Liquidia Announces the Publication of Long-Term Clinical Data from Completed INSPIRE Study in the Journal Pulmonary Circulation

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- *Evaluated doses of YUTREPIA™ (treprostinil) inhalation powder from 26.5 mcg to 212 mcg which are comparable to 3 to 24 breaths of nebulized Tyvaso® per session*
- *Achieved therapeutic levels by Month 2 and continued to titrate to higher levels for approximately one year on average before rolling into extension trial*
- *Safety profile was consistent with known prostacyclin side effects and mostly mild to moderate in severity whether naïve to prostacyclin therapy or transitioning from a stable dose of Tyvaso*

MORRISVILLE, N.C., Aug. 01, 2022 (GLOBE NEWSWIRE) -- Liquidia Corporation (NASDAQ: LQDA) announced today the publication of clinical data from the completed INSPIRE study evaluating YUTREPIA™ (treprostinil) inhalation powder, formerly known as LIQ861, in patients with pulmonary arterial hypertension (PAH) in the journal *Pulmonary Circulation*.

This publication reports clinical outcomes of PAH patients treated with YUTREPIA who were naïve to prostacyclin therapy (Naïve patients) or transitioning from nebulized Tyvaso® (Transition patients). The average length of treatment was approximately one year, with patients rolling into an open-label safety extension trial at varying times between 8 to 18 months. Observations noted in the publication include:

- YUTREPIA can be safely titrated using a dry powder inhaler to doses comparable to 24 breaths of Tyvaso, indicating that a wide range of therapeutic doses can be more easily administered in just a few breaths compared to nebulizers
- Naïve patients can safely achieve known therapeutic levels within 2 months and titrate to doses above the recommended, comparable dose range of 9-12 breath of Tyvaso
- Both Naïve and Transition patients maintained or improved in exploratory measures of clinical efficacy; however, the study was not designed or controlled to make definitive statements

Dr. Rajeev Saggar, Chief Medical Officer at Liquidia, said: “The INSPIRE study confirms the long-term safety profile, dosing convenience, and tolerability of YUTREPIA in PAH patients. We are grateful to the efforts of the clinical investigation teams, our steering committee, and most importantly to the PAH community who participated in this study and those continuing in the open-label extension.”

INSPIRE Study. The pivotal, open label INSPIRE study enrolled 121 adult PAH patients naïve to prostacyclin (n=66, Naive) and patients who transitioned from nebulized Tyvaso (n=55, Transition). The primary objective of the study was to evaluate the safety and tolerability of YUTREPIA as measured by the incidence of adverse events (AEs) and Serious Adverse Events (SAEs) after 2 months of treatment.

A total of 113 out of 121 (93 percent) completed the 2-month treatment phase. Top line data showing that the INSPIRE study met its primary objective were released in April 2020 and presented at the International Society for Heart and Lung Transplantation Virtual Congress. The pivotal data served as the basis for the New Drug Application for YUTREPIA, which received tentative approval by the US Food & Drug Administration (FDA) in November 2021.

The INSPIRE protocol allowed patients to continue treatment with YUTREPIA beyond the primary endpoint at Month 2. The average length of treatment during the INSPIRE study was approximately one year, with patients rolling over into an open label extension trial at varying times between 8 to 18 months. A total of 69 patients completed at least 12 months of treatment in the INSPIRE study.

Upon completion of the INSPIRE study, YUTREPIA had been evaluated at doses ranging from 26.5 mcg to 212 mcg (comparable to 3 to 24 breaths of Tyvaso) four times daily. At Month 12, 79% of Transition patients and 91% of Naïve patients achieved a dose greater than or equal to 79.5 mcg YUTREPIA, the comparable dose to 9 breaths of Tyvaso, four times a day. There were no study drug-related SAEs. Most AEs experienced during the study were mild to moderate in severity and consistent with the known side effects of inhaled treprostinil therapy. The safety observations generally did not hinder patients’ ability to continue treatment with YUTREPIA and titrate to higher doses as needed.

The INSPIRE study was not designed or powered to evaluate a specific efficacy-related hypothesis; however, exploratory endpoints of clinical efficacy were collected, with the following observations:

- **6MWD.** A slight increase in the 6MWD in Naïve and Transition patients, with larger increases evident in the Transition group which remained stable at Month 12.

- **NYHA Functional Class.** An increase in the percentage of patients in New York heart Association (NYHA) Functional Class I and II for both Naïve and Transition groups, compared with baseline, was observed at Month 2 and maintained at Month 12. The data at Month 2 was presented at American Thoracic Society 2020 International Conference.

- **PAH Risk Score.** A higher percentage of patients had two or more low risk criteria at Months 2, 4, 8, and 12. At Month 12, the percentage of patients meeting 2 or 3 low-risk criteria was 59.0% for Naïve patients and 67.8% for Transition patients.

- **NT-proBNP levels.** Variable changes in the levels of N-terminal-pro hormone BNP (NT-proBNP) with no clear trends in Transition patients, though Naïve patients saw a progressive decline from Month 4 to Month 12.

- **Quality of Life.** Overall, there was a clinically meaningful improvement from baseline to Month 2 and Month 4 in Minnesota Living with Heart Failure questionnaire (MLHFQ) total score, with significant decreases in both emotional and physical dimension scores in both patient groups. The MLHFQ was not administered after Month 4.

- **Device preference.** All the Transition patients preferred or strongly preferred the YUTREPIA device when surveyed about their preferences compared to nebulized Tyvaso at Month 4.

While the INSPIRE trials provide approximately one year's safety data, data from an ongoing open-label extension study will provide up to three years of long-term safety and clinical effectiveness information for YUTREPIA in PAH.

The manuscript, entitled "INSPIRE: Safety and Tolerability of Inhaled YUTREPIA (Treprostinil) in Pulmonary Arterial Hypertension (PAH)" is available on the Pulmonary Circulation Journal website and at https://doi.org/10.1002/pul2.12119.

In November 2021, the US Food & Drug Administration (FDA) issued tentative approval of YUTREPIA based on the primary endpoint of the INSPIRE trial and comparable bioavailability to Tyvaso in a Phase 1 study. Due to a regulatory stay pursuant to the Drug Price Competition and Patent Term Restoration Act (Hatch-Waxman Act), YUTREPIA cannot yet be marketed in the United States until the expiration of the regulatory stay or resolution of the lawsuit filed by United Therapeutics against Liquidia in June 2020 for alleged infringement of three patents related to Tyvaso. A decision from the District Court is expected before the expiration of the regulatory stay on October 27, 2022.

Tyvaso® is a registered trademark of United Therapeutics Corporation.

About Liquidia Corporation
Liquidia Corporation is a biopharmaceutical company focused on the development and commercialization of products in pulmonary hypertension and other applications of its PRINT® Technology. The company operates through its two wholly owned subsidiaries, Liquidia Technologies, Inc. and Liquidia PAH, LLC. Liquidia Technologies has developed YUTREPIA™ (treprostinil) inhalation powder for the treatment of pulmonary arterial hypertension (PAH). Liquidia PAH provides the commercialization for rare disease pharmaceutical products, such as generic Treprostinil Injection. For more information, please visit www.liquidia.com.

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