

AT THE FOREFRONT OF  
THERAPIES FOR RARE DISEASES

# 1Q25 Results Conference Call & Webcast

May 1, 2025



# Forward-Looking Statements

*This presentation contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to the prospects and timing of the potential regulatory and pricing approval of our products, commercialization plans, manufacturing and supply plans, financing plans, the collaboration with Dimerix, and the projected revenues and cash position for the Company. The inclusion of forward-looking statements should not be regarded as a representation by us that any of our plans will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong and can be affected by inaccurate assumptions we might make or by known or unknown risks and uncertainties. For example, statements regarding the goals, progress, timing, and outcomes of discussions with regulatory authorities and pricing and reimbursement authorities, are based on current information. Actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in our business, including, without limitation: the potential that regulatory authorities may not grant or may delay approval for our product candidates; the potential that required regulatory inspections may be delayed or not be successful and delay or prevent product approval; the potential that we may not be successful in negotiations with pricing and reimbursement authorities; the potential that we may not be successful in commercializing Galafold and/or Pombiliti and Opfolda in Europe, the UK, the US and other geographies; the potential that the Dimerix collaboration and license agreement for DMX-200 may not be successful, including without limitation expectations of the timing of Phase 3 clinical trial evaluating DMX-200; the likelihood of success of such clinical trial; the prospects for FDA approval of DMX-200 for FSGS or other indications; the estimated prevalence of FSGS; the achievement of any milestone and timing of any payments associated with milestones and the success of any efforts to commercialize DMX-200, including any projections of future financial performance or payments; ; the potential that we may not be able to manufacture or supply sufficient commercial products; and the potential that we will need additional funding to complete the manufacturing and commercialization of our products. With respect to statements regarding corporate financial guidance and financial goals and the expected attainment of such goals and projections of the Company's revenue, non-GAAP profitability and cash position, actual results may differ based on market factors and the Company's ability to execute its operational and budget plans. In addition, all forward-looking statements are subject to other risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2024 and our Quarterly Report on Form 10-Q to be filed today. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, and we undertake no obligation to revise or update this news release to reflect events or circumstances after the date hereof.*

## Non-GAAP Financial Measures

*In addition to financial information prepared in accordance with U.S. GAAP, this presentation also contains adjusted financial measures that we believe provide investors and management with supplemental information relating to operating performance and trends that facilitate comparisons between periods and with respect to projected information. These adjusted financial measures are non-GAAP measures and should be considered in addition to, but not as a substitute for, the information prepared in accordance with U.S. GAAP. We typically exclude certain GAAP items that management does not believe affect our basic operations and that do not meet the GAAP definition of unusual or non-recurring items. Other companies may define these measures in different ways. When we provide our expectation for non-GAAP operating expenses on a forward-looking basis, a reconciliation of the differences between the non-GAAP expectation and the corresponding GAAP measure generally is not available without unreasonable effort due to potentially high variability, complexity and low visibility as to the items that would be excluded from the GAAP measure in the relevant future period, such as unusual gains or losses. The variability of the excluded items may have a significant, and potentially unpredictable, impact on our future GAAP results.*

