Open-label Study to Evaluate the Safety, Tolerability and Efficacy of Liposomal Treprostinil Inhalation Suspension (L606) in Subjects with Pulmonary Arterial Hypertension (PAH) or Pulmonary Hypertension Associated with Interstitial Lung Disease (PH-ILD)

Poster #: 68

Liquidia

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RATIONALE

L606 is a novel investigational inhaled sustained-release formulation of nanosized, liposomal treprostinil designed to prolong lung retention

The objectives of the trial are to evaluate the safety, tolerability, and exploratory efficacy of L606 up to 48 weeks

L606 is administered twice daily using a breath actuated nebulization device

METHODS

- Open-label study in 28 patients with PAH or PH-ILD who transitioned from inhaled Tyvaso® to L606 or were naïve to prior prostacyclin therapy

Figure 1. Phase 3 Study Evaluating Safety & Tolerability In PAH or PH-ILD Patients

PATIENTS
- N=28
- Age 18-80 years

KEY INCLUSION CRITERIA

Cohort A (Transitions):
- PAH and PH-ILD
- Stable on Tyvaso

Cohort B (Naïve):
- PAH
- Naïve to prostacyclin

Screening 28 days	48-week TREATMENT PERIOD L606 + Vibrating mesh nebulizer				
Week	Week 2,4,8	Week 12	Week 24	Week 36	Week 48
Endpoints	●	●Δ	●Δ	●Δ	●Δ

● PRIMARY SAFETY ENDPOINTS
- **Incidence of treatment-emergent drug- or device-related AEs and serious AEs**

Δ EXPLORATORY ENDPOINTS
- **Change in peak and trough 6MWD**
- **Treatment Satisfaction Questionnaire for Medication (TSQM)** transition patients only

clinicaltrials.gov: NCT04691154

RESULTS

- 23 patients were in the transition group (18 PAH: 5 PH-ILD), with 5 in the naïve group (PAH). 24 (85.7%) patients completed 48 weeks' treatment. Three discontinued due to TEAE
- The majority patients were female (75%), white (78.6%) with a mean age of 61 years. At baseline, 55.6% were NYHA FC II; 44.4% FC III with a median 6MWD of 395m. In the transition group, 17/18 (94%) were on triple combination treatment. In the naïve group, 2/5 (40%) were on dual treatment (**Table 1**)
- L606 median dose at 48 weeks was 229 mcg BID, a daily dose comparable to at least 19 breaths QID of inhaled Tyvaso (**Figure 2**)
- Overall, 25 (89.3%) patients experienced at least 1 TEAE. Ten (35.7%) experienced treatment-related TEAE. There were no deaths. There were no related SAE. The most common related TEAE was cough (all mild) in 14.3% of patients (**Table 2**)
- Following 48-weeks of treatment with L606, there was a mean (SD) change from baseline of +29.4 m (64.3) with median change from baseline of +22.5 m in the peak 6MWD (**Figure 3**)
- 30% of patients with PAH improved 6MWD by 30m or more (**Figure 4)**
- 3 of 4 patients with PH-ILD improved 6MWD by 60m or more (**Figure 4)**

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Table 1. Baseline Demographics and Clinical Characteristics

