

# **Corporate Overview**

J.P. Morgan Healthcare Conference 2025

January 15, 2025

# Forward-looking statements

This presentation includes, and our response to questions may include, forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995 ("PSLRA"). All statements contained in this presentation 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 U.S. Food and Drug Administration approval of the New Drug Application for YUTREPIA, which may occur after the expiration of the exclusivity period of TYVASO DPI, if at all, the timelines or outcomes related to patent litigation with United Therapeutics in the U.S. District Court for the District of Delaware, litigation with United Therapeutics and FDA in the U.S. District Court for the District of Columbia or other litigation instituted by United Therapeutics or others, including rehearings or appeals of decisions in any such proceedings, the issuance of patents by the United States Patent and Trademark Office 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 decision 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 U.S. Securities and Exchange Commission 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 presentation may not occur and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements. Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee that future results, levels of activity, performance, achievements or events and circumstances reflected in the forward-looking statements will occur. We are under no duty to update any of these forward-looking statements after the date of this presentation to conform these statements to actual results or revised expectations, except as required by law. This presentation also contains estimates and other statistical data made by independent parties and by us relating to market size and growth and other data about our industry. This data involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. In addition, projections, assumptions and estimates of our future performance and the future performance of the markets in which we operate are necessarily subject to a high degree of uncertainty and risk. This presentation includes long-term goals that are forwardlooking, are subject to significant business, economic, regulatory and competitive uncertainties and contingencies, many of which are beyond our control and are based upon assumptions with respect to future decisions, which are subject to change. Actual results will vary, and those variations may be material. Nothing in this presentation should be regarded as a representation by any person that these goals will be achieved. We have no obligation under the PSLRA to update any forward-looking statements, 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.



# Enhancing drug delivery to the lungs to make every breath count



Liquidia Corporation is a biopharmaceutical company dedicated to the development & commercialization of **best-in-class therapies** with potential to improve standard of care for patients with **pulmonary hypertension**.

- > Improving drug delivery
- > Using proprietary technologies
- > Reducing burden of administration
- > Helping patients breathe easier & live longer



# Liquidia positioned for rapid growth in 2025 and beyond

## **Product**

## YUTREPIA™ (treprostinil) inhalation dry powder

- Tentative approval for PAH & PH-ILD
- Seeking final FDA approval in 1H'25
- Differentiated product profile

## **Pipeline**

## L606 (treprostinil) inhaled liposomal suspension

- Sustained release formulation
- Only 2x daily with 24-hour exposure
- Rapid, portable, next-gen nebulizer

# Advanced pipeline of inhaled treprostinil products to treat PAH & PH-ILD

## **Platform**

## PRINT® Technology

- Precise, uniform drug particles
- Enhance delivery to deep lung

Pulmonary arterial hypertension (PAH), Pulmonary hypertension associated with interstitial lung disease (PH-ILD) YUTREPIA™ and PRINT® are trademarks of Liquidia Technologies, Inc.



# PAH and PH-ILD present attractive market opportunities

Clear opportunity for better product profiles to capture market share

	WHO Group 1 (PAH)	WHO Group 3 (PH-ILD)	
Market dynamic	Established market (since 1995)	Emerging, rapidly growing (since 2021)	
Addressable patients	~100,000 prevalent ~45,000 treated	~60,000 prevalent ~27,000 addressable (if 45% like PAH)	
Prostacyclin patients 2024 estimate	<ul> <li>~18,000 treated with prostacyclin</li> <li>Oral 10,000</li> <li>IV&amp;SC 4,000</li> <li>DPI 2,500</li> <li>Nebulized 1,500</li> </ul>	<ul> <li>~6,000 treated in first 3.5 years</li> <li>DPI 4,000</li> <li>Nebulized 2,000</li> </ul> Inhaled treprostinil is only FDA approved MOA/route	
	~6,000 annual new starts ~30% discontinue or change PGI2	21,000+ untreated patients without including any switches	

