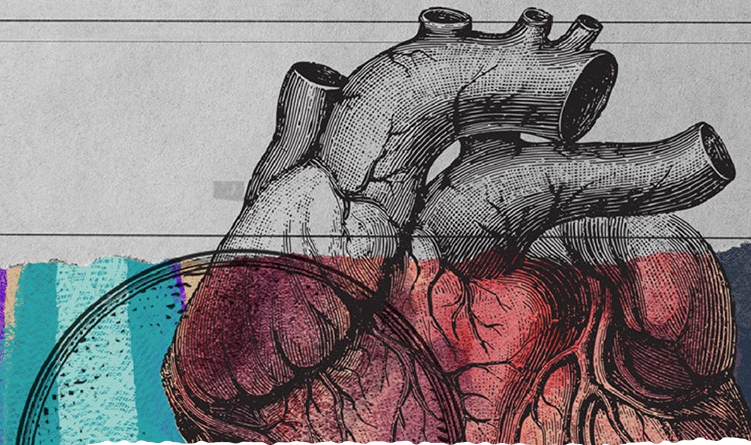




**United  
Therapeutics**

A PUBLIC BENEFIT CORPORATION



# United Therapeutics Corporation Third Quarter 2024 Corporate Update

October 30, 2024

## INTRODUCTION

# Safe Harbor Statement

**All statements in this presentation** are made as of October 30, 2024. We undertake no obligation to publicly update or revise these statements, whether as a result of new information, future events or otherwise.

**Statements included in this presentation** that are not historical in nature are “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements include, among others, statements related to our revenue growth expectations, the timing and success of our pipeline programs, our planned manufacturing and field force expansions, our organ manufacturing efforts and similar statements concerning anticipated future events and expectations.

**We caution you** that these statements are not guarantees of future performance and are subject to numerous evolving risks and uncertainties that we may not be able to accurately predict or assess, including the risk factors that we describe in our Securities and Exchange Commission filings, including our most recent Annual Report on Form 10-K or Quarterly Report on Form 10-Q. Any of these factors could cause actual results to differ materially from the expectations we express or imply in this presentation.

**This presentation** and any related discussions or statements are intended to educate investors about our company. Sometimes that process includes reporting on the progress and results of clinical trials or other developments with respect to our products. This presentation and any related discussions or statements are not intended to promote our products, to suggest that our products are safe and effective for any use other than what is consistent with their FDA-approved labeling, or to provide all available information regarding the products, their risks, or related clinical trial results. Anyone seeking information regarding the use of one of our products should consult the full prescribing information for the product available on our website at [www.unither.com](http://www.unither.com).



## INTRODUCTION

# Today's Speakers



**Dr. Martine Rothblatt**

Chairperson & Chief Executive Officer



**Michael Benkowitz**

President & Chief Operating Officer



**James Edgemon**

Chief Financial Officer & Treasurer

## INTRODUCTION

# Other Executives Present Today



## Dr. Leigh Peterson

Executive Vice President,  
Product Development &  
Xenotransplantation



## Pat Poisson

Executive Vice President,  
Technical Operations

## INTRODUCTION

# Upcoming Investor Events



## UBS Global Healthcare Conference

November 11-14, 2024



## Oppenheimer Movers in Rare Disease Summit

December 12, 2024



## 43rd Annual J.P. Morgan Healthcare Conference

January 13-16, 2025

## INTRODUCTION

# Upcoming Medical Conferences



## PHenomenal Hope 2024

December 6, 2024



## PVRI 2025 Annual Congress

January 29 - February 1, 2025



# Dr. Martine Rothblatt

CHAIRPERSON & CHIEF EXECUTIVE OFFICER



# 3Q 2024 Performance Summary

Product	Product Revenue	Percent Change <sup>1</sup>
Tyvaso DPI®/ Nebulized Tyvaso®	\$434 M	▲ 33%
Remodulin®	\$128 M	▼ 2%
Orenitram®	\$113 M	▲ 23%
Unituxin®	\$61 M	▲ 19%
Other + Adcirca®	\$13 M	NM <sup>2</sup>
<b>Total Revenue</b>	<b>\$749 M</b>	<b>▲ 23%</b>

