A Continued ASCENT to Week 24: Safety and Exploratory Efficacy Data on LIQ861 Dry Powder Inhaled Treprostinil in PH-ILD Patients

Poster #83

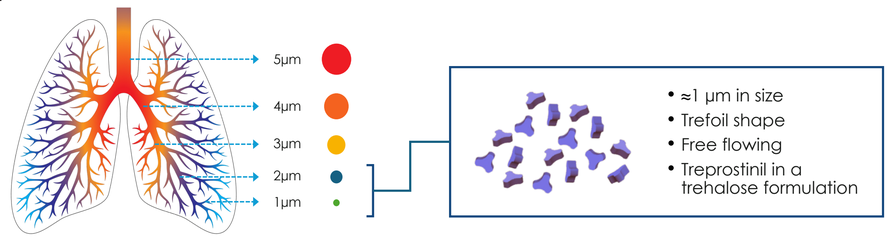
Rajan Saggar,[1] Ashwin Ravichandran,[2] Nicholas Kolaitis,[3] Akshay Muralidhar,[4] Jeremy Feldman,[5] M. Patricia George,[6] Ricardo Restrepo-Jaramillo,[7] John Ryan,[8] Savan Patel,[9] Ashley Galloway,[9] Rajeev Saggar,[9] James White,[10] Vallerie McLaughlin[11]

[1]UCLA Health, Los Angeles, California; [2]Ascension St. Vincent, Indianapolis, Indiana; [3]UCSF Health, San Francisco, California; [4]Arizona Pulmonary Associates, Phoenix, Arizona; [5]Summit Health Oregon, Bend, Oregon; [6]National Jewish Health, Denver, Colorado; [7]University of South Florida Health, Tampa, Florida; [8]University of Utah Health, Salt Lake City, Utah; [9]Liquidia Technologies, Morrisville, North Carolina; [10]University of Rochester Medical Center, Rochester, New York; [11]University of Michigan Health, Ann Arbor, Michigan

Rationale

- Pulmonary hypertension (PH) is a frequent complication of interstitial lung disease (ILD), and PH-ILD is associated with reduced exercise capacity and substantially increased morbidity and mortality[1]
- Nebulized treprostinil has demonstrated improvements in 6-minute walk distance (6MWD), especially at doses above 9 breaths per session (54 mcg)[2]
- LIQ861 (YUTREPIA™) is a dry powder inhaled formulation of treprostinil developed by Liquidia Technologies (**Figure 1**)
  - LIQ861 particles are designed to enhance deep-lung delivery[3, 4]
  - PRINT® technology produces uniformity of particle size, shape, and composition for deep-lung delivery[4-6]

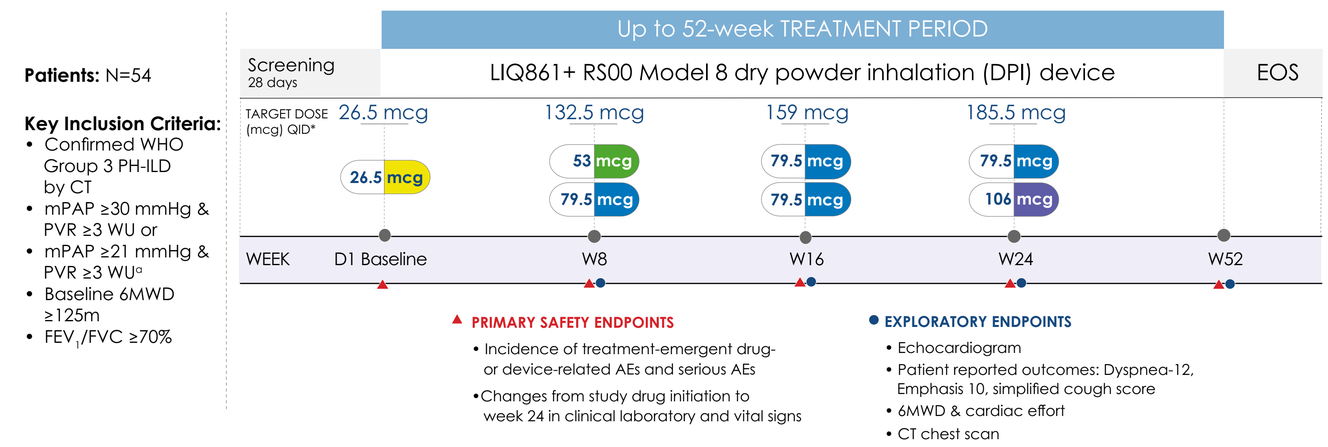
Figure 1. LIQ861 particles



Methods

- ASCENT (NCT06129240) is a prospective, multicenter, open-label study evaluating the safety and tolerability of LIQ861 in patients with PH-ILD, including combined pulmonary fibrosis and emphysema (CPFE; **Figure 2**)
- Here, we present data for the 6MWD exploratory endpoint and the Dyspnea-12,[7] EmPHasis-10,[8] and simplified cough score[9] patient-reported outcome questionnaires from patients who completed their Week 24 visit, including only data collected up to that timepoint