Intravenous (IV), Subcutaneous (SC), Mechanism of Action (MOA), Prostacyclin (PGI2)
Liquidia analysis informed by statements and reports from competitors, data from specialty pharmacies, third-party market research



# PAH and PH-ILD are large markets with unmet needs

Inhaled treprostinil is the only product approved to treat both groups

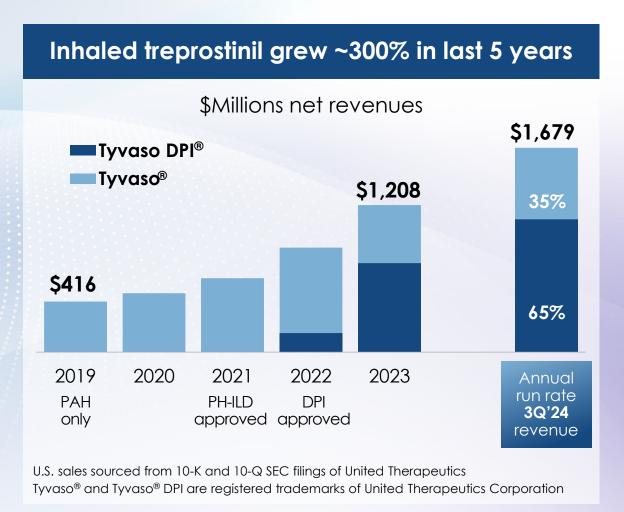
\$1.685

# Prostacyclin market is \$3.3B and growing \$Billions net sales U.S. 2023 **Treprostinil** Inhaled, represented \$1.208 Oral, **\$2.0B (59%)** of

IV & SC.

\$0.415







prostacyclin

market

# Ideal product profile

Therapeutic goal is to optimize each aspect

# Targeted lung delivery



Reduces off-target toxicity from oral, IV/SC delivery

# **Portable**



Convenience & ease-of-use to support compliance

## **Titratable**



Customizable per patient and not dose limited

# **Durable**



Wide dose range to extend time on treatment

# Dosing frequency

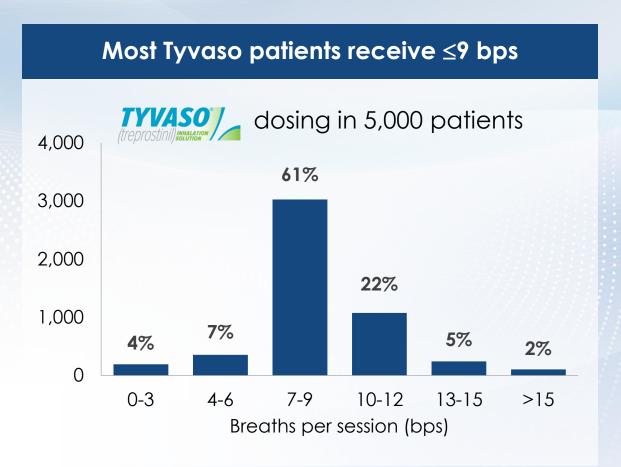


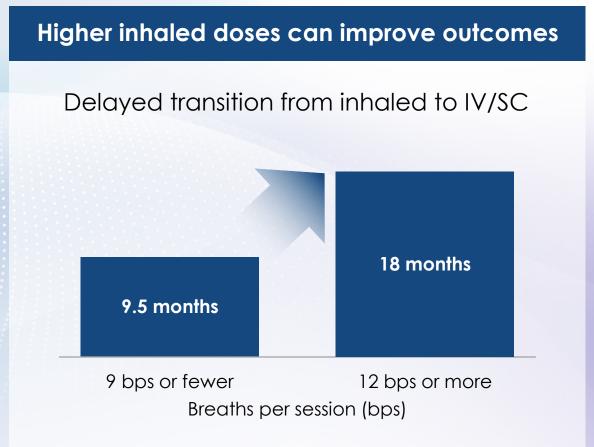
Easy and simple regimen



# In PAH, Tyvaso dose range has been limited despite clear benefits

Tyvaso is nebulized 4x daily and titrates dose by increasing breaths per session





Shapiro et al, Pulmonary Circulation 2021; 11(4) 1–7, which described a retrospective analysis of 5,000 PAH patients using Tyvaso to treat PAH from 2009–2018