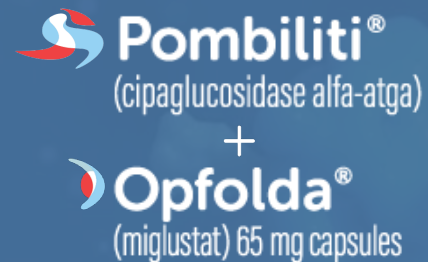
# A Rare Company

A unique story in biotech with significant revenue growth and profitability

**First Oral  
Precision  
Medicine for  
Fabry Disease**



**First Two-  
Component Therapy  
for Pompe Disease**



**10-15%**

**FY 2025  
Galafold Revenue  
Growth<sup>1</sup>**

**50-65%**

**FY 2025  
Pombiliti + Opfolda  
Revenue Growth<sup>1</sup>**

**Expanded  
Portfolio with  
U.S. Licensing of  
DMX-200  
Phase 3 Program**

**Leverageable  
Global  
Commercial  
Organization**

**\$125M**

**1Q 2025 Total Revenue  
(+15% Growth)<sup>1</sup>**

**\$1B+**

**Total Revenue  
Expected in FY 2028**

# Galafold<sup>®</sup> (*migalastat*)

## Continued Growth

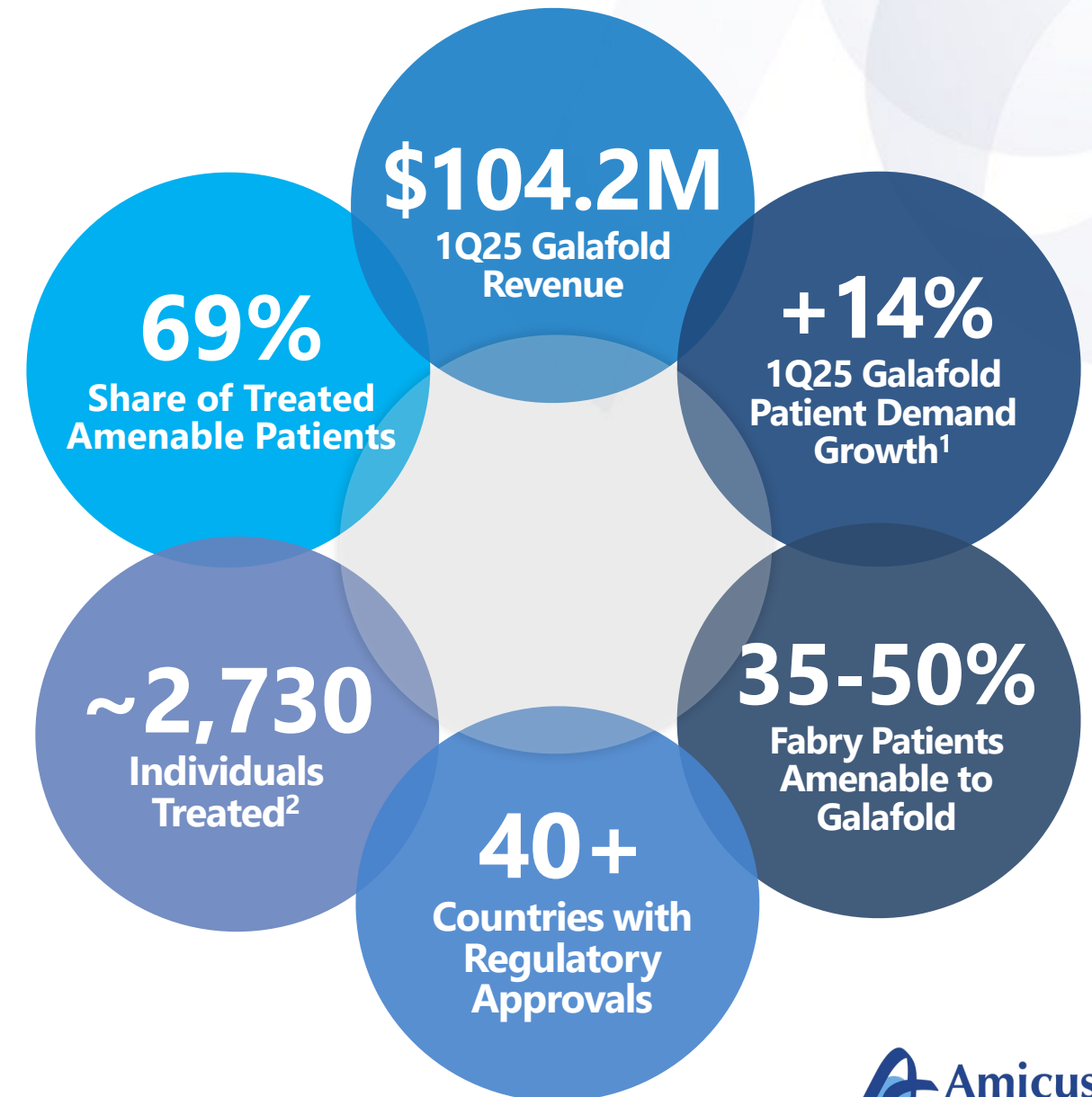
Building a leadership position  
in the treatment of Fabry disease



# 2025 Galafold Success (as of March 31, 2025)

Only approved oral treatment in Fabry disease and standard of care for amenable patients

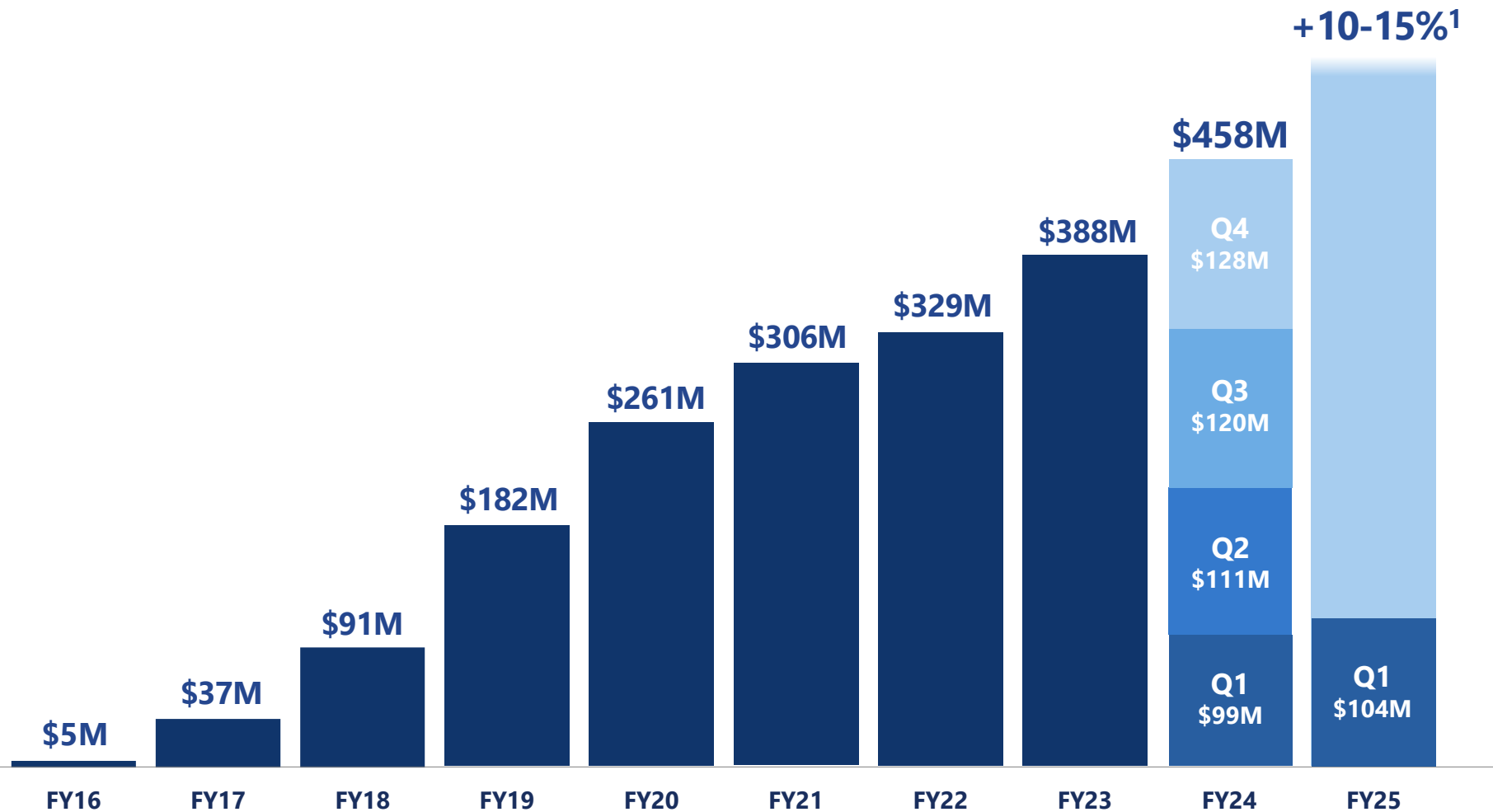
A unique mechanism of action for Fabry patients with amenable variants



