

AT THE FOREFRONT OF
THERAPIES FOR RARE DISEASES

Q3 2025 Results Conference Call & Webcast

November 4, 2025



 **Amicus**
Therapeutics®

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 license agreement for DMX-200 may not be successful, including without limitation expectations of the timing of the 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, GAAP and 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

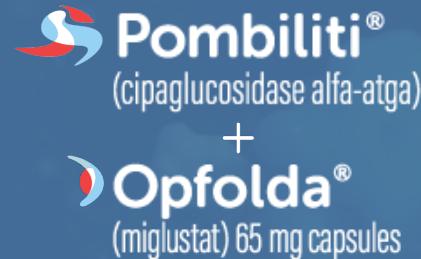
A unique story in biotech with significant revenue growth and profitability

**First Oral
Precision
Medicine for
Fabry Disease**



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

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



10-15%

**FY 2025
Galafold Revenue
Growth¹**

50-65%

**FY 2025
Pombiliti + Opfolda
Revenue Growth¹**

\$169M

**Q3 2025 Total Revenue
(+17% Growth)¹**

\$1B+

**Total Revenue
Expected in FY 2028**

Galafold® *(migalastat)* Continued Growth

Building a leadership position
in the treatment of Fabry disease



2025 Galafold Success (as of September 30, 2025)

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

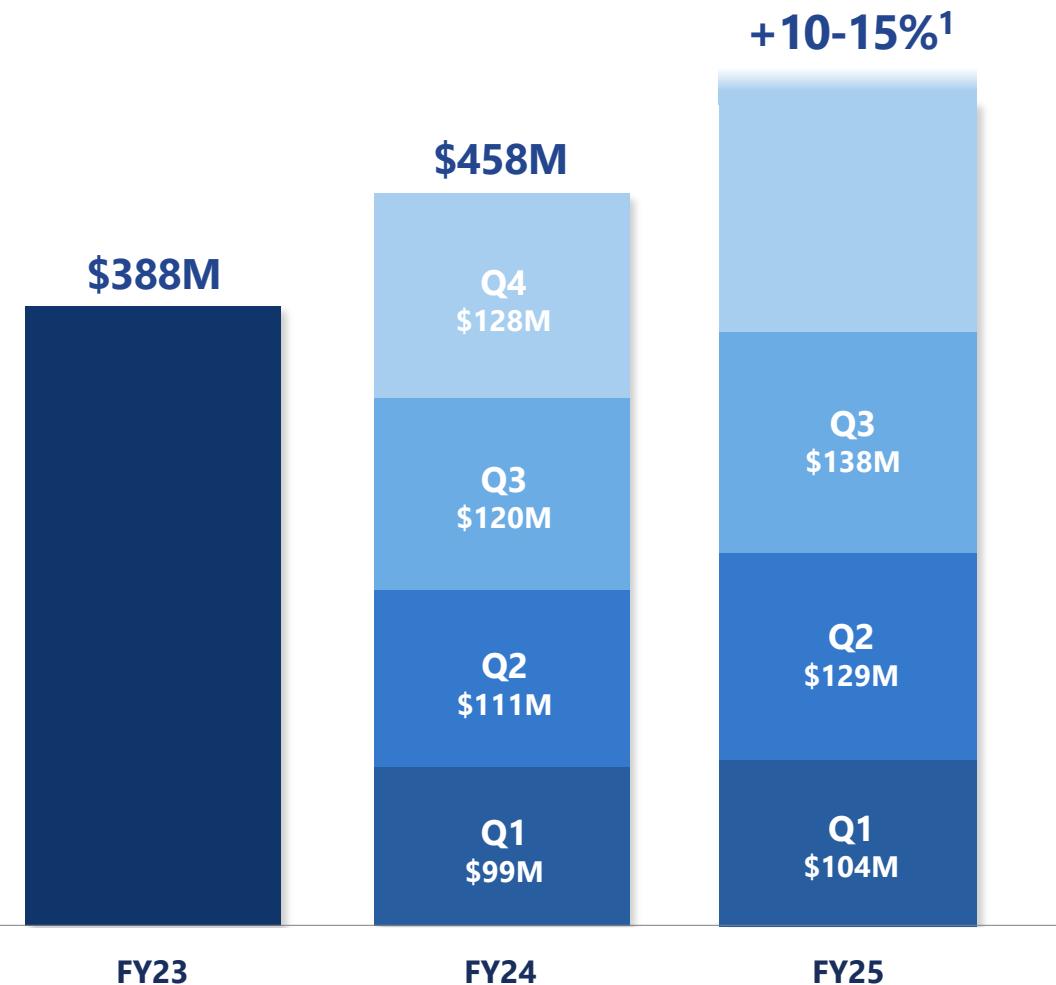
**A unique mechanism of action for
Fabry patients with amenable variants**