**\$1.1 B**

TTM Operating Cash Flow

**\$4.6 B**

Cash, Cash Equivalents, &  
Marketable Investments

**Highest Quarterly Tyvaso®<sup>3</sup>,  
Orenitram®, Unituxin®,  
and Total Revenue**

1. Change vs. 3Q 2023.

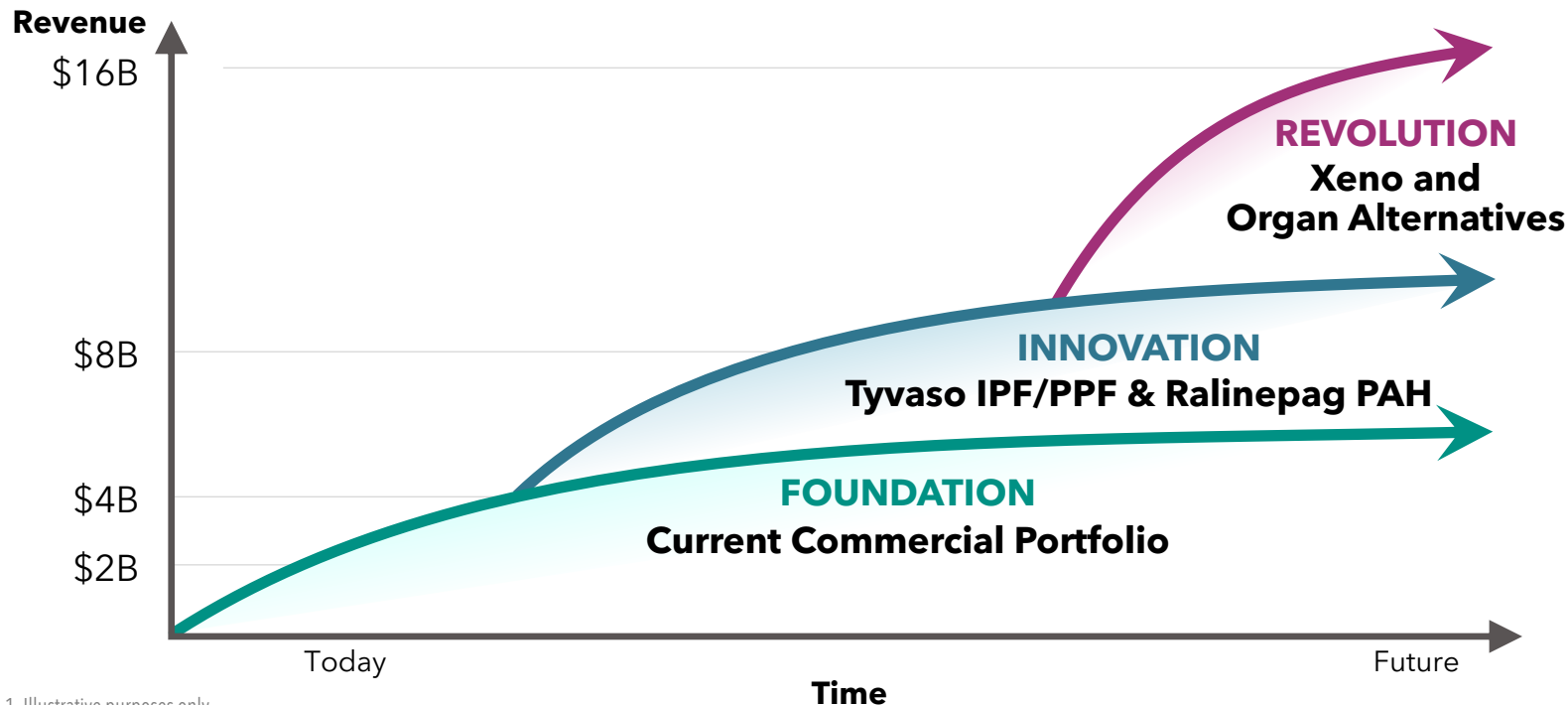
2. Not meaningful.

3. Tyvaso DPI + nebulized Tyvaso



## HOW WE OPERATE

# Positioned for Multiple Waves of Growth<sup>1</sup>



1. Illustrative purposes only.

## INNOVATION

# Development Engine Addressing Unmet Needs

NON-REGISTRATION

REGISTRATION

APPROVED

## Tyvaso®

**TETON 1 - Idiopathic Pulmonary Fibrosis - U.S. and Canada**

**TETON 2 - Idiopathic Pulmonary Fibrosis - ROW<sup>1</sup>**

**TETON PPF - Progressive Pulmonary Fibrosis**

## Ralinepag

**ADVANCE OUTCOMES - PAH<sup>2</sup>**

## Organs and Organ Alternatives

**EVLP<sup>3</sup>/CLES<sup>4</sup> - Lung Transplant**

**miroliverELAP<sup>5</sup> - Acute Liver Failure**

## Pre-clinical Xeno and Organ Alternative Programs

UKidney™

UHeart™

ULung™

miroliver®

UTHymoKidney™

ULobe™

IVIVA Kidney

mirokidney®

1. ROW = rest of world outside the U.S. and Canada. 2. PAH = pulmonary arterial hypertension. 3. EVLP = ex-vivo lung perfusion. 4. CLES = centralized lung evaluation system.  
5. ELAP = external liver assist product.

## INNOVATION

# Tyvaso® *TETON 1* and *2* Studies

	<i>TETON 1</i>	<i>TETON 2</i>
Indication	Idiopathic pulmonary fibrosis	
U.S. Addressable Population	100,000 patients	
Study Size	576	597 <sup>5</sup>
Study Geography	U.S./Canada	ROW <sup>1</sup>
Primary Endpoint	Change in absolute FVC <sup>2</sup> from baseline to week 52	
Enrollment Progress <sup>3</sup>	~90%	100%

1. ROW = rest of world outside the United States and Canada. 2. FVC = forced vital capacity, or the amount of air that can be forcibly exhaled from your lungs after taking the deepest breath possible. 3. As of October 14, 2024. 4. Enrollment target is our current target for full enrollment of the study at the time of this presentation. Our timing expectations may change. 5. *TETON 2* targeted 576 patients for full enrollment and ultimately enrolled 597 patients.