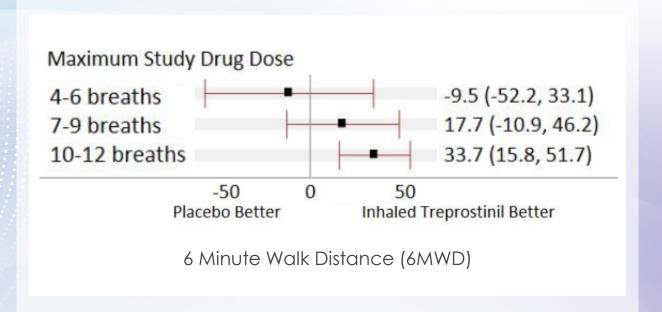
# In PH-ILD, higher inhaled doses correlated to better patient response

**INCREASE trial of Tyvaso for PH-ILD** 

## Doses >9 breaths led to clinical improvement

# TYVASO | 17.3% | 17.3% | 17.3% | 17.3% | 17.3% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 17.3% | 17.3% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4% | 16.4

## Doses >9 breaths led to better peak 6MWD at wk 16



1. Nathan et al, CHEST Journal, February 2023, Vol. 163, Issue 2, P398-406; 2. Supplement to: Waxman et al, N Engl J Med 2021;384:325-34



# Real-world challenges with Tyvaso DPI in PH-ILD

# Prostacyclin naïve patients showed worse tolerability and discontinued faster

#### **Methods**

- We prospectively gathered data on patients with PH-ILD who we initiated on treprostinil DPI (either naively or transition from inhaled treprostinil) to analyze safety and tolerability: BNP, 6-minute walk data, spirometry with DLCO, and RVSP and TAPSE on echocardiogram.
- Following transition, we recorded data obtained through routine standard-of-care testing at our center to ensure safety and tolerability in this patient population. (Table 2)
- This study was approved by the IRB at National Jewish Health.





Table 2: Discontinuation Rates of Tyvaso DPI

#### Naïve DPI Discontinuation Rate

26 started, 18 discontinued

11 transitioned to nebulizer 7 discontinued treatment completely

#### 69% discontinuation rate

Discontinued due to cough (5), Hypotension (1), Clinical Worsening (9), Self-discontinuation (2), Death (1)

#### **Transition DPI Discontinuation Rate**

22 started, 11 discontinued

7 transitioned back to nebulizer 5 discontinued treatment completely

#### 50% discontinuation rate

Discontinued due to cough (1), Hypotension (1), Clinical Worsening (6), Lung Transplant (2), Death (1)

#### Results

- 22 patients with PH-ILD were transitioned from the nebulized treprostinil to the treprostinil DPI at equivalent doses between July 2022 and April 2023. The most common form of ILD was CTD-associated ILD. (Table 1)
  - 50% of whom discontinued the treprostinil DPI
     Average treatment duration on DPI prior to discontinuation was a mean of 195 days, median 223 days (min 13 max 358)
- 26 patients with Pri-iLD started the treprosunil DPI naively, titrating from 16 mcg to 64 mcg between July 2022 and April 2023.
  - 69% of whom discontinued the treprostinil DPI
  - Average treatment duration on DPI prior to discontinuation was a mean of 78 days prior to stopping, median 40 days (min 4 –max 171)
- The most common reasons for discontinuation were clinical worsening and cough
  - Clinical worsening was defined by our group as at least having one of the following: worsening testing upon follow up (PFT, Echo, 6MWT), reported increased shortness of breath, and/or increased oxygen requirement.