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.

Galafold Performance

Q3 2025 Galafold reported revenue of \$138.3M (+12% at CER)

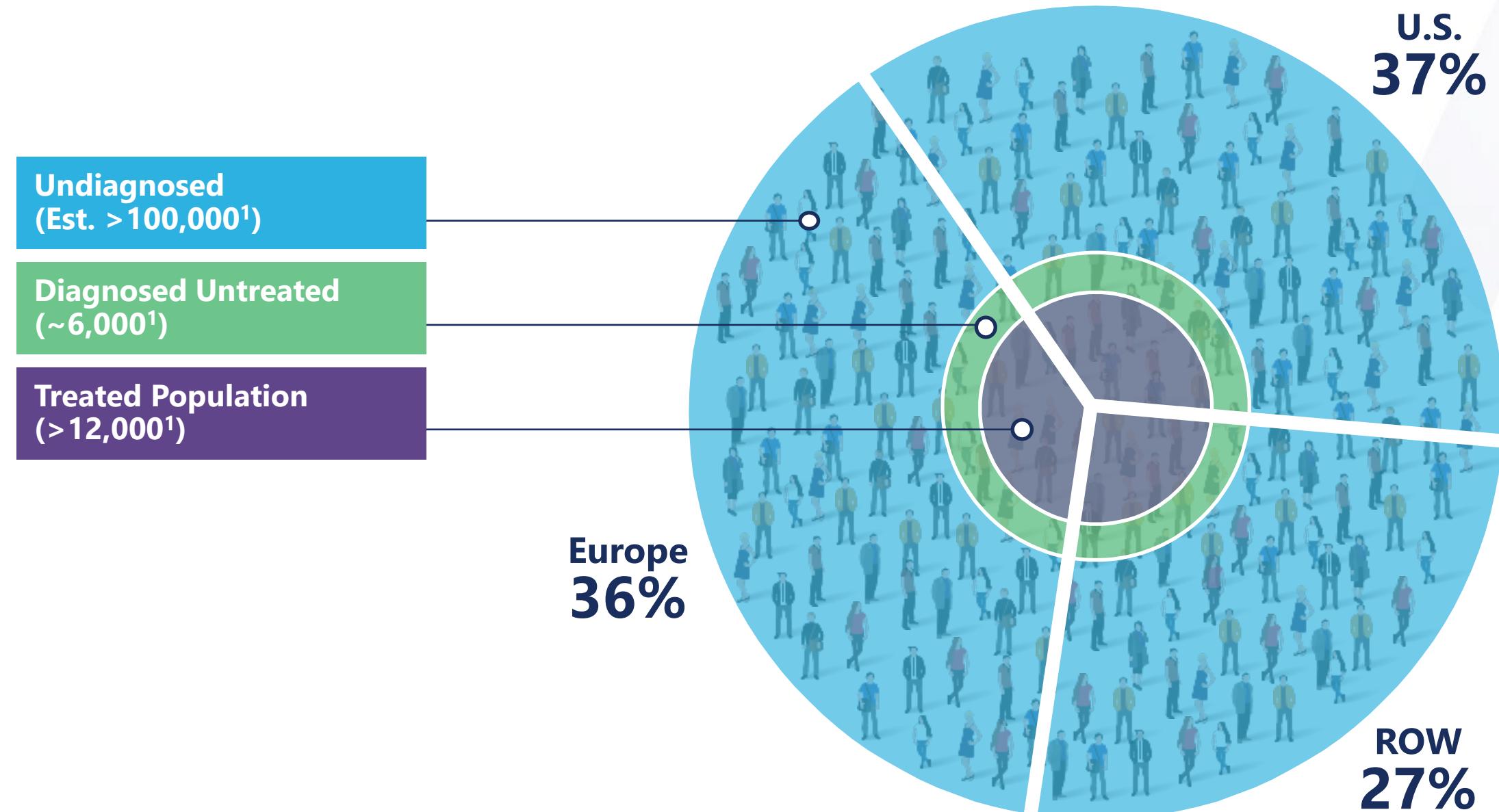


- Quarterly patient starts remain strong: +13% growth YoY in Q3 2025
- Q3 2025 one of the highest demand quarters and best number of starts YTD since launch
- Global mix of naïve (~65%) and switch (~35%) patients²
- Expanding market through uptake in naïve population as well as label and geographic expansion
- Maintaining >90% adherence and compliance through HCP and patient education and support

Reiterating FY 2025 Galafold growth guidance of 10-15% at CER

Fabry Market: Significant Remaining Unmet Need

Research suggests there could be >100k people living with Fabry disease who remain undiagnosed



Based on publicly reported sales of Fabry therapies

¹Applying incidence of multiple newborn screening studies to population estimates in commercial markets:
Burton 2017 J Pediatr 2017;190:130-5 ; Mechtler *et al.*, The Lancet, 2011 Dec.; Hwu *et al.*, Hum Mutation, 2009 Jun; Spada *et al.*, Am J Human Genet., 2006 Jul

New **FINDING FABRY** Initiative in the U.S.



New **FINDING FABRY** website & campaign now live

For US healthcare professionals only

FINDING FABRY

What Causes Fabry Disease? ▾ Recognizing Fabry Disease ▾ Testing & Management Options ▾ Resources

FABRY DISEASE CAN HIDE IN PLAIN SIGHT

Fabry disease can blend in with other diseases. Symptoms vary, making diagnosis a challenge