**Complete Enrollment for  
*TETON 1* Targeted  
for Year-End 2024<sup>4</sup>**

***TETON 2* Fully Enrolled**

## INNOVATION

**Tyvaso<sup>®</sup> TETON PPF Study**

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Indication	Progressive pulmonary fibrosis
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U.S. Addressable Population	60,000 patients <sup>1,2,3</sup>
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Study Size	698 patients
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Study Geography	Global
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Primary Endpoint	Change in absolute FVC <sup>4</sup> from baseline to week 52
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Enrollment Progress	Currently enrolling
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**Currently Enrolling  
Patients**

1. Am J. Respir Crit Care Med, vol 150. pp 967-972, 1994. 2. Adv Ther (2021) 38:4100-4114. 3. Cottin V, Teague R, Nicholson L, Langham S and Baldwin M (2022) The Burden of Progressive-Fibrosing Interstitial Lung Diseases. Front. Med. 9:799912. doi: 10.3389/fmed.2022.799912. 4. FVC = forced vital capacity, or the amount of air that can be forcibly exhaled from your lungs after taking the deepest breath possible.

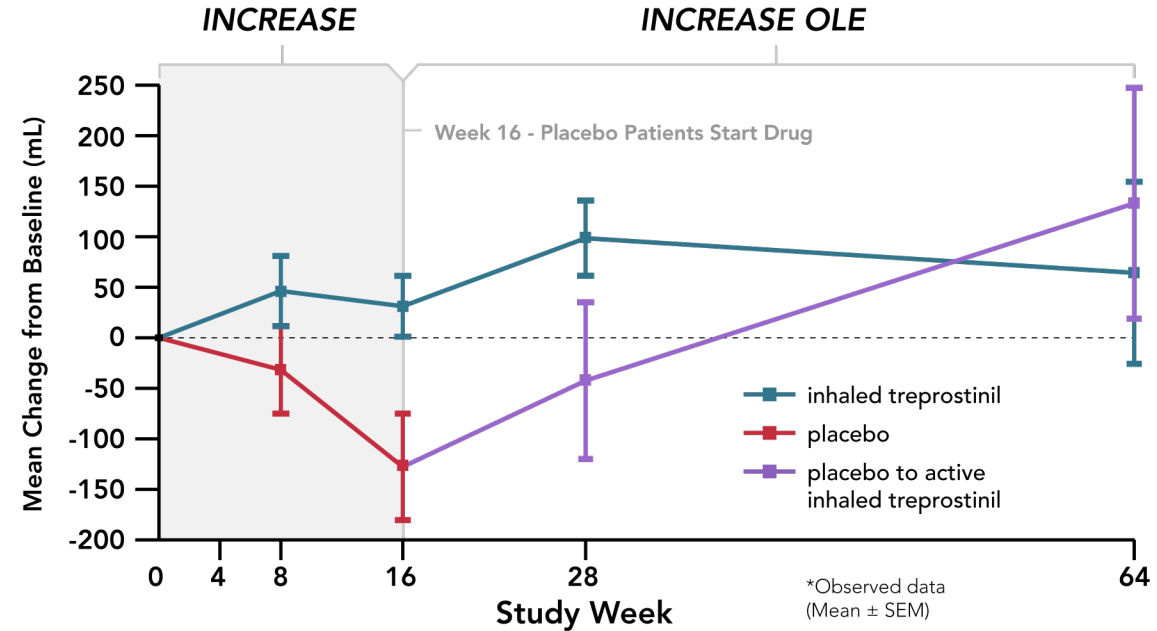
## INNOVATION

# Tyvaso for IPF<sup>1,2</sup>

The *TETON* studies evolved from UT-sponsored in vitro studies and FVC<sup>3</sup> observations in *INCREASE*<sup>4</sup> and *INCREASE OLE*<sup>5</sup>

IPF subgroup showed meaningful and sustained FVC improvement, including when placebo patients were crossed over in the open-label extension

## MEANINGFUL, SUSTAINED FVC IMPROVEMENT



1. IPF = idiopathic pulmonary fibrosis. 2. Tyvaso is not approved to treat IPF. 3. FVC = forced vital capacity. 4. N Engl J Med 2021; 384:325-334 DOI: 10.1056/NEJMoa2008470.

5. The Lancet Respiratory Medicine, Volume 9, Issue 11, 1266 - 1274 DOI: 10.1016/S2213-2600(21)00165-X

## INNOVATION

# Ralinepag

## ADVANCE OUTCOMES Study

Indication	Group 1 PAH <sup>1</sup>
U.S. Addressable Population	50,000 patients
Study Size	~700 to 1,000 patients
Study Geography	Global
Primary Endpoint	Time from randomization to the first adjudicated protocol-defined clinical worsening event
Enrollment Progress <sup>2</sup>	~625 patients

1. PAH = pulmonary arterial hypertension. 2. As of October 14, 2024. 3. We plan to close enrollment in mid-2025, and accrue clinical worsening events through the end of 2025, data is expected to be available in 2026. Our timing estimates may change. 4. [https://posters.unithermedaffairs.com/ralinepag\\_XRIR\\_ISHLT2019.pdf](https://posters.unithermedaffairs.com/ralinepag_XRIR_ISHLT2019.pdf).

