Liquidia Technologies Reports Positive Interim LIQ861 Safety Data on 109 Patients from Pivotal INSPIRE Trial

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- *LIQ861 was well-tolerated in PAH patients at two-weeks of treatment, the safety endpoint requested by U.S. FDA*
- *NDA submission targeted for late 2019*

RESEARCH TRIANGLE PARK, N.C., Jan. 07, 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 to transform the lives of patients, today reported positive interim safety data from its open-label, multicenter Phase 3 clinical trial (INSPIRE) evaluating LIQ861, an inhaled dry powder formulation of treprostinil, for the treatment of pulmonary arterial hypertension ("PAH"). The safety data at the two-week timepoint addresses the U.S. Food and Drug Administration's ("FDA") data request for inclusion in a New Drug Application ("NDA") submission. Liquidia anticipates submitting the full NDA for LIQ861 to the FDA in late 2019.

LIQ861 was observed to be well-tolerated at the two-week timepoint in PAH patients. During this time period, LIQ861 was evaluated at doses up to approximately 125 mcg with no study-drug related serious adverse events or dose-limiting toxicities. Reported treatment-emergent adverse events ("TEAEs") were mostly mild in nature and consistent with inhaled prostacyclin therapy. The most common TEAEs reported with LIQ861 in ≥4% of PAH patients (n=109) were cough (25%), headache (13%), throat irritation (12%), diarrhea (7%), dizziness (6%), oropharyngeal pain (5%) and chest discomfort (5%). Patients have continued to receive treatment beyond two-weeks with the first patient dosed in March 2018. To date, a maximum tolerated dose of LIQ861 has not yet been reached, with patients having been administered doses up to approximately 150 mcg.

"LIQ861 has the potential to maximize the therapeutic benefit of inhaled treprostinil in treating PAH by safely delivering higher doses into the lungs," stated Nicholas Hill, MD, Chief Pulmonary, Critical Care & Sleep Division and Professor of Medicine at Tufts University School of Medicine and INSPIRE Principal Investigator. "Enabled by Liquidia's proprietary PRINT technology, LIQ861 is designed to provide the benefits of delivering prostacyclin analogs locally to the lungs via inhalation, potentially offering a targeted and effective approach with an acceptable systemic side effect profile."

The INSPIRE clinical trial 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. Patients adding LIQ861 to current non-prostacyclin oral therapies started at a dose of approximately 25 mcg and those transitioned from nebulizer-delivered treprostinil at a stable dose were initiated at a dose of LIQ861 lower than their current stable treprostinil dose. In both cases, LIQ861 was uptitrated in 25 mcg incremental doses to symptom relief or the limit of tolerance.

"Patient demographics and baseline characteristics in the trial suggest that LIQ861 may be attractive across disease severity and may have utility as a first-line prostacyclin," added Robert Roscigno, PhD, Liquidia's Senior Vice President of Product Development and LIQ861 Program Lead. "Interestingly, enrollment of the safety portion of the trial was driven primarily by stronger than anticipated interest from New York Heart Association Functional Class II add-on patients, which may imply that dry-powder delivery could be an alternative to oral delivery in prostacyclin naïve patients. We are pleased with these findings and believe they support the therapeutic potential and versatility of LIQ861 among patients across different functional classes."

Liquidia continues to enroll patients in the INSPIRE clinical trial in support of the one-directional crossover pharmacokinetic ("PK") sub-study. The sub-study is designed to compare bioavailability and PK of treprostinil as patients are transitioned from nebulizer-delivered treprostinil to LIQ861. PK results are expected to be reported in the second quarter of 2019. To further support Liquidia's future marketing and commercial activities with additional medical information, Liquidia expects to continue to treat patients and collect data until the launch of LIQ861 in the United States, if approved.

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 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 pulmonary arterial hypertension 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.

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