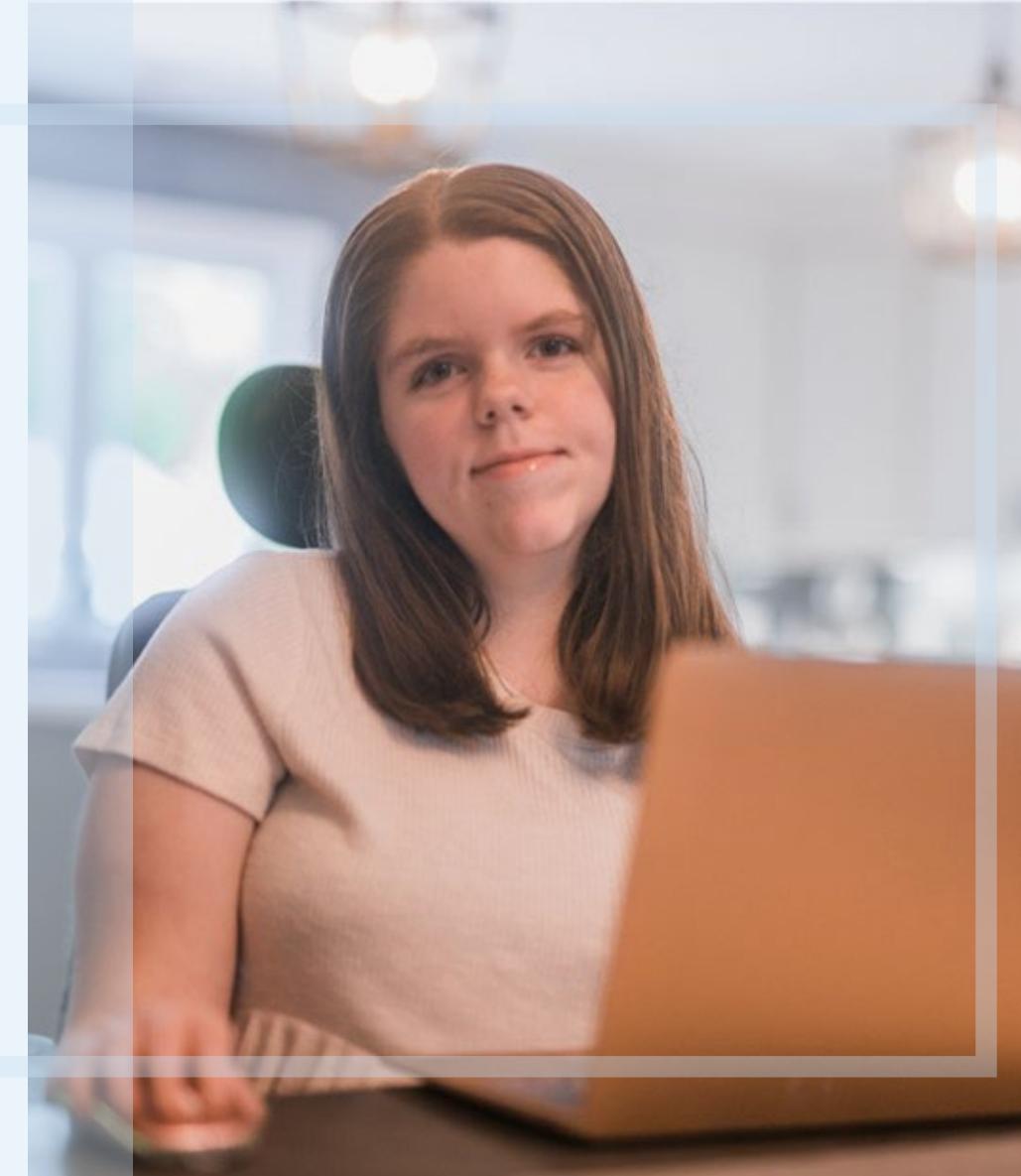


A photograph of a man sitting on a couch. The couch and the man's pajamas are covered in a pattern of red and orange kidney shapes, symbolizing how Fabry disease