**Data expected in 2026<sup>3</sup>**

**One pill, once a day, with a ~24-hour half-life that can approximate IV prostacyclin blood levels<sup>4</sup>**

## INNOVATION

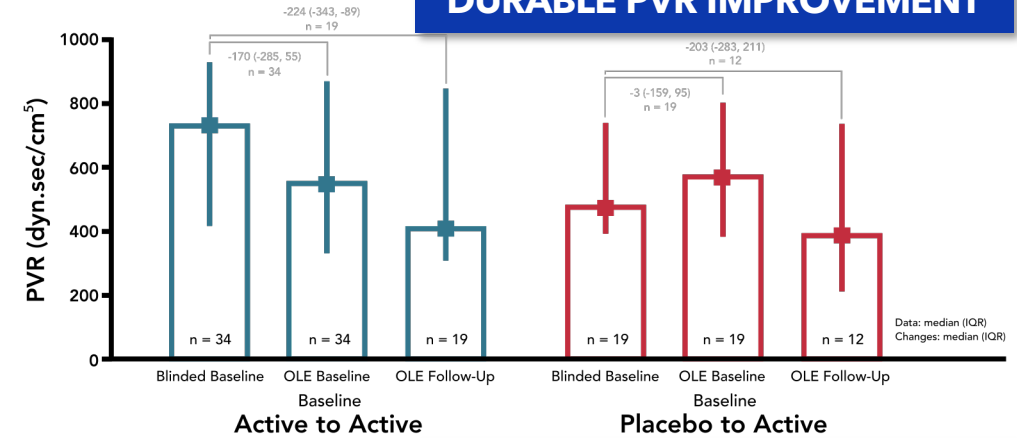
# Ralinepag for PAH<sup>1,2</sup>

Phase 2 OLE<sup>3</sup> data demonstrate long-term treatment with ralinepag produces durable and clinically-relevant responses for PVR<sup>4</sup> and 6MWD<sup>6</sup> with a manageable adverse event profile<sup>7</sup>

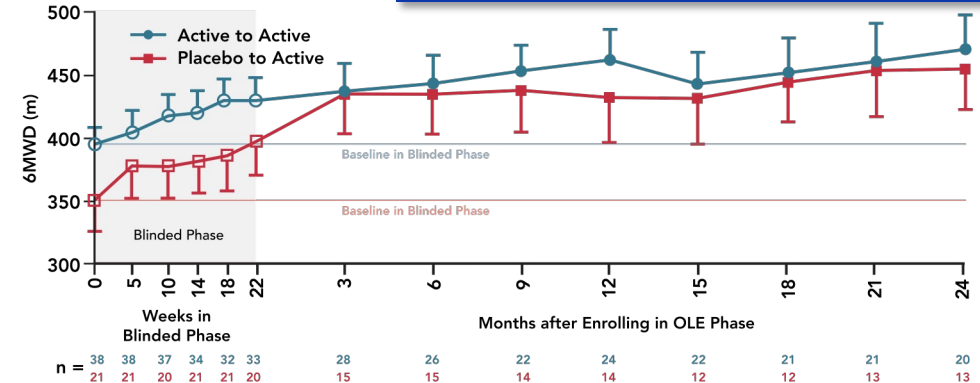
In 24-month open-label data, a 52 dyn.s/cm<sup>5</sup> reduction in PVR and a 36m 6MWD increase was observed on top of improvements from the blinded phase of the study.

1. PAH = pulmonary arterial hypertension. 2. Ralinepag is an investigational drug and is not approved to treat PAH. 3. OLE = open label extension. 4. PVR = pulmonary vascular resistance. 6. 6MWD = six-minute walk distance. 7. Barberà, et al. Ralinepag Phase II Open-Label Extension Study in Patients with Pulmonary Arterial Hypertension. *J. Adv Ther.* 2023. <https://doi.org/10.1007/s12325-023-02769-7>.

## DURABLE PVR IMPROVEMENT



## SUSTAINED 6MWD INCREASE



## INNOVATION

# Ex-Vivo Lung Perfusion

- More than 830 lung evaluations, resulting in over **500 successful transplants**<sup>1</sup>
- Lungs transplanted through EVLP<sup>2</sup> would have **otherwise been discarded**
- Two methods used:
  - **XPS**<sup>3</sup>: Third-party supplied EVLP device
  - **CLES**<sup>4</sup>: Internally-developed device with potentially broader applications
- CLES PMA<sup>5</sup> submitted in September; FDA decision expected in 2025.



**CLES PMA SUBMITTED**

1. As of October 15, 2024. 2. EVLP = ex-vivo lung perfusion. 3. XPS = XVIVO Perfusion System.

4. CLES = centralized lung evaluation system. 5. PMA = premarket application.



REVOLUTION

# Four Platforms with Four Organs and Organ Alternatives; Multiple Shots on Goal

1. 3DAP = 3D autologous printed.
2. Bio-Art = Bioartificial.
3. ELAP = external liver assist product.

