Liquidia Releases Exploratory Endpoint Data from INSPIRE Study at the American Thoracic Society (ATS) Annual Meeting

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New data from INSPIRE LIQ861 safety and tolerability study in pulmonary arterial hypertension (PAH) patients demonstrate positive trends in exploratory endpoints, including quality of life (QoL)

RESEARCH TRIANGLE PARK, N.C., Aug. 05, 2020 (GLOBE NEWSWIRE) -- Liquidia Technologies, Inc. (NASDAQ: LQDA), a late-stage clinical biopharmaceutical company focused on the development and commercialization of novel products using its proprietary PRINT® technology, announced that exploratory endpoint data from the INSPIRE study evaluating LIQ861 in patients with pulmonary arterial hypertension (PAH) was released today through a virtual presentation at the American Thoracic Society (ATS) Annual Meeting.

LIQ861 is an investigational, inhaled dry powder formulation of treprostinil designed and engineered using Liquidia's novel PRINT technology with the goal of enhancing deep-lung delivery of treprostinil in PAH patients by means of a convenient, palm-sized dry powder inhaler.

The data from the INSPIRE study were made available via a prerecorded poster session and showed results from standard measures used to evaluate clinical symptoms and functional ability, which were exploratory endpoints and not subject to formal statistical analysis. Overall, improvements from baseline following treatment with LIQ861 for two months included an increase in six-minute walk distance (6MWD); clinically meaningful reduction (improvement) in the Minnesota LIVING WITH HEART FAILURE® Questionnaire (MLHFQ) total score; lower (improved) New York Heart Association functional class (NYHA FC) status; and the percentage of subjects who met two or three PAH low-risk criteria increased from baseline.

"We are very pleased with the results of LIQ861 on these exploratory endpoints and believe these findings warrant further evaluation in this difficult to treat patient population," said Nicholas Hill, MD, Chief Pulmonary, Critical Care & Sleep Division and Professor of Medicine at Tufts University School of Medicine and INSPIRE Principal Investigator. "Previously reported primary endpoints from the INSPIRE study support the safety and tolerability profile of LIQ861 and a continuation of positive trends seen in these exploratory endpoints will only reinforce its potential as a viable treatment option, if approved, for patients with PAH."

INSPIRE, a phase 3, open-label, multicenter study of two World Health Organization (WHO) Group 1 pulmonary arterial hypertension (PAH) patient cohorts, enrolled a total of 121 patients. The first patient group included stable PAH patients on ≤2 non-prostacyclin PAH therapies (add-on) and the second group included subjects who transitioned from treatment with Tyvaso® (treprostinil) (transition).

The initial dose for transition patients (n=55) was based on their Tyvaso® dose at the time of transition and the starting dose for add-on patients (n=66) was 26.5 mcg QID. Titration to a higher LIQ861 dose was permitted in both groups based on symptom relief at the discretion of the physicians.

Results of the exploratory endpoints evaluated in the INSPIRE study showed:

- More than 70 percent of patients were able to titrate to a LIQ861 dose ≥79.5 mcg.
- NYHA functional class improved in 20.5 percent of patients and maintained in 75.9 percent.
- Overall median 6MWD increased by 10.1 m.
- There was no clinically meaningful change in NT-proBNP.
- MLHFQ showed an improved total score (>5-point reduction), as well as in both emotional and physical dimensions.
- A larger percentage of patients met two-or-three PAH low-risk criteria at month 2 compared with baseline.
- The majority of transition patients preferred the LIQ861 dry-powder inhaler to the Tyvaso® Inhalation System.

"The PAH community has substantial unmet needs, like so many others with rare disease," said Tushar Shah, M.D., Chief Medical Officer of Liquidia. "By leveraging our propriety PRINT technology, we were able to develop a convenient, dry powder inhaler that delivers the required dose of highly uniform treprostinil particles in one-to-two breaths. If approved, we believe that the LIQ861 dry powder inhaler could offer PAH patients a much-needed alternative treatment option and we are deeply committed to delivering such options to the PAH community."

The INSPIRE study safety and tolerability outcomes at month 2 were recently presented at the International Society for Heart & Lung Transplantation (ISHLT) Annual Meeting. Furthermore, Liquidia submitted the New Drug Application (NDA) for LIQ861 to the U.S. Food and Drug Administration (FDA) under the 505(b)(2) regulatory pathway, which includes data from three clinical studies establishing the safety, tolerability and pharmacokinetic profile of LIQ861. On April 8, 2020, Liquidia announced the FDA had accepted the NDA for review and that it had been provided a Prescription Drug User Fee Act (PDUFA) goal date of November 24, 2020.

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, was 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 under 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 was to evaluate the long-term safety and tolerability of LIQ861 and was observed to be well-tolerated in 121 patients, with 113 patients (93 percent) completing their two-month visit. During that period, LIQ861 was evaluated at doses ranging from 26.5 mcg to 159 mcg 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 (reported in ≥4 percent) being cough (42 percent), headache (26 percent), throat irritation (16 percent),

dizziness (11 percent), diarrhea (9 percent), chest discomfort (8 percent), nausea (7 percent), dyspnea (5 percent), flushing (5 percent) and oropharyngeal pain (4 percent).

For more information, please visit https://clinicaltrials.gov/ct2/show/NCT03399604.

About PAH
PAH is a chronic, progressive disease caused by the hardening and narrowing of the pulmonary arteries that can lead to right heart failure and eventually death. Treprostinil is a synthetic analog of prostacyclin, a vasoactive mediator essential to normal lung function that is deficient in patients with PAH. PAH is a rare disease, with an estimated prevalence in the United States of approximately 30,000 patients. The exact cause of PAH is often unknown and, although the symptoms are treatable, there is no known cure for the disease.

About Liquidia
Liquidia is a late-stage clinical biopharmaceutical company focused on the development and commercialization of novel products using its proprietary PRINT® technology to transform the lives of patients. PRINT is a particle engineering platform that enables precise production of uniform drug particles designed to improve the safety, efficacy and performance of a wide range of therapies. Currently, Liquidia is focused on the development of two product candidates for which it holds worldwide commercial rights: LIQ861 for the treatment of pulmonary arterial hypertension (PAH) and LIQ865 for the treatment of local post-operative pain. Liquidia is headquartered in Research Triangle Park, NC. For more information, please visit www.liquidia.com.

Cautionary Statements Regarding 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 strategic and financial initiatives, 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 potential U.S. Food and Drug Administration (FDA) approval of the New Drug Application (NDA) for LIQ861, the timeline or outcome related to our patent litigation pending in the U.S. District Court for the District of Delaware or two petitions for *inter partes* review with the Patent Trial and Appeal Board, and our ability to execute on our strategic or financial initiatives, involve significant risks and uncertainties and actual results could differ materially from those expressed or implied herein. The words “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “plan,” “potential,” “predict,” “project,” “should,” “target,” “would,” 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, including the risk that our proposed acquisition of RareGen, LLC is not consummated, the impact of the coronavirus (COVID-19) outbreak on our company and our financial condition and results of operations, 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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