		Cohort A Tyvaso Transition			Cohort B PCY Naïve	Overall
		PAH (N=18)	PH-ILD (N=5)	Total (N=23)	PAH (N=5)	(N=28)
Age, years median (min, max)		64.5 (21, 75)	66 (56, 74)	65 (21, 75)	57 (29, 60)	61 (21, 75)
Female, n (%)		11 (88.9)	5 (100)	16 (69.6)	5 (100)	21 (75.0)
White, n (%)		15 (83.3)	4 (80.0)	19 (82.6)	3 (60.0)	22 (78.6)
BMI, kg/m^2 median (min, max)		27.3 (22.3, 39.7)	32.6 (27.3, 36.8)	28.3 (22.3, 39.7)	26.1 (21.7, 33.6)	27.4 (21.7, 39.7)
Duration of Dx, yr median (min, max)		7.8 (0.6, 21.2)	3.9 (1.2, 9.9)	6.95 (0.6, 21.2)	1.05 (0.4, 15.5)	6.57 (0.4, 21.2)
No. PAH Rx, n (%)	1	-	1 (20.0)	1 (4.3)	3 (60.0)	4 (14.3)
	2	1 (5.6)	4 (80.0)*	5 (21.7)	2 (40.0)	7 (25.0)
	3	17 (94.4)	-	17 (73.9)	-	17 (60.7)
NYHA Class n (%)	II	11 (61.1)	2 (40.0)	13 (56.5)	2 (50.0)	15 (55.6)
	III	7 (38.9)	3 (60.0)	10 (43.5)	2 (50.0)	12 (44.4)

*PH-ILD Patients on 2 PAH medicines included inhaled treprostinil & PDE5
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Table 2. Safety Outcomes

	Cohort A Tyvaso Transition			Cohort B PCY Naïve	Overall
	PAH (N=18) n (%)	PH-ILD (N=5) n (%)	Total (N=23) n (%)	PAH (N=5) n (%)	(N=28) n (%)
Any TEAE	15 (83.3)	5 (100)	20 (87.0)	5 (100)	25 (89.3)
Treatment Related TEAE	5 (33.3)	2 (40.0)	8 (34.8)	2 (40.0)	10 (35.7)
Serious TEAE (SAE)	3 (16.7)	2 (40.0)	5 (21.7)	1 (20.0)	6 (21.4)
Treatment Related SAE	-	-	-	-	-
Treatment related TEAE Led to Dose Reduction	1 (5.6)		1 (4.3)	-	1 (3.6)
Treatment Discontinuation	1 (5.6)	1 (20.0)	2 (8.7)	1 (20.0)	3 (10.7)
Death	-	-	-	-	-

Overall	
Treatment-Related Cough Severity	**(N=28) n (%)**
Mild	4 (14.3)
Moderate	-
Leading to DC	-

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Figure 2. Majority of Patients Titrated to Doses Comparable to >19 breaths per session (bps) Tyvaso QID

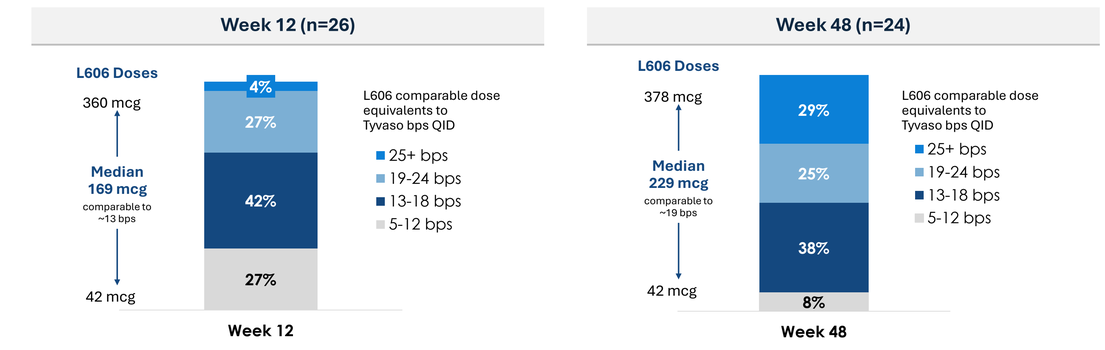

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Figure 3. Minimal Variability Between Peak and Trough 6MWD