Galafold is indicated for adults with a confirmed diagnosis of Fabry disease and an amenable variant. The most common adverse reactions reported with Galafold ( $\geq 10\%$ ) were headache, nasopharyngitis, urinary tract infection, nausea, and pyrexia. For additional information about Galafold, including the full U.S. Prescribing Information, please visit <https://amicusrx.com/pi/galafold.pdf>. For further important safety information for Galafold, including posology and method of administration, special warnings, drug interactions, and adverse drug reactions, please see the European SmPC for Galafold available from the EMA website at [www.ema.europa.eu](http://www.ema.europa.eu).

# Galafold Performance

1Q 2025 Galafold reported revenue of \$104.2M (+6% growth at CER)



- Quarterly patient starts remains strong: +14% growth in 1Q YoY<sup>2</sup>
- 1Q revenue impacted by ordering patterns and greater than anticipated U.K. rebate
- Global mix of naïve (~65%) and switch (~35%) patients<sup>2</sup>
- Expanding market through uptake in naïve population as well as label and geographic expansion
- Maintaining >90% adherence and compliance through HCP and patient education and support

Revenue growth expected to accelerate throughout the year and FY 2025 Galafold growth guidance of 10-15% at CER<sup>1</sup> reiterated

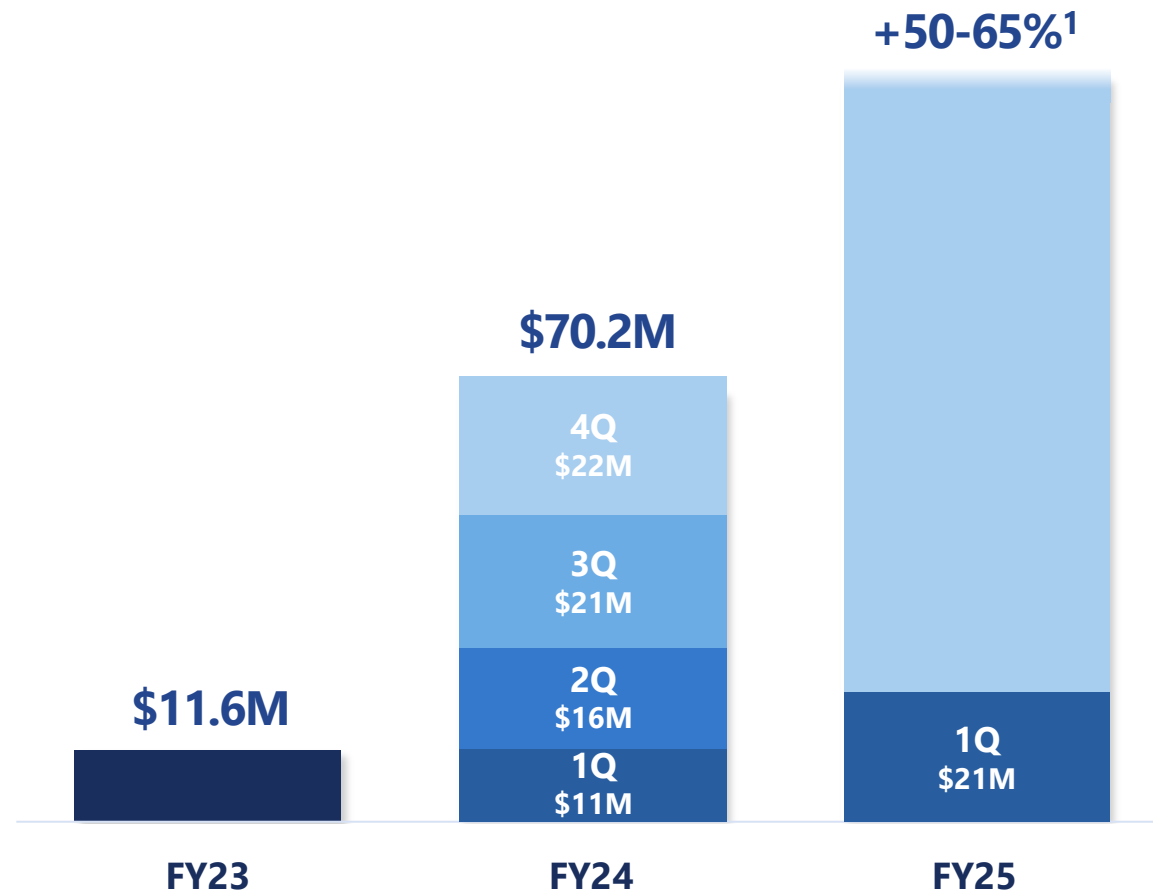
**Pombiliti**<sup>®</sup> (*cipaglucosidase alfa-atga*)  
+  
**Opfolda**<sup>®</sup> (*miglustat*)