Figure 2. Study Design (Cohort A)



[a]Limited subset of patients with mild PH.
*Target dose based on tolerability and clinical response (**Figure 3**).
Abbreviations: 6MWD, 6-minute walk distance; AE, adverse event; CT, computed tomography; D, day; EOS, end of study; FEV_1, forced expiratory volume in 1 second; FVC, forced vital capacity; mPAP, mean pulmonary arterial pressure; PH-ILD, pulmonary hypertension associated with interstitial lung disease; PCWP, pulmonary capillary wedge pressure; PVR, pulmonary vascular resistance; QID, 4 times daily; W, week; WU, Wood units.

Figure 3. Dose Comparison Between TYVASO® and LIQ861

Number of TYVASO® (nebulized) breaths QID	LIQ861 QID dose (mcg)	LIQ861 capsule combination
≤5	26.5	1 Yellow
6 to 8	53	1 Green
9 to 11	79.5	1 Blue
12 to 14	106	1 Purple
15 to 17	132.5	1 Green + 1 Blue
≥18	159	2 Blue
≥21	185.5	1 Blue + 1 Purple
≥24	212	2 Purple

Abbreviation: QID, 4 times daily.
TYVASO® is a registered trademark of United Therapeutics Corporation.

Results

- A total of 73 patients were screened, and 54 patients were enrolled
- A total of 39 patients completed the Week 24 study visit
- The average age was 68.5 years, and 48.1% of the cohort was male (**Table 1**)
- The mean (SD) duration since diagnosis of PH and ILD was 0.5 (0.8) years and 5.1 (5.7) years, respectively
- ILD etiology included idiopathic interstitial pneumonias (48.1%), autoimmune ILDs (35.2%), CPFE (9.3%), and other (5.6%)
- Baseline mean (SD) pulmonary arterial pressure, pulmonary vascular resistance, and pulmonary capillary wedge pressure were 33.4 (8.4) mmHg, 6.0 (2.9) Wood units, and 8.6 (3.3) mmHg, respectively

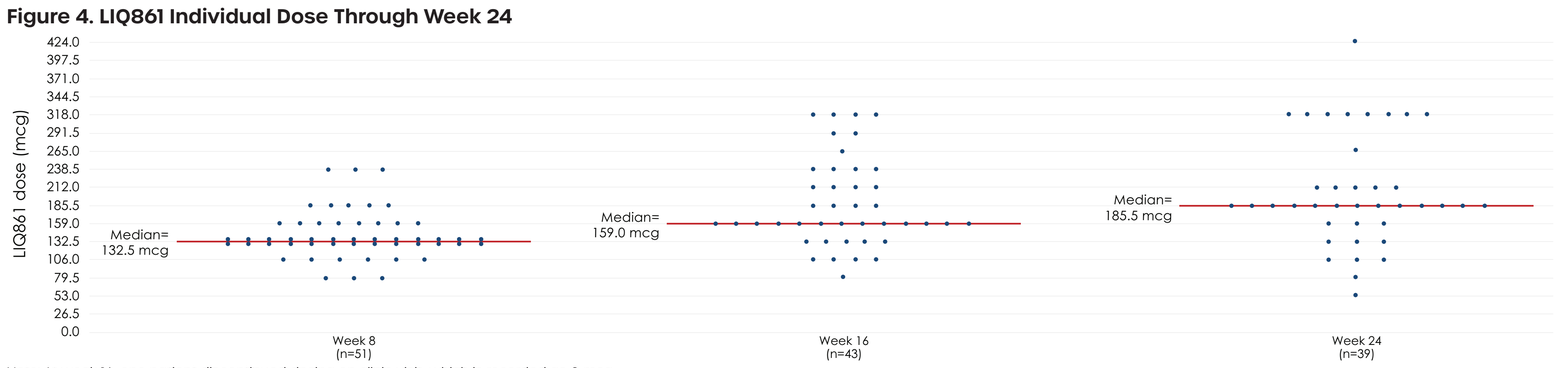
Table 1. Baseline Demographics and Clinical Characteristics

