Liquidia Provides Update on Clinical Pipeline Targeting PAH and PH-ILD

January 5, 2024

- Enrolled first PH-ILD patient in the open-label ASCENT study of YUTREPIA
- Confirmed with FDA that a single pivotal efficacy trial with L606 will support PAH and PH-ILD indications

MORRISVILLE, N.C., Jan. 05, 2024 (GLOBE NEWSWIRE) -- Liquidia Corporation (NASDAQ: LQDA) (Liquidia or the Company) provided an update today on the clinical progress of its pipeline programs to treat pulmonary arterial hypertension (PAH) and pulmonary hypertension associated with interstitial lung disease (PH-ILD) with its investigational drugs, YUTREPIA™ (treprostinil) inhalation powder and L606, a sustained-release inhaled formulation of treprostinil administered twice-daily with a short-duration next-generation nebulizer.

With respect to YUTREPIA, the first PH-ILD patient was enrolled in December 2023 in the Open-Label Prospective Multicenter Study to Evaluate Safety and Tolerability of Dry Powder Inhaled Treprostinil in Pulmonary Hypertension, referred to as the ASCENT study. The ASCENT study will enroll approximately 60 subjects to further inform YUTREPIA's dosing and tolerability profile in patients with PH-ILD. Exploratory efficacy endpoints will also be assessed. YUTREPIA is currently being reviewed for tentative approval for PH-ILD by the U.S. Food and Drug Administration (FDA) with a Prescription Drug User Fee Act (PDUFA) goal date of January 24, 2024. The FDA has previously confirmed that Liquidia may add the treatment of PH-ILD to the label for YUTREPIA without additional clinical studies.

With respect to L606, Liquidia successfully concluded a Type C meeting with the FDA in December 2023 and reached agreement regarding the registration requirements for L606 using the 505(b)(2) regulatory pathway. It was agreed that only a single Phase 3 placebo-controlled efficacy trial in PH-ILD patients would be required to support indications to treat both PAH and PH-ILD. The Company is preparing to initiate a global study later in 2024.

Liquidia will also continue to enroll patients in its ongoing open-label, multicenter safety study of L606 in U.S. patients with PAH or PH-ILD. This study will enroll approximately 60 subjects and include patients with PAH, who are naïve to prostacyclins or transitioning from Tyvaso® or Tyvaso DPI®, and patients with PH-ILD, who are transitioning from Tyvaso® or Tyvaso DPI®. The study is now more than one third enrolled and includes some patients who have been successfully treated with L606 for longer than one year and at doses comparable to 25 to 27 breaths of Tyvaso, four times daily. The Company currently anticipates the open-label study will be fully enrolled in 2024 and intends to provide interim updates at medical conferences later in the year.

Rajeev Saggar, M.D., Chief Medical Officer of Liquidia, stated: "YUTREPIA will provide practitioners and patients the opportunity to comfortably use a low-resistance dry-powder inhaler across a wide range of doses to a broad range of patients with PAH and PH-ILD with varying lung function and clinical severity. We are also pleased by the increasing interest to enroll patients in the L606 open-label study. The real-time data on patient tolerability, dosing titration, and clinical response will greatly inform the pivotal efficacy trial and should provide additional confidence in the benefits of more consistent, sustained exposures over a 24-hour period."

More information about the clinical studies described is available at ClinicalTrials.gov and associated with the trial identifier NCT06129240 (ASCENT study) and NCT04691154 (open-label L606).

About YUTREPIA™(treprostinil) inhalation powder

YUTREPIA is an investigational, inhaled dry powder formulation of treprostinil delivered through a convenient, low-resistance, palm-sized device. On November 5, 2021, the FDA issued a tentative approval for YUTREPIA for the treatment of PAH to improve exercise ability in adult patients with New York Heart Association (NYHA) Functional Class II-III symptoms. In July 2023, Liquidia filed an amendment to its New Drug Application for YUTREPIA, seeking to add PH-ILD to the label. The FDA has set a PDUFA goal date of January 24, 2024 for the amendment. Previously, the FDA has confirmed that YUTREPIA may add the treatment of PH-ILD to the label for YUTREPIA without additional clinical studies. YUTREPIA was designed using Liquidia's PRINT ® technology, which enables the development of drug particles that are precise and uniform in size, shape, and composition, and that are engineered for enhanced deposition in the lung following oral inhalation. Liquidia has completed INSPIRE, or Investigation of the Safety and Pharmacology of Dry Powder Inhalation of Treprostinil, an open-label, multi-center phase 3 clinical study of YUTREPIA in patients diagnosed with PAH who are naïve to inhaled treprostinil or who are transitioning from Tyvaso® (nebulized treprostinil). YUTREPIA was previously referred to as LIQ861 in investigational studies.

About L606 (liposomal treprostinil) inhalation suspension

L606 is an investigational, sustained-release formulation of treprostinil administered twice-daily with a short-duration next-generation nebulizer. The L606 suspension uses Pharmosa Biopharm's proprietary liposomal formulation to encapsulate treprostinil which can be released slowly at a controlled rate into the lung, enhancing drug exposure over an extended period of

time and reducing local irritation of the upper respiratory tract. L606 is currently being evaluated in an open-label study in the United States for treatment of PAH and PH-ILD with a planned pivotal study for the treatment of PH-ILD.

About pulmonary arterial hypertension (PAH)

PAH is a rare, chronic, progressive disease caused by hardening and narrowing of the pulmonary arteries that can lead to right heart failure and eventually death. Currently, an estimated 45,000 patients are diagnosed and treated in the United States. There is currently no cure for PAH, so the goals of existing treatments are to alleviate symptoms, maintain or improve functional class, delay disease progression, and improve quality of life.

About pulmonary hypertension associated with interstitial lung disease (PH-ILD)

Pulmonary hypertension (PH) associated with interstitial lung disease (ILD) includes a diverse collection of up to 150 different pulmonary diseases, including interstitial pulmonary fibrosis, chronic hypersensitivity pneumonitis, connective tissue disease related ILD, and chronic pulmonary fibrosis with emphysema (CPFE) among others. Any level of PH in ILD patients is associated with poor 3-year survival. A current estimate of PH-ILD prevalence in the United States is greater than 60,000 patients, though population growth in many of these underlying ILD diseases is not yet known due to factors including underdiagnosis and lack of approved treatments until March 2021, when inhaled treprostinil was first approved for this indication.

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. (Liquidia Technologies) and Liquidia PAH, LLC (Liquidia PAH). Liquidia Technologies has developed YUTREPIA™ (treprostinil) inhalation powder for the treatment of PAH and PH-ILD. Liquidia Technologies is also developing L606, an investigational liposomal formulation of treprostinil administered twice-daily with a short-duration next-generation nebulizer, for use in North America. Liquidia PAH provides for the commercialization of pharmaceutical products to treat pulmonary disease, such as generic Treprostinil Injection. For more information, please visit www.liquidia.com.

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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 submission contents and timelines, including the potential for final FDA approval of the NDA for YUTREPIA, the timeline or outcome related to patent litigation in the U.S. District Court for the District of Delaware or *inter partes* review proceedings conducted at the PTAB, including appeals of decisions in any such proceedings, the issuance of patents by the USPTO 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 favorable decisions of lower tribunals are not determinative of the outcome of the appeals of the decisions. 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 SEC, including 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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