	Xeno	Regen Med	3DAP <sup>1</sup>	Bio-Art <sup>2</sup>
Kidney	UKidney™ UThymoKidney™			IVIVA Kidney mirokidney®
Heart	UHeart™			
Lung		ULobe™	ULung™	
Liver				miroliverELAP® <sup>3</sup> / miroliver®

## REVOLUTION

# miroliverELAP®: the First Bioengineered Organ Alternative to Enter Human Clinical Trials

- External liver assist product (**ELAP**) intended to provide liver support in the critical care setting
- **Acute liver failure** is a devastating condition with no approved drug or medical device interventions: 30% of adults die and 25% receive liver transplants
- ELAP is intended to give the native liver an opportunity to heal itself, possibly **reducing the need for transplant**



**PHASE 1 STUDY UNDER WAY**

# Xenotransplants March Toward the Clinic

## IND EXPECTED SHORTLY

**Pre-IND<sup>1</sup> feedback received; expect to file an IND shortly for UKidney™ 10-gene edited xenokidney**

1. IND = investigational new drug application. 2. DPF = designated pathogen-free facility.

## DPF<sup>2</sup> OPERATIONS

### **Christiansburg, VA: Operational**

125 organ annual capacity; \$75 million investment

### **Stewartville, MN: Under construction**

125 organ annual capacity; \$110 million investment

**At least one more clinical-scale DPF planned**



# Xenotransplants March Toward the Clinic

## XENO REVIEW MANUSCRIPT

**First** comprehensive review publication on xenotransplantation

Details collective efforts of scientists and physicians to advance xenotransplantation into the clinic

Available in *Physiological Reviews*:

<https://doi.org/10.1152/physrev.00041.2023>

## 10-GENE PRECLINICAL MANUSCRIPT

**First** documented use of standard, FDA-approved, immunosuppression with long-term survival in preclinical xenotransplantation

Details on six preclinical 10-gene UKidney™ transplants to support a future clinical IND<sup>1</sup> application

Available in *Nature Communications*:

<https://doi.org/10.1038/s41467-024-47679-6>

1. IND = investigational new drug application.

**HUMAN CLINICAL STUDIES EXPECTED TO COMMENCE IN 2025**

## FOUNDATION

**Tyvaso DPI®**  
**Nebulized Tyvaso®**  
**Orenitram®**  
**Remodulin®**  
**Unituxin®**

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**PAH<sup>1</sup>**  
**PH-ILD<sup>2</sup>**

## INNOVATION

**Tyvaso DPI**  
**Nebulized Tyvaso**  
**Ralinepag**  
**EVLP<sup>5</sup>**

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**PAH**  
**PH-ILD**  
**IPF<sup>3</sup>**  
**PPF<sup>4</sup>**  
**LUNG TRANSPLANT**

## REVOLUTION

**Xenotransplantation**  
**Regenerative Medicine**  
**3D Organ Alternative**  
**Printing**  
**Bio-Artificial Organ**  
**Alternatives**

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**XENO AND**  
**ORGAN ALTERNATIVES**

# James Edgemond

CHIEF FINANCIAL OFFICER & TREASURER



## CAPITAL ALLOCATION

# Commitment to Balanced Capital Allocation; Accelerated Share Repurchase Complete

PRIORITY 1  
**COMMERCIAL/R&D  
INVESTMENT**

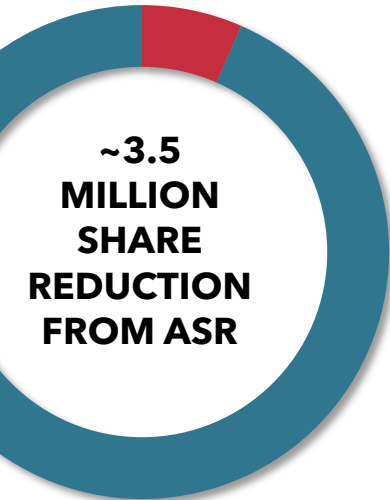
PRIORITY 2  
**CORPORATE  
DEVELOPMENT**

PRIORITY 3  
**RETURN OF  
CAPITAL**

\$1 billion ASR<sup>1</sup> completed in 3Q,  
reducing outstanding UTHR shares  
by approximately 7%<sup>2</sup>

Multi-tranche program resulted in  
retiring additional shares and  
reducing average share repurchase  
price