Rice et al, Tolerability and Efficacy of Treprostinil Dry-Powdered Inhaler in Patients with Pulmonary Hypertension Related to Fibrosing Interstitial Lung Disease at a Large Tertiary Referral Center, 2023 Pulmonary Hypertension Professional Network Symposium, September 28-30, 2023 [Poster]



# YUTREPIA has potential to become the Prostacyclin of First Choice

# Targeted lung delivery



Enhanced local delivery to the deep lung

using PRINT® Technology

## **Portable**



Low-effort, proven DPI

commonly
used by
patients with
impaired lung
function

## **Titratable**



Safely dosed ~3x higher

when compared to target dose of competitor

## **Durable**



Broad dose range

offers potential to prolong inhaled treatment

# Dosing frequency



Same frequency

4x daily like Tyvaso & Tyvaso DPI



# PRINT® particles are designed to enhance deep-lung delivery

Uniform size & shape in deagglomerated powder

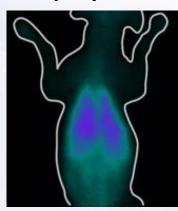


Particle size influences lung deposition within the respirable range (≤ 5 µm)

4.6 µm particle



1.3 µm particle



Tc<sup>99</sup> scintigraphy of MMAD of PRINT particles in canine model<sup>1</sup> Median Mass Aerodynamic Diameter (MMAD)

1. Garcia et al, Microfabricated engineered particle systems for respiratory drug delivery and other pharmaceutical applications. J Drug Deliv. 2012;2012:941243

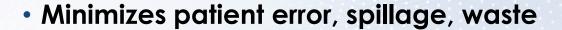


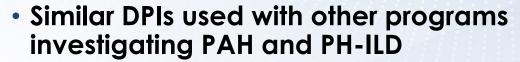
# PRINT® enables use of trusted, low-effort dry-powder inhaler (DPI)



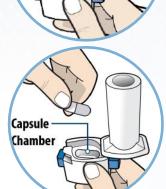
- Used for decades to treat asthma, COPD and other respiratory diseases
- Minimal inspiratory effort due to low resistance







- Seralutinib (Gossamer)<sup>1</sup>
- TPIP (INSMED)<sup>2</sup>

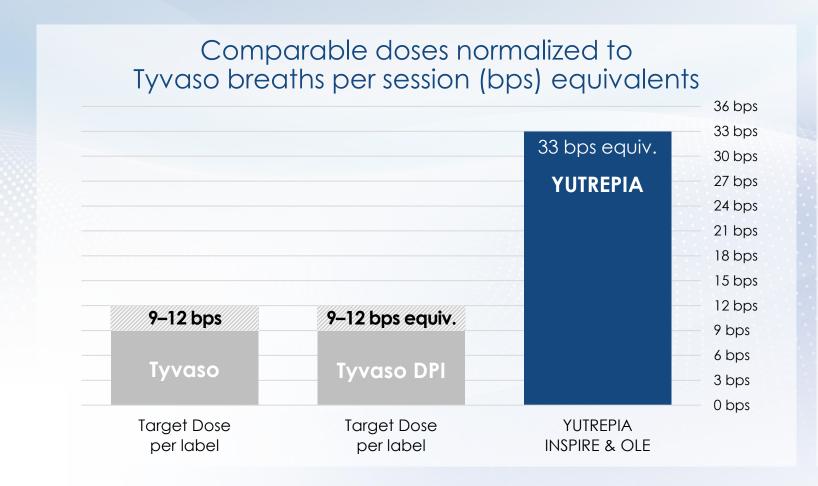




1. Faria-Urbina et al, Pulm. Circ. 2021; 11(2) 1–10; 2. Gauani, H. et al, Front. Drug Deliv., 05 April 2022, Sec. Respiratory Drug Delivery, Vol 2 - 2022



# In PAH, YUTREPIA was tolerated at doses 3x higher than targeted maintenance dose of Tyvaso & Tyvaso DPI



3x higher comparable dose

INSPIRE & OLE study (PAH)

- n=66 patients naïve to prostacyclin
- n=55 patients Tyvaso transitions
- Supported tentative FDA approval for PAH and PH-ILD

