



## Liquidia Presents Positive LIQ861 Results at the Annual World Congress of the Pulmonary Vascular Research Institute

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RESEARCH TRIANGLE PARK, NC

*LIQ861, being evaluated for pulmonary arterial hypertension (PAH), was well-tolerated up to 150 mcg when delivered locally to the lungs*

*LIQ861 demonstrated dose proportionality between 25 mcg and 150 mcg*

[Liquidia Technologies, Inc.](#), a late-stage clinical biopharmaceutical company focused on improving the performance of medicine by precisely engineering drug particles, today announced positive Phase 1 and preclinical results evaluating LIQ861 for the treatment of PAH at the 12<sup>th</sup> Pulmonary Vascular Research Institute (PVRI) Annual World Congress on Pulmonary Vascular Disease in Singapore. LIQ861, developed using [Liquidia's proprietary PRINT® technology](#), is a powder formulation of treprostinil designed for deep-lung delivery using a disposable, dry powder inhaler (DPI). The safety, tolerability and pharmacokinetics results from the studies presented at PVRI support advancing the program into the open-label Phase 3 trial (INSPIRE), [which initiated earlier in January 2018](#).

In the randomized, placebo-controlled, double-blinded Phase 1 clinical trial, 57 healthy volunteers were evaluated, following a single administration of LIQ861 at doses between 25 mcg and 150 mcg. LIQ861 was well-tolerated at all doses tested, with a proportional dose response in pharmacokinetics. No serious adverse events were observed for all LIQ861 doses up to 150 mcg. Notably, at both 100 mcg and 150 mcg doses of LIQ861, 50% of individuals had measurable treprostinil at four hours, which could minimize PAH symptoms between dosing cycles. The scientific poster presentation summarizing the Phase 1 and preclinical results is available on the [Publications](#) section of Liquidia's corporate website.

"We are pleased to be presenting these exciting findings at PVRI and are encouraged by the data generated to-date, as we believe LIQ861 can help overcome some of the limitations of current nebulized therapies," stated Neal Fowler, Chief Executive Officer for Liquidia. "LIQ861 has the potential to maximize the therapeutic benefits of treprostinil in treating PAH by safely and efficiently delivering higher doses of drug deeply into the lungs using a convenient, disposable DPI. These early findings support advancing directly into a Phase 3 clinical trial and bring us closer to being able to offer patients a potential game-changer for treating PAH."

PAH is a rare, chronic and progressive disease caused by the hardening and narrowing of the pulmonary arteries, which often results in heart failure. Previously approved by the U.S. Food and Drug Administration (FDA) in parenteral, nebulized and oral formulations, treprostinil is a synthetic analog of prostacyclin, a vasoactive mediator deficient in patients with PAH yet essential to normal lung function to regulate vessel tone.

"Local delivery of prostacyclin analogs to the lungs is the ideal route of administration, as it minimizes the off-tissue adverse side effects of systemic delivery by delivering the drug where it is needed in the lungs," said Robert Roscigno, Ph.D., Senior Vice President of Product Development and LIQ861 Program Lead for Liquidia. "The clinical use of inhaled prostacyclin analogs is currently limited to nebulized therapies, which are inherently constrained by specific safety profiles, dosing convenience and efficacy. Prostacyclin analogs are central to PAH therapy and as such, there exists a strong need to develop products that can maximize their therapeutic benefits."

LIQ861 is currently being evaluated in INSPIRE (**I**nvestigation of the **S**afety and **P**harmacology of Dry Powder Inhalation of **T**reprostinil), an open-label Phase 3 clinical trial, which expects to enroll at least 100 patients with PAH across multiple U.S. sites. Primary endpoints are long-term safety and tolerability of LIQ861. Topline data from INSPIRE are expected in 2019. Based on feedback from the FDA, Liquidia believes that INSPIRE will support the filing of an NDA for LIQ861. Additional details regarding the trial are available at [www.clinicaltrials.gov](http://www.clinicaltrials.gov).

### ABOUT THE PHASE 1 TRIAL

Liquidia conducted a randomized, placebo-controlled, double-blind, Phase 1 clinical trial via a dry powder inhaler in healthy volunteers to assess safety, tolerability and pharmacokinetics following a single administration of LIQ861 at doses between 25 mcg and 150 mcg. The 57 subjects were enrolled into six dose cohorts. Within each dose cohort, subjects were randomized to receive LIQ861 or a placebo.

### ABOUT PULMONARY ARTERIAL HYPERTENSION

PAH is a rare, chronic and progressive disease caused by the hardening and narrowing of the pulmonary arteries, which often results in heart failure and eventually death. Symptoms of PAH include shortness of breath, dizziness and fatigue, which grow more severe as the disease progresses. There is no cure for PAH; current treatments rely on symptom management. Defined as WHO Group I, PAH is part of a larger classification of pulmonary hypertension which is divided into five groups based on WHO criteria.

### ABOUT LIQUIDIA TECHNOLOGIES

Liquidia Technologies is a late-stage clinical biopharmaceutical company that is focused on improving the performance of medicines by precisely engineering drug particles. Liquidia's proprietary PRINT® technology is designed to optimize the safety, efficacy or route of administration of a wide range of therapies by engineering uniform drug particles in a wide variety of compositions, sizes and shapes. Currently, Liquidia is developing two of its own product candidates using PRINT® particles: LIQ861 for the treatment of PAH and LIQ865 for the treatment of post-operative pain. Our lead product candidate, LIQ861, currently being evaluated in a Phase 3 clinical trial (INSPIRE), is designed to improve the therapeutic profile of treprostinil by enabling deep-lung delivery and higher dose levels than current therapies by using a convenient, disposable dry powder inhaler. LIQ865, currently being evaluated in a Phase 1 clinical trial, is designed to deliver sustained release particles of the non-opioid bupivacaine, a local analgesic, to treat post-operative pain. In addition to developing its own product candidates, Liquidia collaborates with leading pharmaceutical companies to apply its PRINT® technology across a wide range of therapeutic areas, molecule types and routes of administration. Liquidia is based in Research Triangle

Park, North Carolina. For more information, please visit [www.liquidia.com](http://www.liquidia.com).

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