**ASR demonstrated belief in our  
core business and cash flow  
potential**



1. ASR = accelerated share repurchase program. 2. Based on basic share count when ASR was initiated on 3/25/2024.





# Michael Benkowitz

PRESIDENT & CHIEF OPERATING OFFICER





## COMMERCIAL EXECUTION

# Continued Strong Revenue Growth in 3Q/24

## Tyvaso<sup>3</sup>, worldwide

▲ 33% y/y<sup>1</sup> to \$434M

## Remodulin, worldwide

▼ 2% y/y to \$128M

## Orenitram

▲ 23% y/y to \$113M

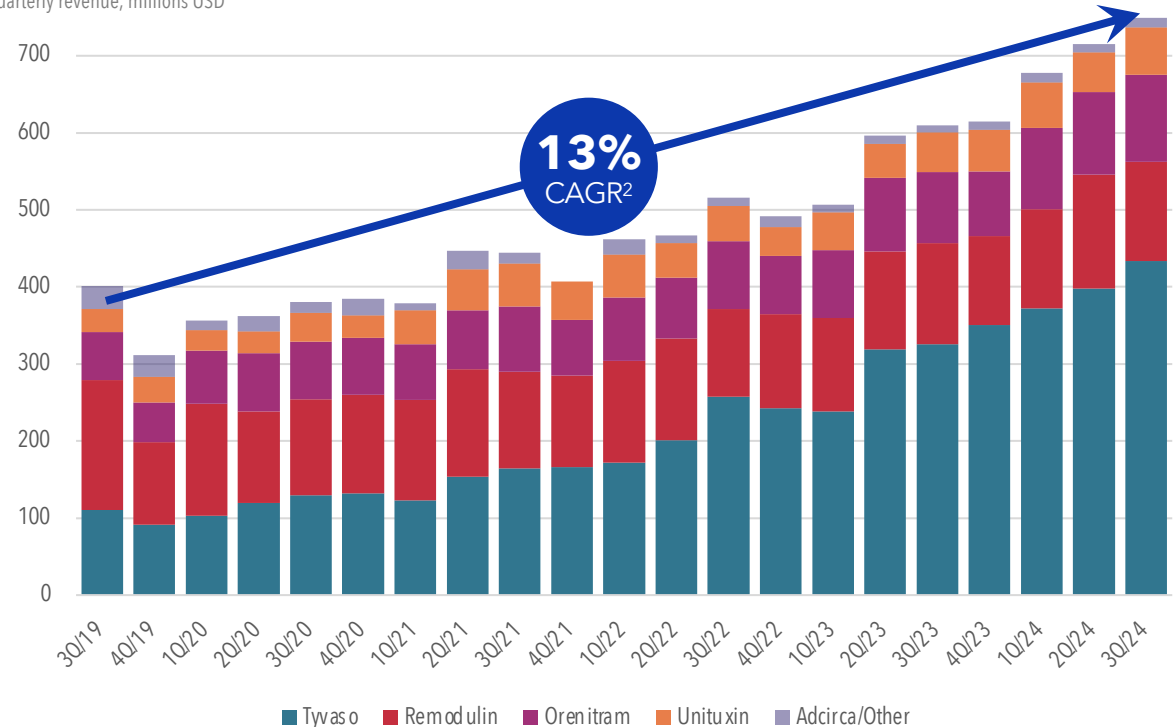
## Unituxin, worldwide

▲ 19% y/y to \$61M

## Total Revenue

▲ 23% y/y to \$749M

Quarterly revenue, millions USD



1. y/y = year over year.

2. CAGR = compound annual growth rate calculated from 3Q/19 to 3Q/24.

3. Tyvaso DPI + nebulized Tyvaso.

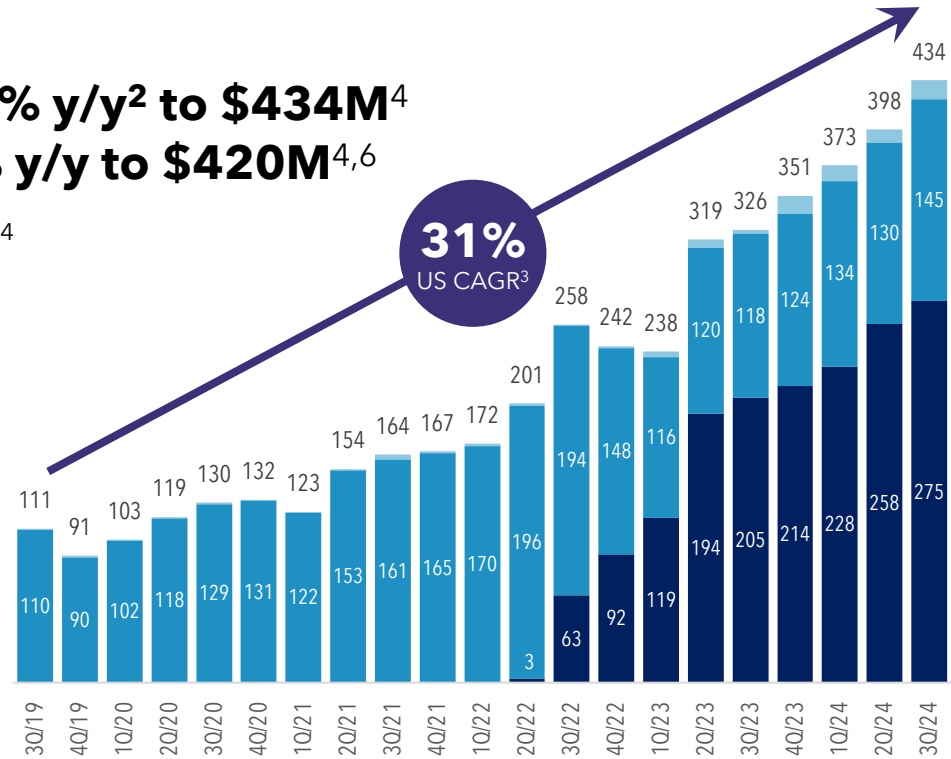
COMMERCIAL EXECUTION

# Tyvaso®

**W/W<sup>1</sup> Combined Revenue ▲ 33% y/y<sup>2</sup> to \$434M<sup>4</sup>**

**U.S. Combined Revenue ▲ 30% y/y to \$420M<sup>4,6</sup>**

- **Most prescribed** prostacyclin in the U.S.<sup>4</sup>
- **Highest revenue** quarter ever<sup>4</sup>
- No material inventory changes q/q<sup>4,5</sup>



**31%**  
US CAGR<sup>3</sup>

1. w/w = worldwide. 2. y/y = year over year. 3. CAGR = compound annual growth rate calculated from 3Q/19 to 3Q/24.  
4. Data reflective of combined Tyvaso DPI + nebulized Tyvaso. 5. q/q = quarter over quarter. 6. Totals may not add due to rounding.