can be hidden within other diseases. The man is smiling and looking towards the camera.



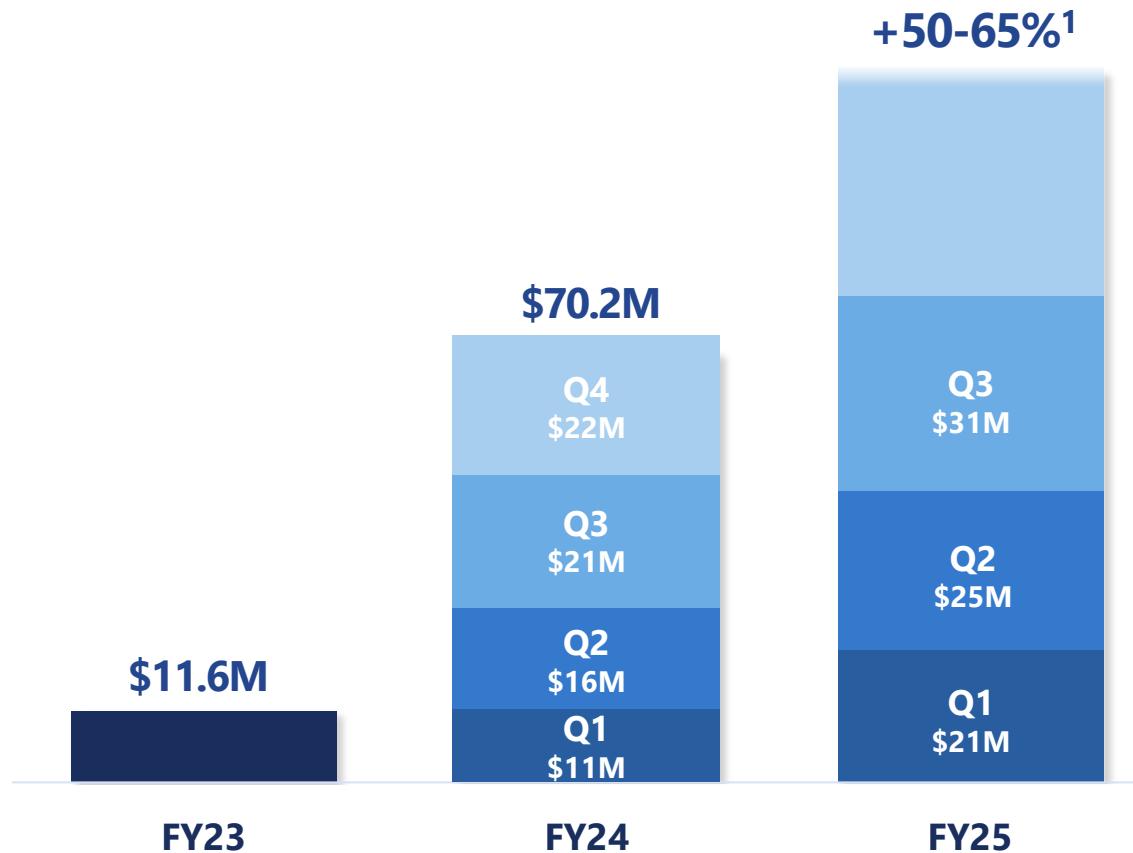
Pombiliti® *(cicapglucosidase alfa-1atga)* + **Opfolda®** *(miglustat)*