Potential to establish a new standard of care  
for people living with late-onset Pompe disease



# Pombiliti + Opfolda Performance

1Q 2025 Pombiliti + Opfolda reported revenue of \$21.0M (+92% at CER)



- Strong 1Q sales growth YoY, particularly in the U.S.
  - Number of net new patients continues to grow
  - Increasing depth and breadth of prescribers
- 1Q 2025 revenue reflected timing of patient starts in new launch countries and higher than anticipated VPAG rebate in the U.K.
- Ramp in new patient starts expected to be weighted to 2H 2025 due to:
  - Multiple new launch markets coming online
  - Anticipated acceleration in U.S. switches
  - Growing body of RWE<sup>2</sup> supporting switch from both alternative therapies

Updating FY 2025 Pombiliti + Opfolda growth guidance to 50-65% at CER<sup>1</sup>

# Pombiliti + Opfolda Expansion

3 regulatory approvals and up to 10 new launch countries in 2025

## Regulatory

- Recently approved in Australia and Canada
- Regulatory approval in Japan anticipated in 2025

## Reimbursement

- Expect to launch in up to 10 new countries this year
  - >650 LOPD patients 18+ in those 10 countries
- First commercial patients from all 6 newly reimbursed countries anticipated no later than 2Q 2025
- Pombiliti + Opfolda selected as preferred treatment for adults with LOPD in the Netherlands
  - 5-year agreement enables broad and sustained access for adults with LOPD currently on ERT

### Regulatory approvals anticipated in 2025:



AUSTRALIA



CANADA



JAPAN

Combined ~150-200 people 18+ living with LOPD and being treated with a Pompe therapy

### New reimbursement agreements completed in:



SWEDEN



SWITZERLAND



CZECH REPUBLIC



NETHERLANDS



ITALY



PORTUGAL

Combined ~325-375 people 18+ living with LOPD and being treated with a Pompe therapy

# Supply Chain

## Ensuring access to our two medicines through supply chain planning

### Limited Tariff Impact

- Anticipate no material impact to P&L or business operations in 2025 of any of the proposed tariffs:
  - Well managed through careful expense management and supply chain planning
  - Majority of revenue outside the U.S.
  - 2025 U.S. sales inventory already within the U.S.

### Diversifying Supply Chain

- New commercial manufacturing and supply services agreement in place with **Sharp** Sterile Manufacturing
  - **Pombiliti drug product** manufacturing capacity to be further expanded to a U.S. site in Lee, MA
  - Diversifying our global supply chain for Pombiliti



Sharp

# Pombiliti + Opfolda Body of Evidence

Growing number of abstracts, manuscripts, and case studies supporting Pombiliti + Opfolda differentiation



## Clinical Trials & Long-Term Data

- Long-term Phase 1/2 open-label safety and efficacy study (ATB200-02)
- 104-week Phase 3 open-label extension study of efficacy and safety (ATB200-07)



## Mechanistic & Translational Insights

- Miglustat: a first-in-class enzyme stabilizer for LOPD
- Linking mechanism of action to clinical outcomes in LOPD



## Comparative & Real-World Data

- Network meta-analysis comparing the efficacy of cipaglucosidase alfa + miglustat with other ERTs
- U.K. EAMS<sup>1</sup> registry post-baseline outcomes



