Liquidia Announces Clinical Update on INSPIRE; Targeting NDA Filing for LIQ861 in Late 2019

June 5, 2019

RESEARCH TRIANGLE PARK, N.C., June 05, 2019 (GLOBE NEWSWIRE) -- Liquidia Technologies, Inc. (Nasdaq: LQDA) ("Liquidia"), a late-stage clinical biopharmaceutical company, today provided an update on the Phase 3 INSPIRE trial and development of LIQ861 for the treatment of pulmonary arterial hypertension (PAH).

As previously announced, the initial analysis of the INSPIRE study indicated the study has met its primary endpoint of safety and tolerability of LIQ861 at the two-month timepoint. Results from the INSPIRE study indicates that the 75 mcg capsule strength of LIQ861 (single capsule, 1-2 breaths) correlates with the 54 mcg dose of Tyvaso® (9 breaths), the maximum recommended label dose of Tyvaso®. INSPIRE also included a one-directional, crossover sub-study to assess the comparable bioavailability and pharmacokinetics (PK) of single doses of LIQ861 and Tyvaso in 18 PAH patients.

The company targets NDA submission in late 2019 that will include additional data generated from ongoing development activities. Analysis of the data from the PK sub-study in patients showed variability in systemic plasma levels of both LIQ861 and Tyvaso, which is believed to be attributed to variation in severity of disease and has been seen in prior studies of treprostinil in patients. To more accurately characterize the PK of LIQ861, Liquidia conducted an additional PK study in healthy volunteers. Post-hoc analysis showed that plasma levels of treprostinil were tightly correlated to the LIQ861 dose delivered. The company is continuing work to supplement the PK data set of LIQ861 and to further assess and minimize the variability in dosing levels, which Liquidia believes may be due to the administration technique by some healthy volunteers in the additional study. The company targets a pre-NDA meeting early in the fourth quarter followed by NDA submission in late 2019.

Lewis J. Rubin, MD, FACP, FCCP, FAHA, FRCP, Professor of Medicine, Emeritus and former Director of the Division of Pulmonary and Critical Care Medicine at the University of California, San Diego School of Medicine, and Adjunct Professor of Medicine at Columbia University College of Physicians and Surgeons, and a senior advisor to the LIQ861 program, stated: "I am very encouraged by the LIQ861 clinical results and believe it could be an important treatment option for PAH patients. Clinical observations, including the Tyvaso Transition group having continued on LIQ861 at a high rate, suggest that LIQ861 is being dosed at therapeutic levels."

As recently reported, 95% of patients transitioning to LIQ861 from stable Tyvaso treatment (Transition patients) and 91% of patients on no more than two non-prostacyclin oral PAH therapies (Add-on patients) have remained on LIQ861 at two months of treatment. Furthermore, positive trends were observed at Month 2 in the exploratory endpoints for both Transition and Add-on patients with median measures of physical activity (6MWD) and quality of life (MLHFQ) remaining stable or improving for patients' having both Functional Class II or III PAH.

Neal Fowler, Chief Executive Officer of Liquidia, stated: "While we have initiated some additional work to supplement the PK data set, we remain encouraged by our clinical observations and the feedback from investigators and the PAH community. We remain highly enthusiastic about LIQ861 as demonstrated in our INSPIRE trial and are fully committed to bringing LIQ861 to the PAH community to better the lives of individuals living with PAH."

About LIQ861

Liquidia has developed LIQ861, a dry powder formulation of treprostinil utilizing PRINT® Technology, which is specifically designed to enhance deep-lung delivery and enables QID delivery of treprostinil doses in 1 to 2 breaths per capsule via a convenient, palm-sized dry powder inhaler (DPI). PRINT® Technology results in a treprostinil drug product with particles of a precise, uniform size, shape and composition that are engineered for optimal deposition in the lung following oral inhalation using a DPI. LIQ861 may enhance lung delivery and pharmacodynamic effects of treprostinil in patients diagnosed with PAH.

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 (Transition) 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 (Add-on). The primary objective of the INSPIRE study is to evaluate the long-term safety and tolerability of LIQ861. INSPIRE also includes exploratory endpoints to assess clinical benefits such as 6 Minute Walk Distance (6MWD) and quality of life factors from the Minnesota Living with Heart Failure Questionnaire (MLHFQ). For more information, please visit https://clinicaltrials.gov/ct2/show/NCT03399604.

About Liquidia Technologies

Liquidia is a late-stage clinical biopharmaceutical company focused on the development and commercialization of 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 dry powder inhaler. 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 Liquidia's website at www.liquidia.com.

Tyvaso® is a registered trademark of United Therapeutics Corporation.

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 a new drug application (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

LIQUIDIA_Logo.jpg

Source: Liquidia Technologies, Inc.