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



Pombiliti + Opfolda Performance

Q3 2025 Pombiliti + Opfolda reported revenue of \$30.7M (+42% at CER)



- Strong Q3 sales growth YoY
 - Increasing depth and breadth of prescribers
 - Doubled number of naïve starts (ex-U.S) in first 9 months of 2025 vs FY 2024
 - Doubled number of avalglucosidase alfa switches in first 9 months of 2025 vs FY 2024
- H2 2025 growth driven by:
 - Benefit of new patient starts in existing and newly launched markets
 - Growing body of RWE² supporting switch from both alternative therapies

Reiterating FY 2025 Pombiliti + Opfolda growth guidance of 50-65% at CER¹

Pombiliti + Opfolda Expansion

Significant progress made towards expanding access to Pombiliti + Opfolda in 2025

Regulatory

- Approved in Australia and Canada in Q1 2025
- Approved in Japan in June 2025

Regulatory approvals in 2025:



AUSTRALIA



CANADA



JAPAN

Reimbursement

- Now reimbursed in 15 countries with 10 countries added in 2025 providing access to ~2,400 treated patients
- First commercial patients in 8 new countries YTD
- Pombiliti + Opfolda selected as preferred treatment for adults with LOPD in the Netherlands

Reimbursement agreements completed in 2025:



SWEDEN



SWITZERLAND



ITALY



JAPAN



IRELAND



NETHERLANDS



PORTUGAL



CZECH
REPUBLIC



BELGIUM



LUXEMBOURG

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. EAMS¹ registry post-baseline outcomes
- CDMU–UCLH² cohort analysis comparing cipaglucosidase alfa + miglustat vs. alglucosidase alfa



