



## Liquidia Technologies Presents Phase 3 Trial Data for LIQ861 at the ISHLT 39th Annual Meeting & Scientific Sessions

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### Positive Data from INSPIRE Trial Continues to Highlight Safety and Tolerability Along with Improvement in Quality of Life Metrics from LIQ861

RESEARCH TRIANGLE PARK, N.C., April 03, 2019 (GLOBE NEWSWIRE) -- Liquidia Technologies, Inc. (Nasdaq: LQDA) ("Liquidia"), a late-stage clinical biopharmaceutical company focused on the development and commercialization of LIQ861, announced today additional detailed safety and exploratory endpoint findings at the two-month timepoint for the Phase 3 INSPIRE trial. Nicholas Hill, MD, Chief Pulmonary, Critical Care & Sleep Division and Professor of Medicine at Tufts University School of Medicine and INSPIRE Principal Investigator, presented the data at the opening plenary session of the 39<sup>th</sup> International Society for Heart & Lung Transplantation (ISHLT) Meeting and Scientific Sessions in Orlando, Florida. Liquidia is pursuing approval of LIQ861, an inhalation dry powder formulation of treprostinil that is produced using its PRINT® Technology, as an alternative to current inhaled treprostinil therapy for the treatment of patients with pulmonary arterial hypertension (PAH) (WHO Group 1).

"The two-month results of the INSPIRE trial are promising for patients with PAH. Inhaled therapy offers the benefit of getting drug directly to the lungs and we are encouraged that the safety, tolerability and quality of life metrics suggest that LIQ861 is an attractive and more convenient therapy versus the currently available inhaled therapies," said Dr. Hill.

In March 2019, Liquidia reported [two-month top-line results](#) of the Phase 3 INSPIRE study, which indicated that the study had met its primary endpoint of safety and tolerability of LIQ861 in (PAH) patients. The ISHLT presentation, "*INSPIRE: A Phase 3 Open-Label, Multicenter Study to Evaluate the Safety and Tolerability of LIQ861 in PAH*," provides detailed results on the treatment emergent adverse event (TEAE) profile, duration of treatment by group (including those adding LIQ861 to non-prostacyclin oral therapy and those transitioning from Tyvaso®\*) along with the performance of LIQ861 against exploratory endpoints such as patient quality of life metrics.

"We are continuing to demonstrate the potential benefits of LIQ861 as we present additional data from the INSPIRE trial," said Neal Fowler, Chief Executive Officer of Liquidia. "LIQ861 is a convenient inhaled therapy that effectively manages PAH with excellent safety and tolerability. It's our belief that LIQ861 can help PAH patients more easily manage their disease, and more importantly, the quality of their lives."

#### Safety

The INSPIRE trial includes two groups of PAH patients, comprised of 65 prostacyclin-naïve patients that were stable on  $\leq 2$  approved oral PAH therapies (add-on) and 44 patients transitioning from Tyvaso® (transition). Most TEAEs occurred in the add-on population and were observed mainly during initial exposure to LIQ861 at the 25mcg dose. Overall, TEAEs observed were consistent with inhaled prostacyclins and considered mild to moderate, including cough, headache and throat irritation. No serious adverse events (SAEs) related to LIQ861 were observed during the two month period.

#### Duration of Treatment and Patient Quality of Life

Most patients, 101 of 109 (92.7%), remained on LIQ861 at two months duration of treatment, with 59 of 65 (90.8%) add-on patients and 42 of 44 (95.5%) transition patients. Patients continue to receive treatment beyond month 2 with initial dosing in March 2018.

Exploratory endpoints of the INSPIRE trial demonstrated favorable functional and patient outcomes, as indicated by the Minnesota Living with Heart Failure Questionnaire (MLWHFQ) and measurement of six-minute walk distance (6MWD). MLWHFQ is a patient-oriented measure of the adverse effects of heart failure on a patient's physical and emotional aspects of life. A five-point change is the minimal difference considered to be clinically important. The median MLWHFQ score at month 2 for all patients improved from 34 to 24, with significant changes observed in both the add-on and transition patient groups, from 37 to 29 and 30 to 19, respectively. Median 6MWD at baseline and 2 months was 395m and 408m in the add-on group and 428m and 449m in the transition group.

#### 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](http://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.

\*Tyvaso® is a registered trademark of United Therapeutics Corporation.

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