Breaths per session (bps), Open Label Extension (OLE), studies listed in clinicaltrials.gov: INSPIRE (NCT03399604), OLE (NCT03992755)



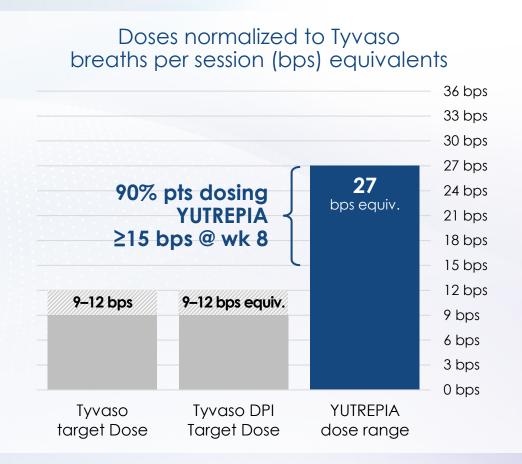
# In PH-ILD, YUTREPIA is taking titration to a whole new level

# ASCENT study data n= first 20 treated for 8 weeks

Patients treated through week 8				
Patients	n=20			
ILD type	<ul><li>IIPs</li><li>Autoimmune ILD</li><li>CPFE</li></ul>	12 (60%) 5 (25%) 3 (15%)		
% FVC	72.5% ± 22.92			
% DLCO	40.1% ± 16.66			
mPAP	34.2 ± 8.71 mmHg			
PVR	6.2 ± 2.42 wu			

## **Tolerability**

- No discontinuations through week 8 (day 56)
- Most frequent TEAE is cough, all mild
- No drug-related SAEs



Treated emergent adverse event (TEAE), serious adverse event (SAE), forced vital capacity (FVC), diffusing capacity of the lungs for carbon monoxide (DLCO), mean pulmonary arterial pressure (mPAP), pulmonary vascular resistance (PVR); Study listed in clinicaltrials.gov: ASCENT (NCT06129240)



# YUTREPIA best (\*)'s the boxes for an ideal product profile

A single solution to treat PAH & PH-ILD		TYVASO (treprostinil) INHALATION (treprostinil) Solution	TYVASO DPI (treprostinil) POWDER	YUTREPIA™ (treprostinil) inhalation powder
Targeted lung delivery	Reducing off-target toxicity from oral, IV/SC	8	8	
Portable	Convenience & ease-of-use to support compliance	<b>(X)</b>	8	8
Titratable	Customizable per patient and not dose limited	<b>(X)</b>		8
Durable	Wide dose range to extend time on treatment	<b>(X)</b>	<b>(X)</b>	8
Dosing frequency	Easy or simple regimen without losing efficacy	8	8	8



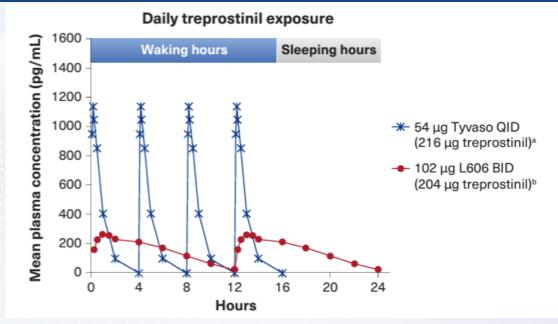
# Advancing L606 sustained release treprostinil

Liposomal formulation using modern nebulizer with DPI-like portability

## Sustained drug exposure over 24 hours

- Less frequent dosing (2x daily)
- Improved tolerability
- Rapid delivery with next-gen, hand-held nebulizer with breath-actuated smart technology
- In on-going, open-label study, PAH patients have safely titrated to doses comparable to 26-28 breaths per session of Tyvaso 4x daily

## ~7x lower Cmax with AUC being similar to Tyvaso



<sup>a</sup>Tyvaso data over 4 hours are from the comparative bioavailability study and the remaining data are simulated to show Tyvaso 24-hour exposure.

<sup>b</sup>L606 data over 12 hours are from the comparative bioavailability study and the remaining data are simulated to show L606 24-hour exposure.