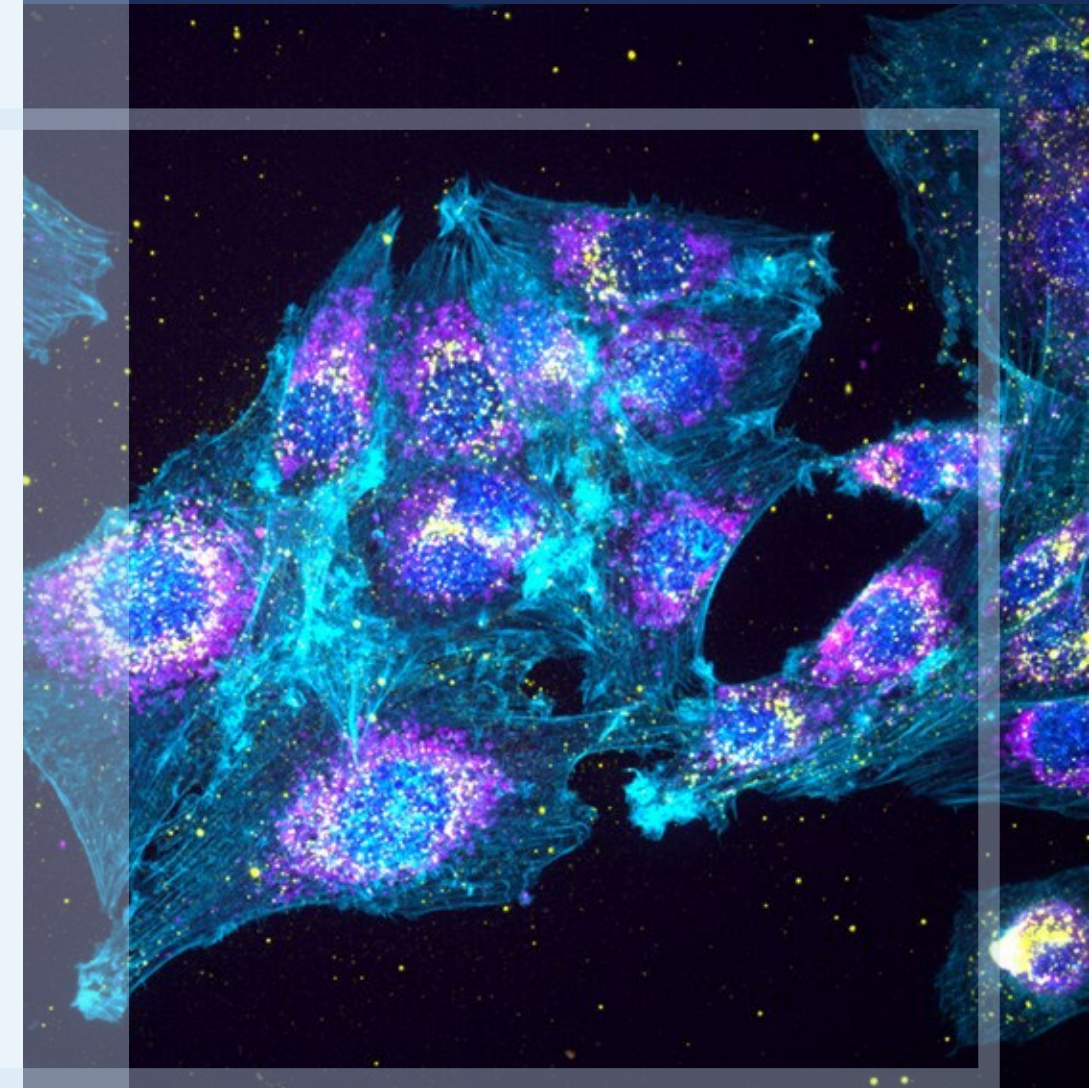
## Case Studies & Real-World Reports

- Case studies supporting the switch from both alternative therapies
- Case studies of patients switching from high dose, high frequency alglucosidase alfa



# DMX-200

Potential first-in-class investigational small molecule for the treatment of FSGS in the U.S.



# Exclusive U.S. License Agreement with Dimerix

Deal adds significant value to Amicus today and aligns with Amicus strategy to leverage our rare disease commercial infrastructure with promising late-stage program

- 1 **Focal Segmental Glomerulosclerosis** (FSGS) is a rare and fatal kidney disease<sup>1</sup> that affects **>40,000 people in the U.S.**, with no approved treatments
- 2 **DMX-200** is a **Phase 3** program, with **Orphan Drug** designation, in development for a disease with **blockbuster market potential** in the U.S.
- 3 **ACTION3 study** is well underway with **positive interim analysis**<sup>2</sup> and agreement with FDA on **proteinuria** as primary endpoint
- 4 **Structured in-licensing deal** with modest upfront and downstream success-based milestones and tiered royalties
- 5 Opportunity to pursue **additional indications**



# DMX-200 Transaction Summary

License agreement is heavily weighted to mutually beneficial success-based milestones

»» Upfront investment of **\$30M** paid with cash on hand

»» Up to **\$560M** in potential **success-based milestone** payments

- ▶ Up to **\$75M** in development & regulatory milestones until FDA approval of DMX-200 in FSGS
- ▶ **\$35M** on first sale
- ▶ Up to **\$410M** in commercial sales milestones
- ▶ Up to **\$40M** in milestones for potential future indications

»» **Tiered royalties** at the **low teens** to **low twenties**

No change to Amicus GAAP profitability guidance during 2H 2025

# Focal Segmental Glomerulosclerosis (FSGS)

is a rare disease leading to irreversible kidney damage

- Irreversible scarring leads to permanent **kidney damage** and eventual **end-stage renal failure**<sup>1</sup>
- Symptoms include **proteinuria**, edema, high cholesterol and blood pressure, low albumin levels
- Average time from diagnosis to onset of complete kidney failure is typically **five to ten years**<sup>2</sup>
- FSGS kidney damage can lead to **dialysis, kidney transplants, or death**







<sup>1</sup> Guruswamy Sangameswaran KD, Baradhi KM. Focal Segmental Glomerulosclerosis (July 2021), online: <https://www.ncbi.nlm.nih.gov/books/NBK532272/>;

<sup>2</sup> Kiffel et. Al. Adv Chronic Kidney Dis. (September 2011), online: <https://pmc.ncbi.nlm.nih.gov/articles/PMC3709971/pdf/nihms286597.pdf>