Characteristic	Overall (N=54*)
Age, y	68.5 (8.9)
Sex, n (%)	
Male	26 (48.1)
Female	28 (51.9)
Duration of PH diagnosis, y	0.5 (0.8)
Duration of ILD diagnosis, y	5.1 (5.7)
ILD type, n (%)	
Idiopathic interstitial pneumonias	26 (48.1)
Autoimmune ILDs	19 (35.2)
Chronic fibrosis with emphysema	5 (9.3)
Other ILDs	3 (5.6)
Hypersensitivity pneumonitis	1 (1.9)
Pulmonary function tests	
FEV_1, L	1.7 (0.6)
Percent FEV_1 predicted	69.7 (20.0)
FVC, L	2.1 (0.8)
Percent FVC predicted	65.9 (20.7)
Corrected DLCO, mmol/min/mmHg	8.2 (4.2)
Percent DLCO predicted	36.2 (13.9)
PIFR, min-max, L/min	39-120
Hemodynamics	
mPAP, mmHg	33.4 (8.4)
PCWP, mmHg	8.6 (3.3)
PVR, Wood units	6.0 (2.9)
Oxygen treatment, n (%)	
No	8 (14.8)
Yes	46 (85.2)
Background antifibrotics, n (%)	
Nintedanib	19 (35.2)
Pirfenidone	4 (7.4)
Background PH drugs, n (%)	
PDE5 inhibitor	7 (13.0)

Data are mean (SD) unless otherwise noted.
*Two patients had protocol violations and were excluded from the exploratory analyses.
Abbreviations: DLCO, diffusing capacity of the lung for carbon monoxide; FEV_1, forced expiratory volume in 1 second; FVC, forced vital capacity; ILD, interstitial lung disease; mPAP, mean pulmonary arterial pressure; PCWP, pulmonary capillary wedge pressure; PDE, phosphodiesterase; PH, pulmonary hypertension; PIFR, peak inspiratory flow rate; PVR, pulmonary vascular resistance.

- At week 24, the median dose of LIQ861 was 185.5 mcg 4 times daily (QID), ranging from 53 mcg to 424 mcg QID (**Figure 4**)

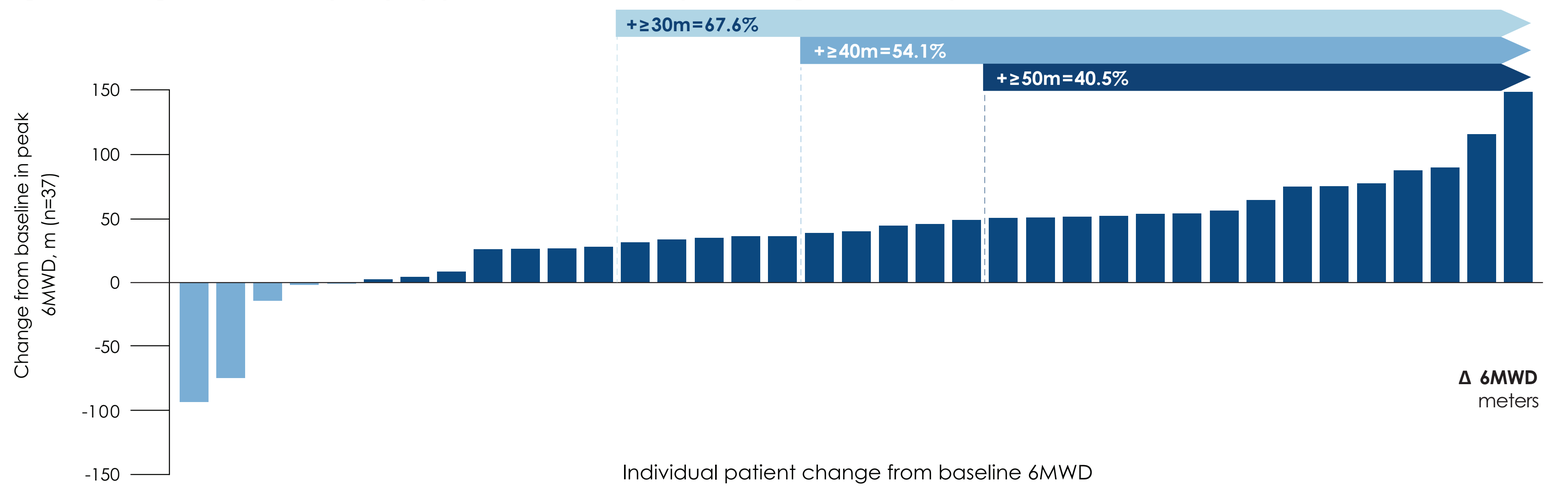
Figure 4. LIQ861 Individual Dose Through Week 24



Note: At week 16, one patient discontinued dosing on clinic visit, which is recorded as 0 mcg.