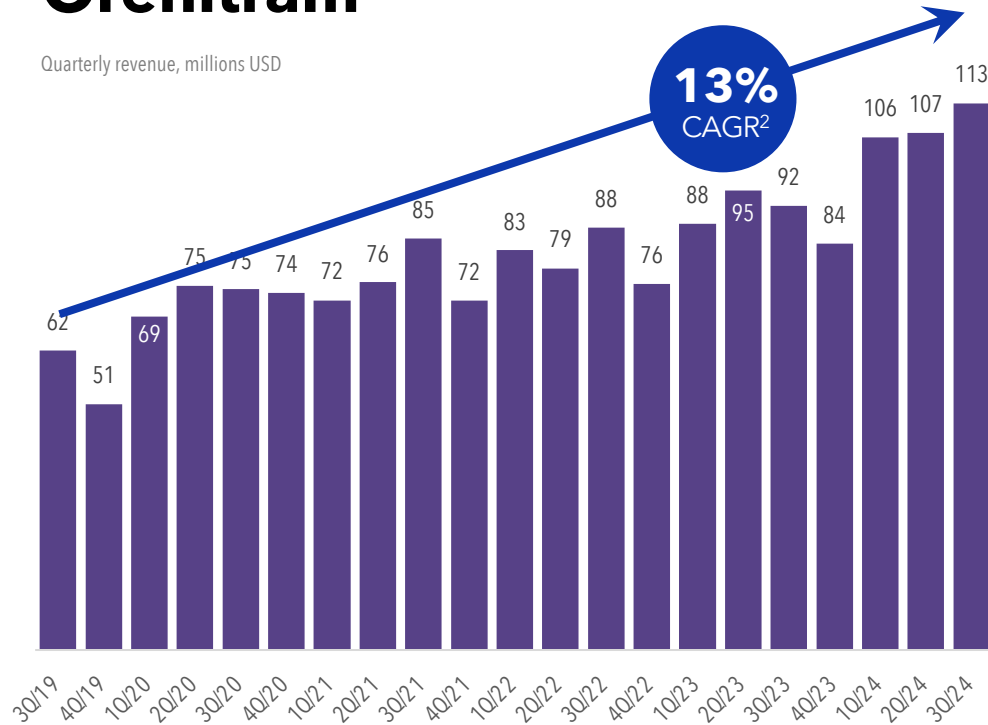
■ Tyvaso DPI ■ U.S. Nebulized Tyvaso ■ ex-U.S. Nebulized Tyvaso

Quarterly revenue, millions USD

## COMMERCIAL EXECUTION

**Orenitram®**

Quarterly revenue, millions USD



**Revenue ▲ 23% y/y<sup>1</sup> to \$113M**

- **Highest revenue** quarter
- **11<sup>th</sup>** sequential quarter of y/y quarterly revenue growth



**orenitram®**  
**treprostinil**

EXTENDED-RELEASE TABLETS

1. y/y = year over year.

2. CAGR = compound annual growth rate calculated from 3Q/19 to 3Q/24.

## COMMERCIAL EXECUTION

# Remodulin®

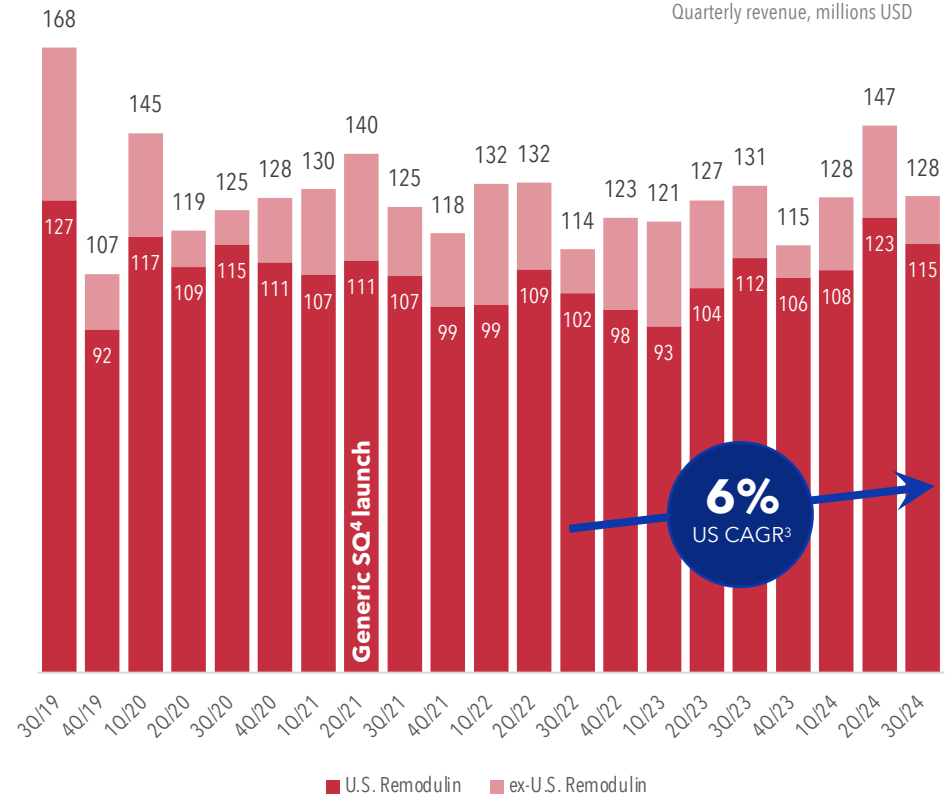
**W/W<sup>1</sup> revenue ▼ 2% y/y<sup>2</sup> to \$128M**