## Pathogenic Feedback Loop in FSGS



# Dimerix has built a strong body of evidence and made significant clinical and regulatory progress with DMX-200 in FSGS

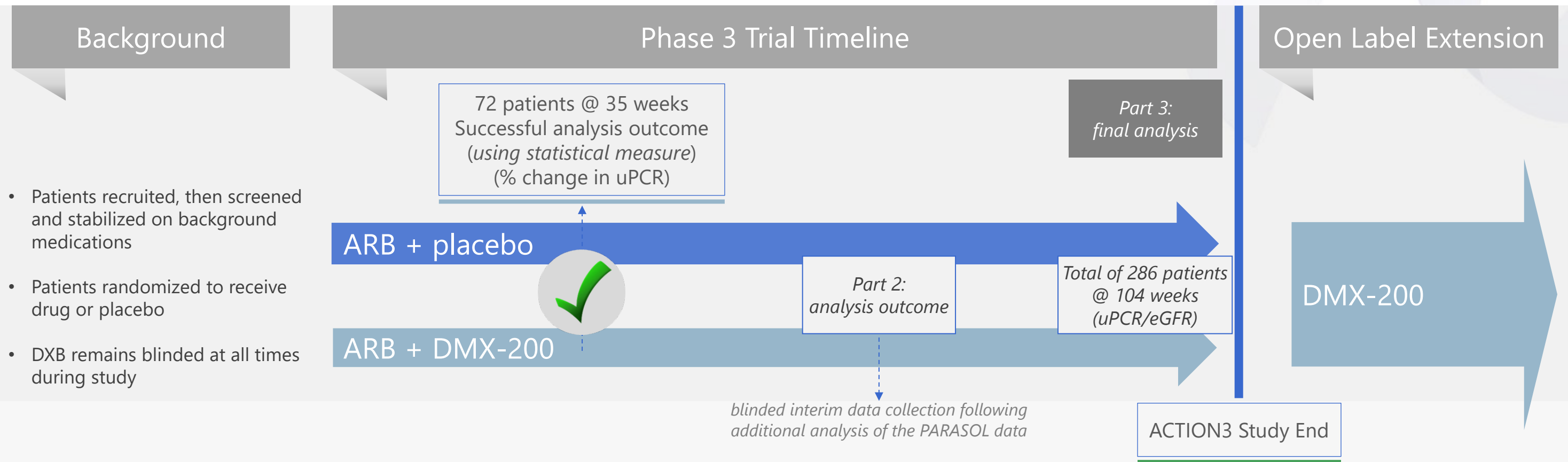
	<b>MOA</b>	→	Precision therapy to disrupt the pathogenic monocyte-driven inflammatory feedback loop in the kidney of patients with FSGS
	<b>Phase 2</b>	→	Positive efficacy signals and well-tolerated across studies (n=80), including impacts on proteinuria and inflammation in FSGS study
	<b>ACTION3 Phase 3</b>	→	Enrollment well underway (185 of 286 pts to date); Interim analysis (n=72 at 36 wks) showed DMX-200 performing better than placebo in reducing proteinuria <sup>1</sup>
	<b>FDA and Project PARASOL</b>	→	Alignment on proteinuria as a primary endpoint for approval
	<b>ACTION3 Part 2 Interim Analysis</b>	→	Expected after planned follow-up meeting with FDA
	<b>ACTION3 Part 3 Final Analysis</b>	→	2-year proteinuria (primary) and eGFR (secondary) data serves as basis for Full Approval (n=286)

# DMX-200 Phase 3 Clinical Trial in FSGS



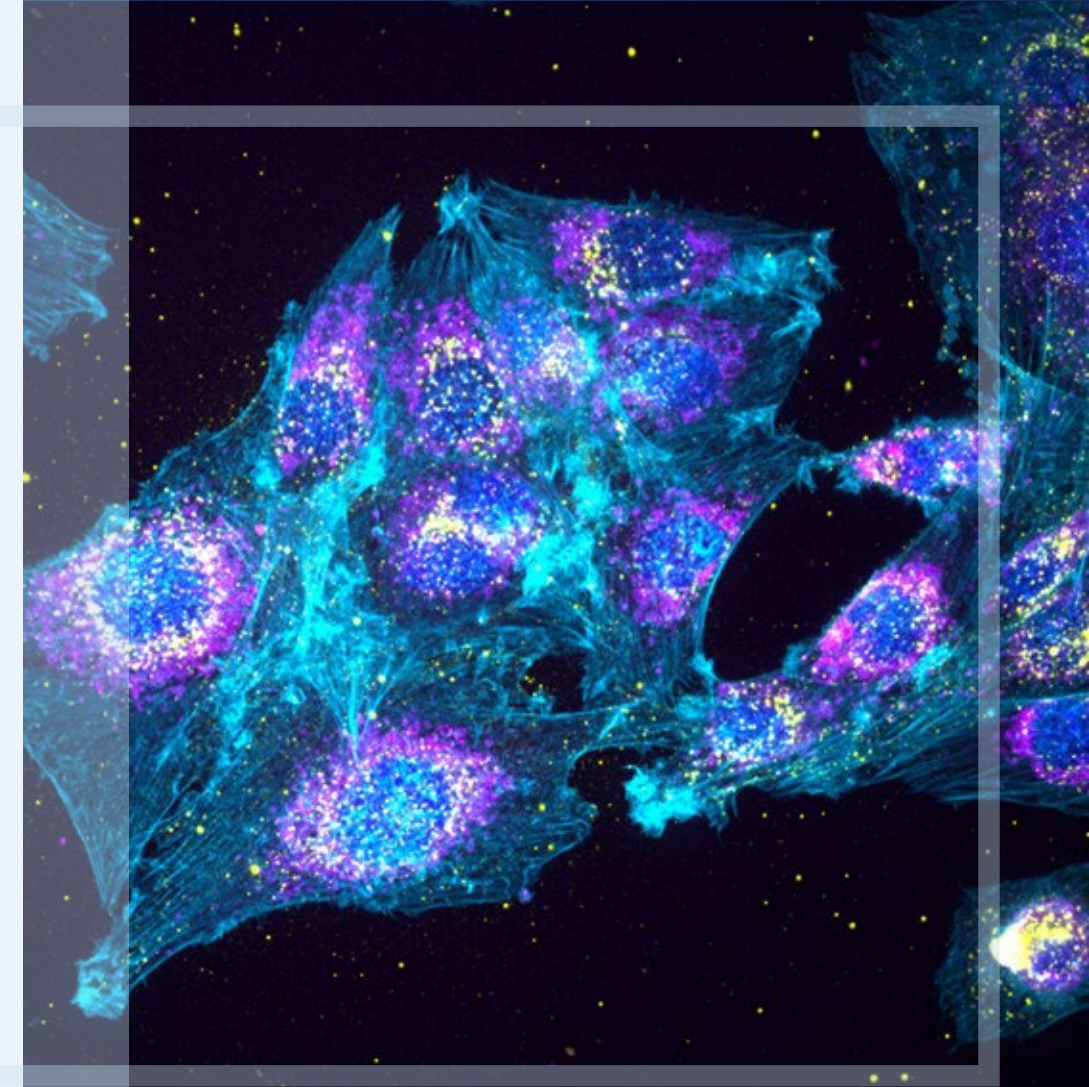
**FDA aligned on proteinuria endpoint for approval; 185 patients already randomized/dosed to date**