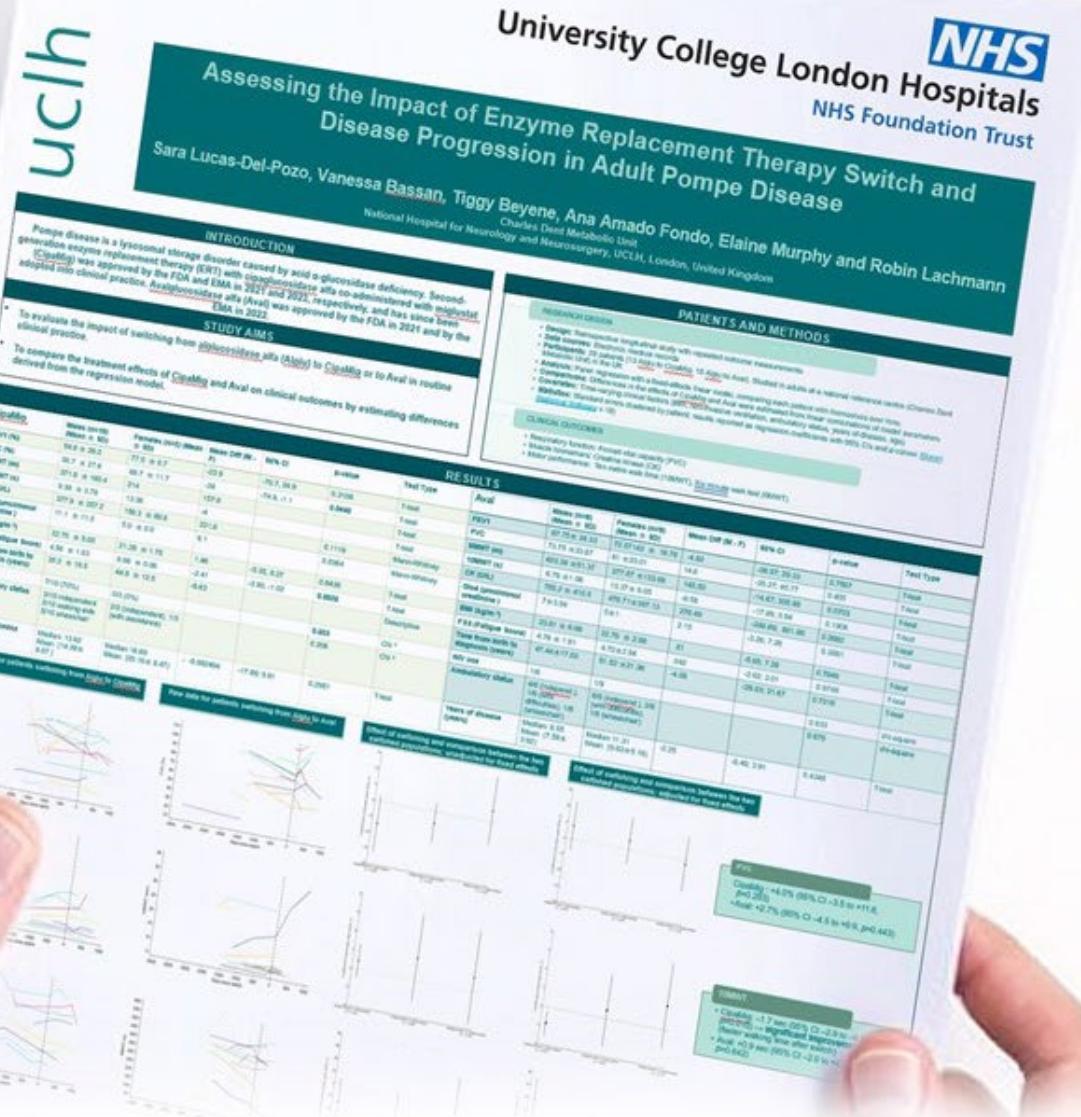
Case Studies & Real-World Reports

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



Emerging Pombiliti + Opfolda Real-World Evidence

Real-world data recently presented by independent researchers



Summary of adjusted models:

cipaglucosidase alfa +
miglustat vs.
alglucosidase alfa

Significant
increase



6MWD



10MWT



FVC



CK

avalglucosidase alfa
vs. alglucosidase alfa

Non-significant
decrease

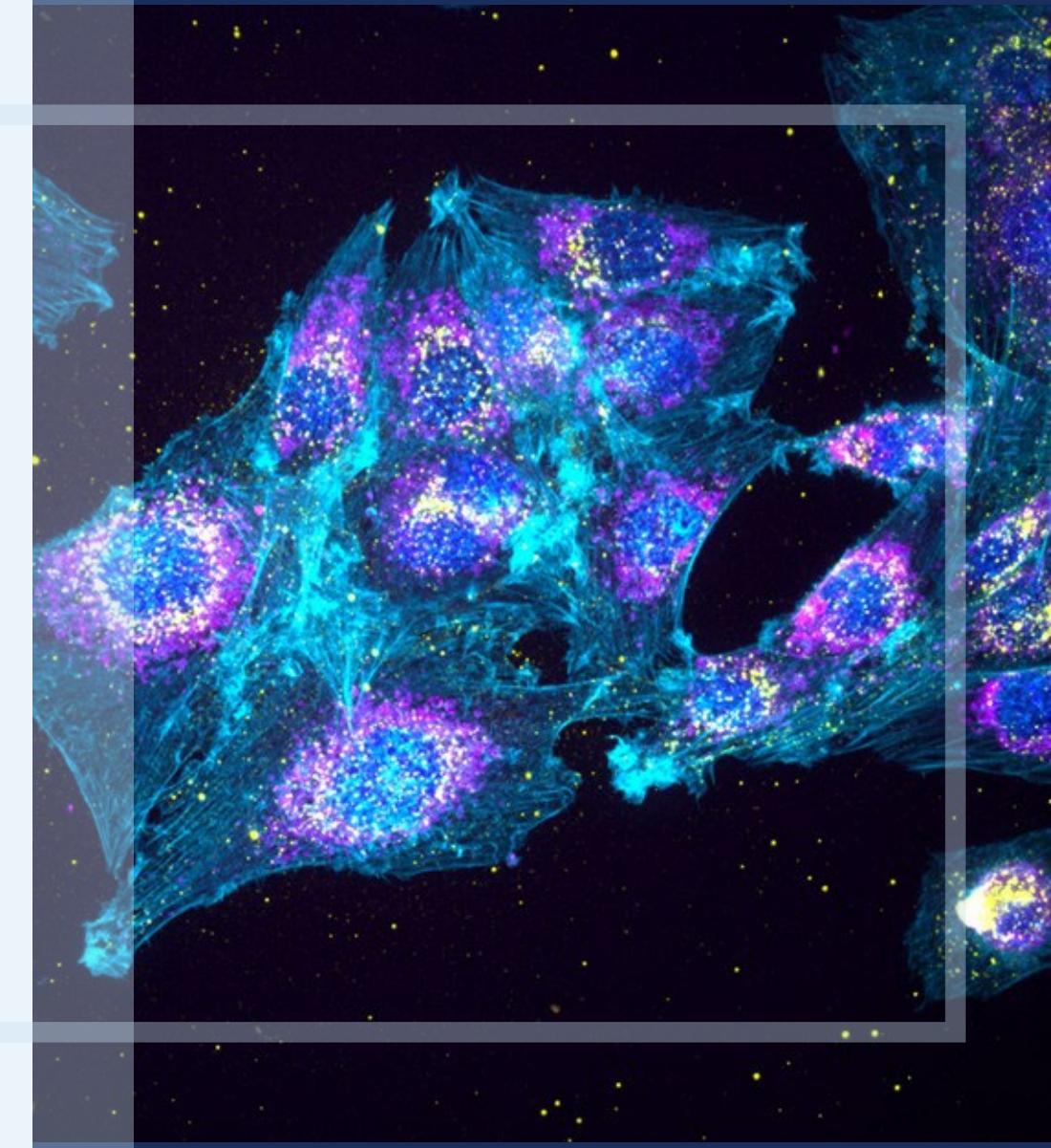
Non-significant
increase (slower)

Non-significant
increase

Significant
decrease

DMX-200

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



Focal Segmental Glomerulosclerosis (FSGS) is a rare disease leading to irreversible kidney damage

