

- *LIQ861 was well-tolerated in PAH patients at two months of treatment*
 - *INSPIRE enrollment complete, including PK sub-study*
 - *Anticipate submitting NDA for LIQ861 to the FDA in late 2019*

RESEARCH TRIANGLE PARK, N.C., March 11, 2019 (GLOBE NEWSWIRE) -- Liquidia Technologies, Inc. (Nasdaq: LQDA) ("Liquidia"), a late-stage clinical biopharmaceutical company focused on the development and commercialization of human therapeutics using its proprietary PRINT® technology, today announced top-line results of its pivotal Phase 3 INSPIRE study in patients with pulmonary arterial hypertension ("PAH") treated with LIQ861, the first inhaled dry powder formulation of treprostinil. Initial analysis indicates the study has met its primary endpoint of safety and tolerability of LIQ861 at the two-month timepoint.

Nicholas Hill, MD, Chief Pulmonary, Critical Care & Sleep Division and Professor of Medicine at Tufts University School of Medicine and INSPIRE Principal Investigator, stated: "The top-line analysis of LIQ861 from the INSPIRE study is highly encouraging for physicians and patients. LIQ861 was safely titrated to therapeutic levels across a wide range of inhaled doses and was very well tolerated. This means that we are moving closer to having an inhaled therapy available for PAH that is much more convenient than previous ones. Most patients tolerated relatively high doses of treprostinil, raising the possibility that the PRINT technology, by virtue of its ability to make microscopic particles of uniform size, could improve distribution of drug to the lung, enhancing therapeutic effect."

LIQ861 was observed to be well-tolerated in 109 patients, with 101 patients (93%) completing at least two months of treatment. During the two-month period, LIQ861 was evaluated at doses up to 150 mcg capsule strength with no study-drug related serious adverse events observed. Reported treatment-emergent adverse events ("TEAEs") were mostly mild to moderate in nature. The most common TEAEs reported with LIQ861 in ≥4% of PAH patients were cough (33%), headache (18%), throat irritation (14%), dizziness (10%), diarrhea (8%), oropharyngeal pain (6%), nausea (6%) dyspnea (6%), flushing (6%) and chest discomfort (5%). These observations are consistent with the safety data at the two-week timepoint reported on January 7, 2019. Of the TEAEs observed, most were reported during the first two weeks of initial exposure and occurred in patients previously naïve to prostacyclin-based therapy in which LIQ861 was added to oral therapy.

Neal Fowler, Chief Executive Officer of Liquidia, commented: "We are extremely grateful to the patients participating in the clinical trial and for the effort and speed with which our investigators completed enrollment. We believe the commitment to this study signals an increasing need for safe, more convenient inhaled treatment options. We are preparing the new drug application submission, while collecting additional longitudinal data on the benefits from LIQ861."

In addition to meeting the primary endpoint, the one-directional crossover sub-study to compare bioavailability and pharmacokinetics of treprostinil as the patients transition from Tyvaso to LIQ861 has been fully enrolled. Liquidia expects to report its pharmacokinetics results in the second quarter of 2019 and plans to provide more detailed clinical results through scientific disclosures at upcoming congresses and in peer-reviewed publications.

About LIQ861

LIQ861 is an inhaled dry powder formulation of treprostinil designed using Liquidia's PRINT technology to enhance deep-lung delivery using a convenient, palm-sized, disposable dry powder inhaler ("DPI") for the treatment of PAH. Liquidia believes LIQ861 can overcome the limitations of current inhaled therapies and has the potential to maximize the therapeutic benefits of treprostinil in treating PAH by safely delivering higher doses into the lungs.

About INSPIRE Clinical Trial

Liquidia's pivotal open-label Phase 3 clinical trial, known as INSPIRE, or Investigation of the Safety and Pharmacology of Dry Powder Inhalation of Treprostinil, is designed to evaluate patients who have either been under stable treatment with nebulizer-delivered treprostinil for at least three months and are transitioned to LIQ861 under the protocol or patients who have been on stable treatment with no more than two non-prostacyclin oral PAH therapies for at least three months and have their treatment regimen supplemented with LIQ861 under the protocol. The primary objective of the INSPIRE study is to evaluate the long-term safety and tolerability of LIQ861. For more information, please visit <https://clinicaltrials.gov/ct2/show/NCT03399604>.

About Liquidia Technologies

Liquidia Technologies is a late-stage clinical biopharmaceutical company focused on the development and commercialization of human therapeutics using its proprietary PRINT® technology to transform the lives of patients. Currently, Liquidia is focused on the development of two product candidates using its PRINT® particle engineering platform: LIQ861 for the treatment of PAH and LIQ865 for the treatment of local post-operative pain. Being evaluated in a Phase 3 clinical trial (INSPIRE), LIQ861 is designed to improve the therapeutic profile of treprostinil by enhancing deep-lung delivery and achieving higher dose levels than current inhaled therapies by using a convenient, palm-sized, disposable DPI. LIQ865, for which Liquidia has completed two Phase 1 clinical trials, is designed to deliver sustained-release particles of bupivacaine, a non-opioid anesthetic, to treat local post-operative pain for three to five days through a single administration. For more information visit our website at www.liquidia.com.

Forward-Looking Statements

This press release may include forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. All statements contained in this press release other than statements of historical facts, including statements regarding our future results of operations and financial

position, our business strategy and plans and our objectives for future operations, are forward-looking statements. Such forward-looking statements, including statements regarding clinical trials, clinical studies and other clinical work (including the funding therefor, anticipated patient enrollment, safety data, study data, trial outcomes, timing or associated costs), regulatory applications and related timelines, including the filing of an NDA for LIQ861, involve significant risks and uncertainties and actual results could differ materially from those expressed or implied herein. The words “anticipate,” “believe,” “continue,” “estimate,” “expect,” “intend,” “may,” “will” and similar expressions are intended to identify forward-looking statements. We have based these forward-looking statements largely on our current expectations and projections about future events and financial trends that we believe may affect our financial condition, results of operations, business strategy, short-term and long-term business operations and objectives and financial needs. These forward-looking statements are subject to a number of risks discussed in our filings with the Securities and Exchange Commission, as well as a number of uncertainties and assumptions. Moreover, we operate in a very competitive and rapidly changing environment and our industry has inherent risks. New risks emerge from time to time. It is not possible for our management to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements we may make. In light of these risks, uncertainties and assumptions, the future events discussed in this press release may not occur and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements. Nothing in this press release should be regarded as a representation by any person that these goals will be achieved, and we undertake no duty to update our goals or to update or alter any forward-looking statements, whether as a result of new information, future events or otherwise.

Contact Information**Investors:**

Jenny Kobin
IR Advisory Solutions
919.328.4389
IR@liquidia.com

Media:

Christy Curran
Sam Brown Inc.
615.414.8668
media@liquidia.com



Source: Liquidia Technologies, Inc.