A randomised, double-blind, multi-center, placebo-controlled study of renal outcomes of DMX-200 in patients with FSGS receiving an ARB



# Corporate Outlook

Delivering on our mission for patients  
and shareholders



# Q1 2025 Select Financial Results

Q1 2025 revenue of \$125M, up 15% at CER and non-GAAP net income of \$9.0M

Q1'25

*(in thousands, except per share data)*

	Mar. 31, 2025	Mar. 31, 2024
<b>Net product sales</b>	\$ 125,249	\$ 110,403
<b>Cost of goods sold</b>	11,698	13,567
<b>GAAP operating expenses</b>	121,503	124,557
<b>Non-GAAP operating expenses</b>	94,494	85,555
<b>GAAP net loss</b>	(21,686)	(48,419)
<b>Non-GAAP net income (loss)</b>	8,963	(4,581)
<b>GAAP net loss per share – basic and diluted</b>	\$ (0.07)	\$ (0.16)
<b>Non-GAAP net income (loss) per share – basic and diluted</b>	\$ 0.03	\$ (0.02)

# FY 2025 Financial Guidance

FY 2025 Financial Guidance <sup>1</sup>	Previous Guidance	Updated Guidance
Total Revenue Growth <sup>1</sup>	17% to 24%	15% to 22%
Galafold Revenue Growth <sup>1</sup>	10% to 15%	10% to 15%
Pombiliti + Opfolda Revenue Growth <sup>1</sup>	65% to 85%	50% to 65%
Gross Margin	Mid 80%	Mid 80%
Non-GAAP Operating Expense	\$350M to \$370M	\$380M to \$400M inclusive of \$30M upfront fee
GAAP Net Income	Positive during H2 2025	Positive during H2 2025

## FY 2025 Revenue Sensitivity

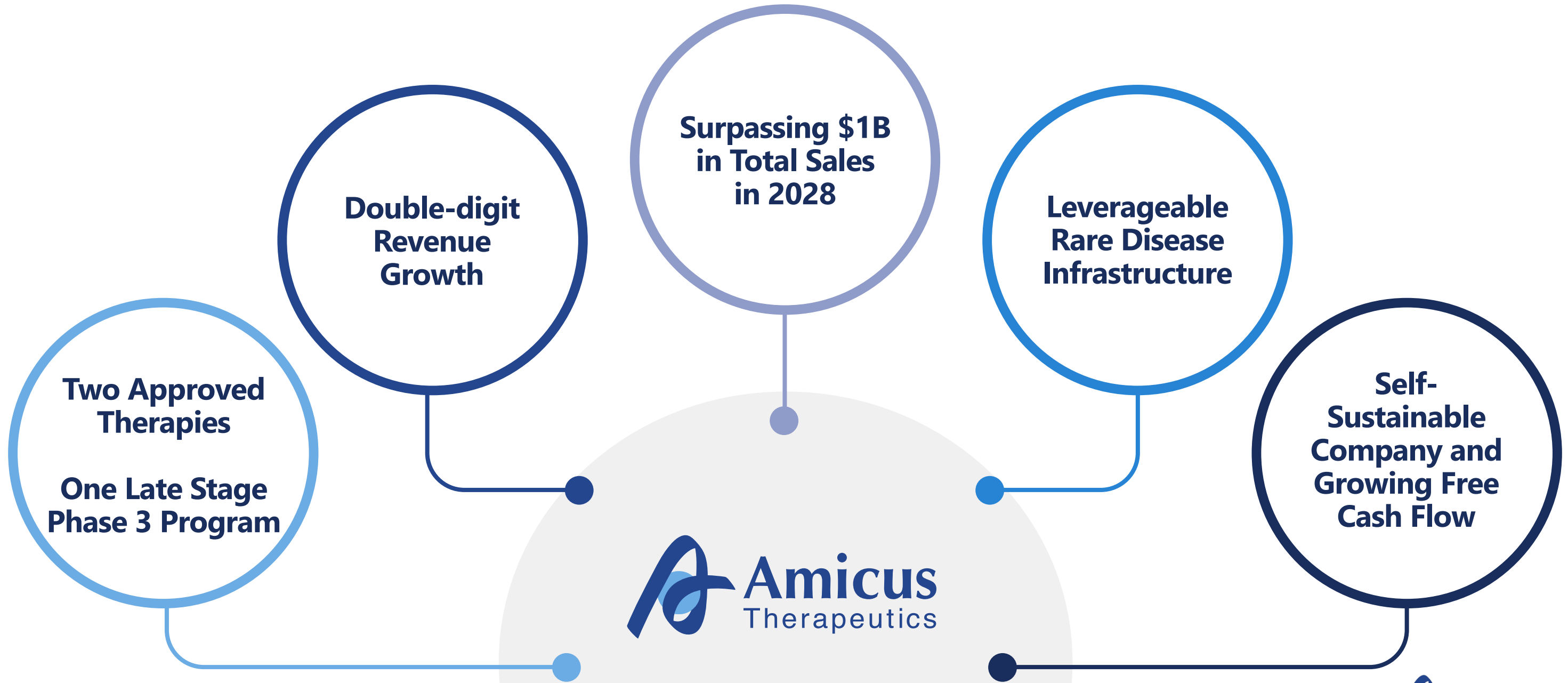
Given the proportion of Amicus revenue ex-US (~60% in 2024), a change in USD exchange rates of +/- 1% compared to 2024 rates could lead to a ~\$4M move in Total Reported Revenues in 2025

# 2025 Strategic Priorities

- 1 Deliver total revenue growth of 15-22% at CER<sup>1</sup>
- 2 Double-digit Galafold<sup>®</sup> revenue growth of 10-15% at CER<sup>1</sup>
- 3 Pombiliti<sup>®</sup> + Opfolda<sup>®</sup> revenue growth of 50-65% at CER<sup>1</sup>
- 4 Advance ongoing studies in Fabry, Pompe and *now FSGS*
- 5 Deliver positive GAAP net income during H2 2025