Median change from baseline in 6MWD

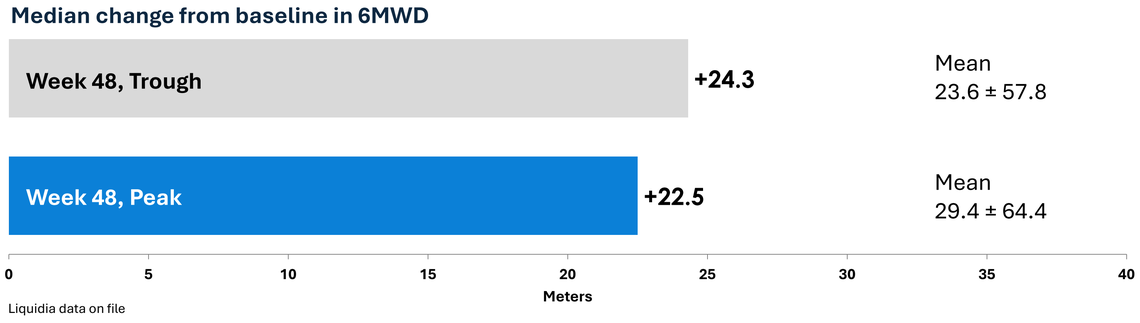

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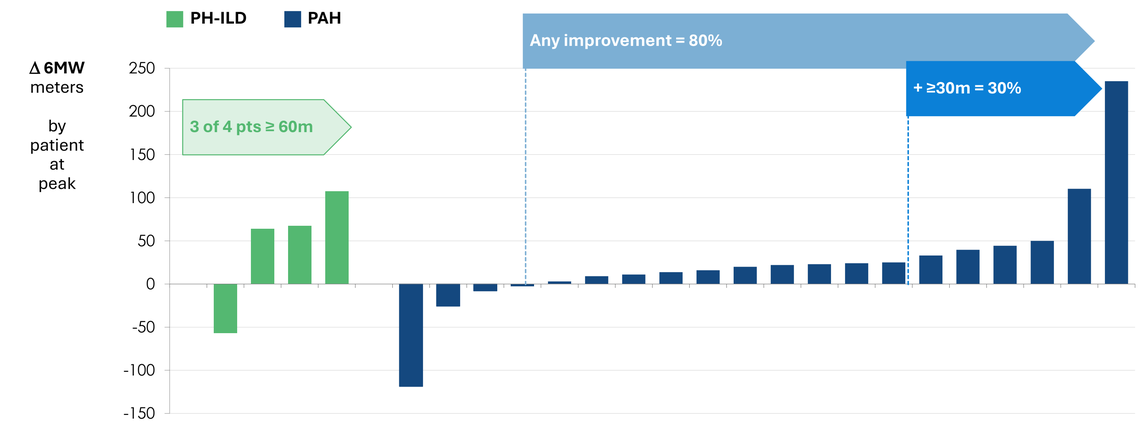
Figure 4. 6MWD Improvement Week 48



PAH patient (-118m) had knee surgery prior to conducting the 6MWD at Week 48; Liquidia data on file

SUMMARY

- **Inhaled L606 was safe and well tolerated in both patients transitioning from inhaled Tyvaso® or naïve to prior prostacyclin therapy over 48 weeks**
- **Most TEAEs were mild. Cough (14.3%) was the most common drug-related TEAE**
- **There was a mean improvement of 29.4m in peak 6MWD**
- **30% of PAH patients improved peak 6MWD by 30m or more**
- **3 of 4 patients with PH-ILD improved peak 6MWD by 60m or more**

ABBREVIATIONS:
6MWD, six minute walk distance; AEs, adverse events; BMI, body mass index bps, breaths per session; DC, discontinuation; Dx, diagnosis; FVC, forced vital capacity; FEV, forced expiratory volume; mcg, microgram; PAH, pulmonary arterial hypertension; PH-ILD, pulmonary hypertension associated with interstitial lung disease; Rx, doctor's prescription; TEAE, treatment emergent adverse event; TSQM, treatment satisfaction questionnaire for medication; NT-proBNP, N-terminal pro B-type natriuretic peptide; NYHA, New York Heart Association; QID, four times a day.

REFERENCES
Liquidia data on file.

L606 is an investigational product.
Safety and efficacy have not been established.

Presented at PVRi 2026 | Jan 28 – Feb 1, 2026 | Dublin, Ireland