**U.S. revenue ▲ 3% y/y to \$115M**

- **Most prescribed** U.S. parenteral prostacyclin
- **Highest** number of patients on therapy
- International revenue impacted by order timing



**REMODULIN®**  
(treprostinil) Injection

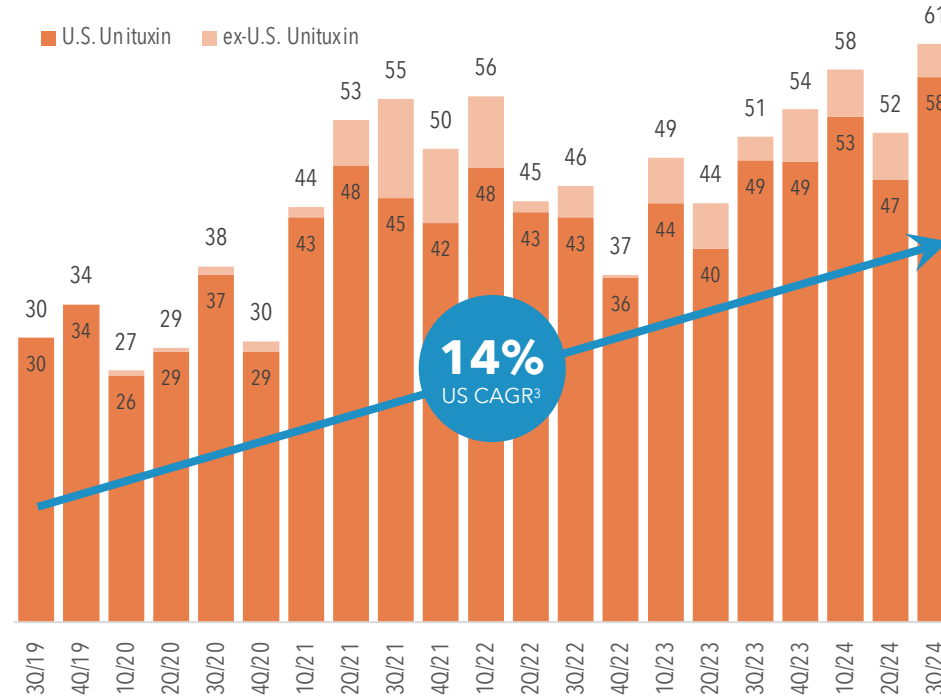


1. w/w = worldwide. 2. y/y = year over year. 3. CAGR = compound annual growth rate calculated from 3Q/22 to 3Q/24. 4. SQ = subcutaneous.

## COMMERCIAL EXECUTION

## Unituxin®

Quarterly revenue, millions USD



**W/W<sup>1</sup> revenue ▲ 19% y/y<sup>2</sup> to \$61M**  
**U.S. revenue ▲ 18% y/y to \$58M<sup>4</sup>**

- **Record** total and U.S. revenue
- The **most prescribed** antibody therapy for high-risk neuroblastoma in the U.S.

**Unituxin®**  
 (dinutuximab)  
 Injection

1. w/w = worldwide. 2. y/y = year over year. 3. CAGR = compound annual growth rate calculated from 3Q/19 to 3Q/24. 4. Percentages may not align due to rounding.



A PUBLIC BENEFIT CORPORATION

**Record** total revenue  
**16<sup>th</sup> consecutive quarter** of  
 y/y<sup>1</sup> revenue growth

1. y/y = year over year.

**TYVASO DPI**<sup>®</sup>  
 (treprostinil) INHALATION  
 POWDER

**TYVASO**<sup>®</sup>  
 (treprostinil) INHALATION  
 SOLUTION

**Most prescribed** U.S. prostacyclin  
**Record** revenue

**REMODULIN**<sup>®</sup>  
 (treprostinil) Injection

**Most prescribed**  
 parenteral prostacyclin in the U.S.  
**Highest number of patients** on therapy



**orenitram**<sup>®</sup>  
 treprostinil

EXTENDED-RELEASE TABLETS

**11<sup>th</sup>** sequential quarter of  
 quarterly y/y revenue growth  
**Record** revenue

**Unituxin**<sup>®</sup>  
 (dinutuximab)  
 Injection

**Record** revenue  
 The **most prescribed**  
 antibody therapy for  
 high-risk neuroblastoma in the U.S.

## Q&A

### **Dr. Martine Rothblatt**

Chairperson & Chief Executive Officer

### **Michael Benkowitz**

President & Chief Operating Officer

### **James Edgmond**

Chief Financial Officer & Treasurer

### **Dr. Leigh Peterson**

EVP, Product Development & Xenotransplantation

### **Patrick Poisson**

EVP, Technical Operations

### **Dewey Steadman**

Head of Investor Relations



An artistic illustration featuring a human heart as the central focus. The heart is rendered in a detailed, anatomical style with fine lines and shading. A hand from the right side of the frame points towards the heart. Below the heart, another hand is shown holding a medical instrument, possibly a catheter or a probe, which is inserted into the heart's structure. The background consists of vertical, textured brushstrokes in shades of teal, blue, and purple, overlaid on a light brown, paper-like texture. A white, torn-edge rectangular strip is positioned above the heart, containing a horizontal line.

**United  
Therapeutics**

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