Maximum peak concentration (Cmax), Area Under the Curve (AUC) Liquidia data on file from study NCT04691154

Tully, J. et al. PVRI 2024 Annual Congress.



# Seeking PAH & PH-ILD indication for L606 with one pivotal trial

Three data sets required per FDA and EMA feedback



Comparable bioavailability to Tyvaso® in Phase 11



**Open-label safety study** in U.S. of PAH & PH-ILD<sup>2</sup>



Randomized placebo-controlled for efficacy in PH-ILD

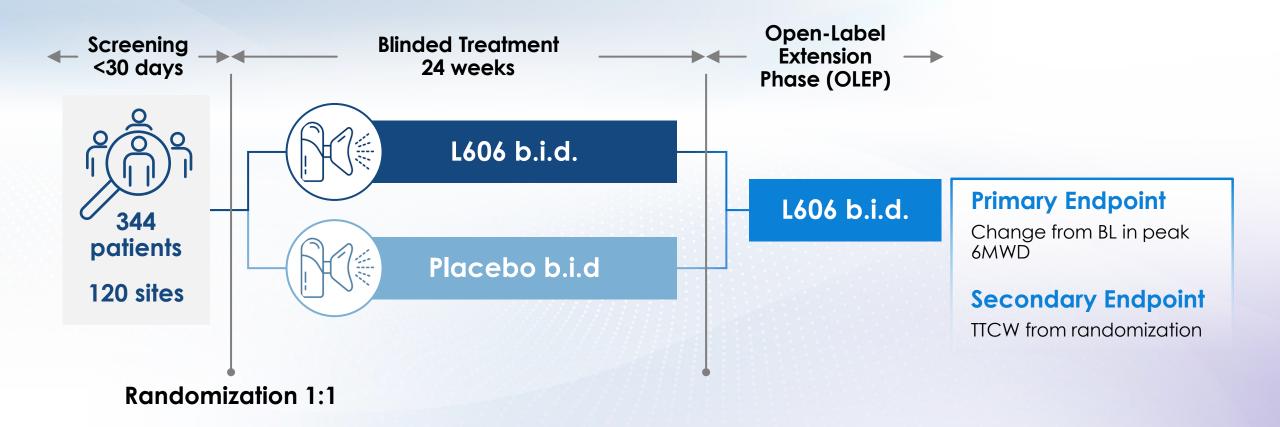


1. Phase 1 study NCT04041648; 2. Open label PAH & PH-ILD study NCT04691154



# Phase 3, multi-center, randomized (1:1), double-blind, placebo-controlled, parallel group study





Baseline (BL), Six Meter Walk Distance (6MWD), Time to Clinical Worsening (TTCW)



# Will seek final FDA approval of YUTREPIA in first half 2025

No existing legal barriers are blocking

# Legal decisions favorable



- Liquidia does not infringe any valid claims of three originally asserted patents
- Rulings final and not subject to further appeal

# **Exclusivity** expiring



- Regulatory exclusivity awarded to Tyvaso DPI expires May 23, 2025
- May resolve earlier if Liquidia successful in challenging FDA's award

# Injunctions rejected



- UTHR denied PI in original suit vs. FDA alleging YUTREPIA NDA violated FDA's policy; after dropping suit, UTHR refiled claims and awaits court response
- UTHR denied PI in '327 patent infringement suit and trial set Jun 23, 2025

United Therapeutics Corporation (UTHR), Preliminary Injunction (PI)



# Well-capitalized to achieve objectives in 2025

Ended 3Q24 with

\$204M

cash and cash equivalents

 Added \$~100M to balance sheet in September

# Prepared to Launch YUTREPIA at any time

- Sales force and Medical Affairs in place
- Commercial inventory on-hand
- Distribution channel established
- Active in the PH patient community





# Q&A

Dr. Roger Jeffs

Chief Executive Officer

Michael Kaseta

Chief Operating Officer & Chief Financial Officer

**Russell Schundler** 

General Counsel