# A Rare Company

A unique story in biotech with significant revenue growth and profitability



# Appendix



# Reconciliation of Non-GAAP Financial Measures

**Amicus Therapeutics, Inc.**  
**Reconciliation of Non-GAAP Financial Measures**  
(in thousands)  
(Unaudited)

	Three Months Ended March 31,	
	2025	2024
<b>Total GAAP operating expenses</b>	<b>\$ 121,503</b>	<b>\$ 124,557</b>
<b>Research and development:</b>		
Share-based compensation	4,004	4,871
<b>Selling, general and administrative:</b>		
Share-based compensation	21,168	25,932
<b>Restructuring charge</b>	—	6,045
<b>Depreciation and amortization</b>	1,837	2,154
<b>Total Non-GAAP operating expense adjustments</b>	27,009	39,002
<b>Total Non-GAAP operating expenses</b>	<b>\$ 94,494</b>	<b>\$ 85,555</b>

# Reconciliation of Non-GAAP Financial Measures *(Cont'd)*

**Amicus Therapeutics, Inc.**  
**Reconciliation of Non-GAAP Financial Measures**  
**(in thousands, except share and per share amounts)**  
**(Unaudited)**

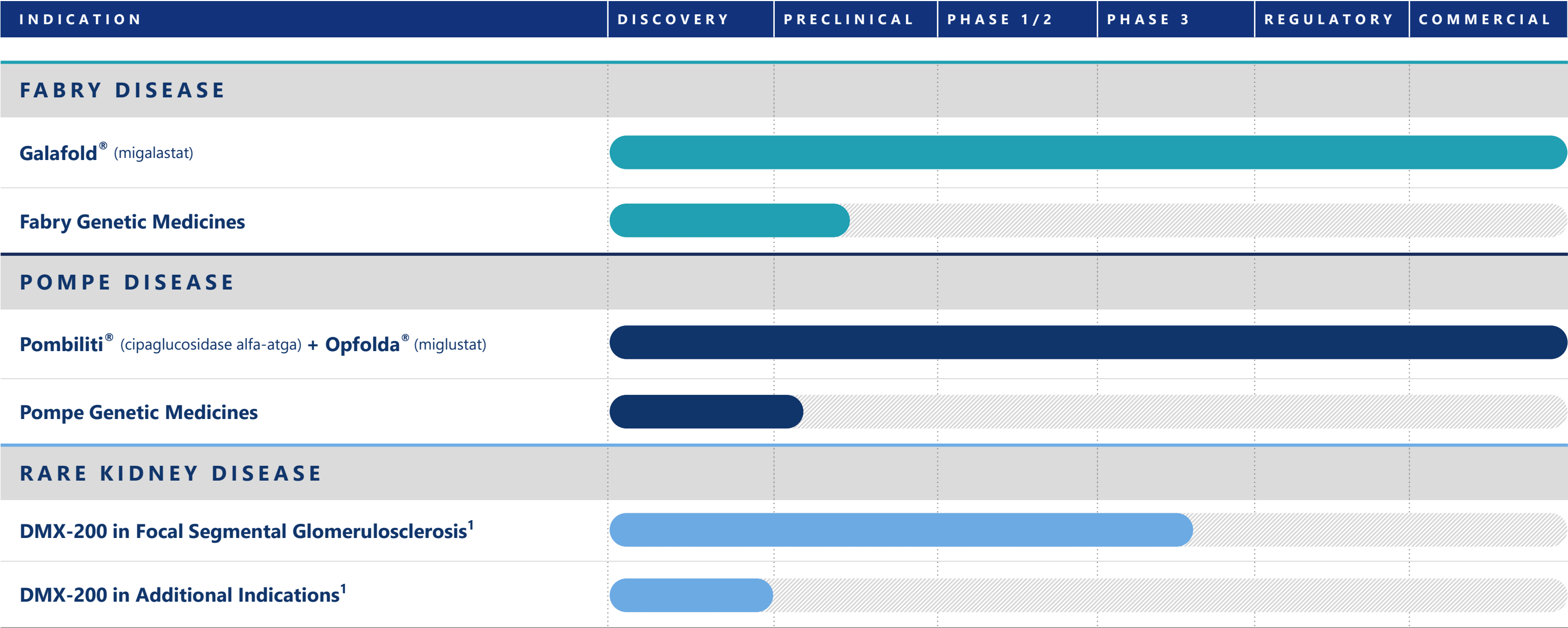
	Three Months Ended March 31,	
	2025	2024
<b>GAAP net loss</b>	<b>\$ (21,686)</b>	<b>\$ (48,419)</b>
Share-based compensation	25,172	30,803
Depreciation and amortization	1,837	2,154
Restructuring charges	—	6,045
Income tax expense	3,641	4,836
Non-GAAP net income (loss)	<b>\$ 8,963</b>	<b>\$ (4,581)</b>
Non-GAAP net income (loss) attributable to common stockholders per common share — basic and diluted	\$ 0.03	\$ (0.02)
Weighted-average common shares outstanding — basic	307,689,207	302,903,009
Weighted-average common shares outstanding — diluted	309,654,136	302,903,009

# Exchange Rates

## Currency Average Rates

FX Rates	Q1 2024	Q1 2025	Variance
USD/EUR	1.086	1.052	(3.1%)
USD/GBP	1.268	1.259	(0.7%)
USD/JPY	0.007	0.007	(2.7%)

# Rare Disease Pipeline



<sup>1</sup> Exclusive rights to commercialize in the United States