- The mean (SD) 6MWD increased from 304.0 (74.7) m at baseline to 343.6 (89.2) m at week 24, with a mean change of +39.6 (45.0) m
  - The median (min-max) 6MWD increased from 311.5 (148-502) m at baseline to 364.0 (135-567) m at week 24, with a median change from baseline of +41.0 m; 54.1% of patients improved ≥ +40 m and 40.5% improved ≥ +50 m (**Figure 5**)
- The mean (SD) Dyspnea-12 score improved from 10.3 (6.2) to 9.1 (6.3) and the mean (SD) EmPHasis-10 score improved from 23.0 (9.4) to 19.9 (9.8)
- Importantly, the mean (SD) simplified cough score remained stable, 1.4 (0.8) at baseline and 1.1 (0.7) at week 24

Figure 5. Change in Exercise Capacity, Dyspnea-12 Score, and Simplified Cough Score in Response to LIQ861 Treatment



Abbreviation: 6MWD, 6-minute walk distance.

- Treatment-related treatment-emergent adverse events (TEAEs) were reported in 70.4% of patients (**Table 2**)
  - The most common treatment-related TEAEs were cough (48.1% [n=24, 92.3% mild; n=2, 7.7% moderate]) and headache (18.5% [n=9, 90% mild; n=1, 10% moderate])
  - 1 patient had severe hypoxia and another patient had severe respiratory tract irritation
  - No treatment-related serious adverse events (SAEs) were observed
  - No patient discontinued the study drug due to cough
- 15 (27.8%) patients discontinued, none for drug-related reasons

Table 2. Summary of TEAEs

	Overall (N=54)
Patients with ≥1 TEAE, n (%)	52 (96.3)
Patients with ≥1 treatment-related TEAE, n (%)	38 (70.4)
Treatment-related TEAEs occurring in >2 patients, n (%)	
Cough	26 (48.1)
Headache	10 (18.5)
Fatigue	4 (7.4)
Oropharyngeal pain	4 (7.4)
Throat irritation	4 (7.4)
Diarrhea	3 (5.6)
Dry throat	3 (5.6)

Abbreviation: TEAE, treatment-emergent adverse event.

References
1. Waxman AB, et al. *Eur Respir Rev.* 2022;31(165):210220; **2.** Waxman A, et al. *N Engl J Med.* 2021;384(4):325-334; **3.** Hill NS, et al. *Pulm Circ.* 2022;12(3):e12119; **4.** Roscigno RF, et al. *Vascul Pharmacol.* 2021;138:106840; **5.** Garcia A, et al. *J Drug Deliv.* 2012;2012:941243; **6.** Henao MP, et al. *Int J Environ Res Public Health.* 2020;17(19):7287; **7.** Yorke J, et al. *Chest.* 2011;139(1):159-164; **8.** Yorke J, et al. *Eur Respir J.* 2014;43(4):1106-1113; **9.** Wang Z, et al. *J Thorac Dis.* 2019;11(10):4379-4388.

Acknowledgment and Funding
This study was funded by Liquidia Technologies, Inc. Medical writing and editorial support were provided by Vivek Shah, PhD, from Citrus Health Group (Chicago, Illinois) and were funded by Liquidia Technologies, Inc.

Author Disclosures
SS: Received compensation for advisory roles, advisory board participation, and speaker programs from Gossamer Bio, Johnson & Johnson, Liquidia, and United Therapeutics. **AR:** Receives speaking fees from United Therapeutics, and consulting and speaking fees from Janssen Pharmaceuticals. **CM:** Consultant for Actelion, Gossamer Bio, and Liquidia. **JR:** Honoraria and advisory board participation for Johnson & Johnson, Liquidia, Merck, and United Therapeutics. **MPG:** Received compensation for advisory board participation from Gossamer, Janssen, Liquidia, Merck, and United Therapeutics; for steering committee participation from Liquidia and United Therapeutics; for consulting from Liquidia; and for speaking from Bayer, Janssen, and United Therapeutics. **SM:** Served as a scientific consultant or sub-investigator for Gossamer Bio, Janssen, Liquidia, Merck, and United Therapeutics. **SP, AG, and RS:** Employees of Liquidia Technologies,Inc. **MW:** Received compensation for speaker bureau participation and consulting from Johnson & Johnson, Liquidia, Merck, and United Therapeutics; for speaker bureau participation from Bayer; for principal investigator roles from Merck and United Therapeutics; and for sub-investigator roles from Liquidia and Merck.

Presented at Pulmonary Vascular Research Institute | January 28 - February 1, 2026 | Dublin, Ireland

Conclusions

- Inhaled LIQ861 was well tolerated for the first 24 weeks in patients with PH-ILD
- The median dose was 132.5 mcg, 159 mcg, and 185.5 mcg QID at week 8, 16, and 24, respectively. The highest dose achieved was 424 mcg QID (comparable to ≥48 breaths of TYVASO®)
- Improvements in exercise capacity were observed at week 24, with a median change from baseline of +41.0 m in 6MWD and 40.5% of patients improved ≥50 m
- Improvements in patient-reported outcome measures, including the Dyspnea-12 and EmPHasis-10 scores, were also observed at week 24
- The mean simplified cough score was relatively unchanged to slightly improved from baseline to week 24