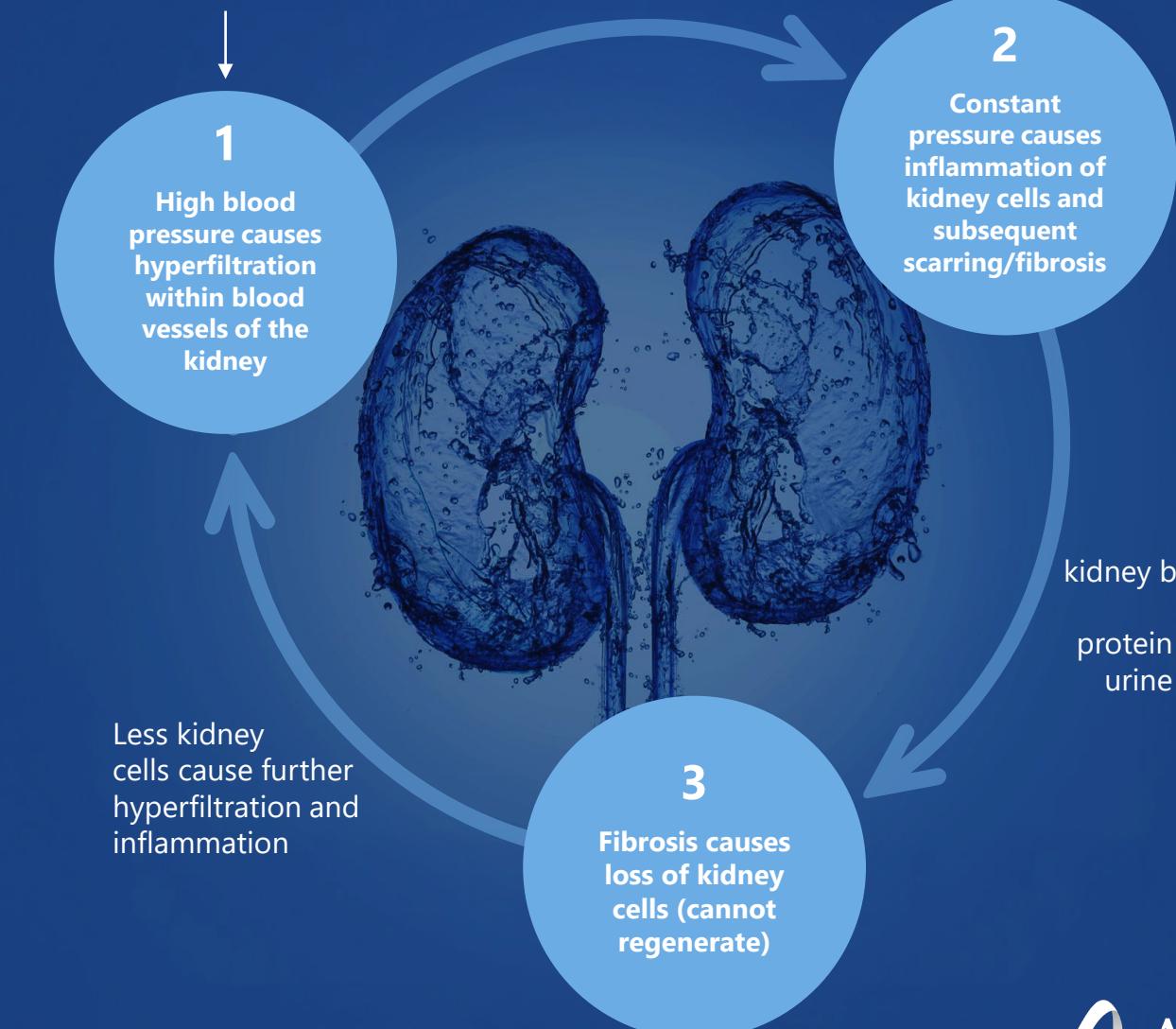
- Irreversible scarring leads to permanent **kidney damage** and eventual **end-stage renal failure**¹
- Symptoms include **proteinuria**, edema, high cholesterol and blood pressure, low albumin levels
- Average time from diagnosis to onset of complete kidney failure is typically **five to ten years**²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

Pathogenic Feedback Loop in FSGS

Existing blood pressure medication targets angiotensin receptor blocker (ARB) lowers blood pressure

Kidney vessels have to work harder under high pressure

DMX-200 specifically blocks kidney inflammatory signaling



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

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

DMX-200 Clinical and Regulatory Progress

Dimerix has built a strong body of evidence and made significant clinical and regulatory progress



MOA

Precision therapy to disrupt the pathogenic monocyte-driven inflammatory feedback loop in the kidney of patients with FSGS



Phase 2

Positive efficacy signals and well-tolerated across studies (n=80), including impacts on proteinuria and inflammation in FSGS study



**ACTION3
Phase 3**

Enrollment well underway (259 of 286 pts to date); Interim analysis (n=72 at 36 wks) showed DMX-200 performing better than placebo in reducing proteinuria¹



**FDA and
Project PARASOL**

Alignment on proteinuria as a primary endpoint for approval



**ACTION3 Part 2
Interim Analysis**

Expected after planned follow-up meeting with FDA

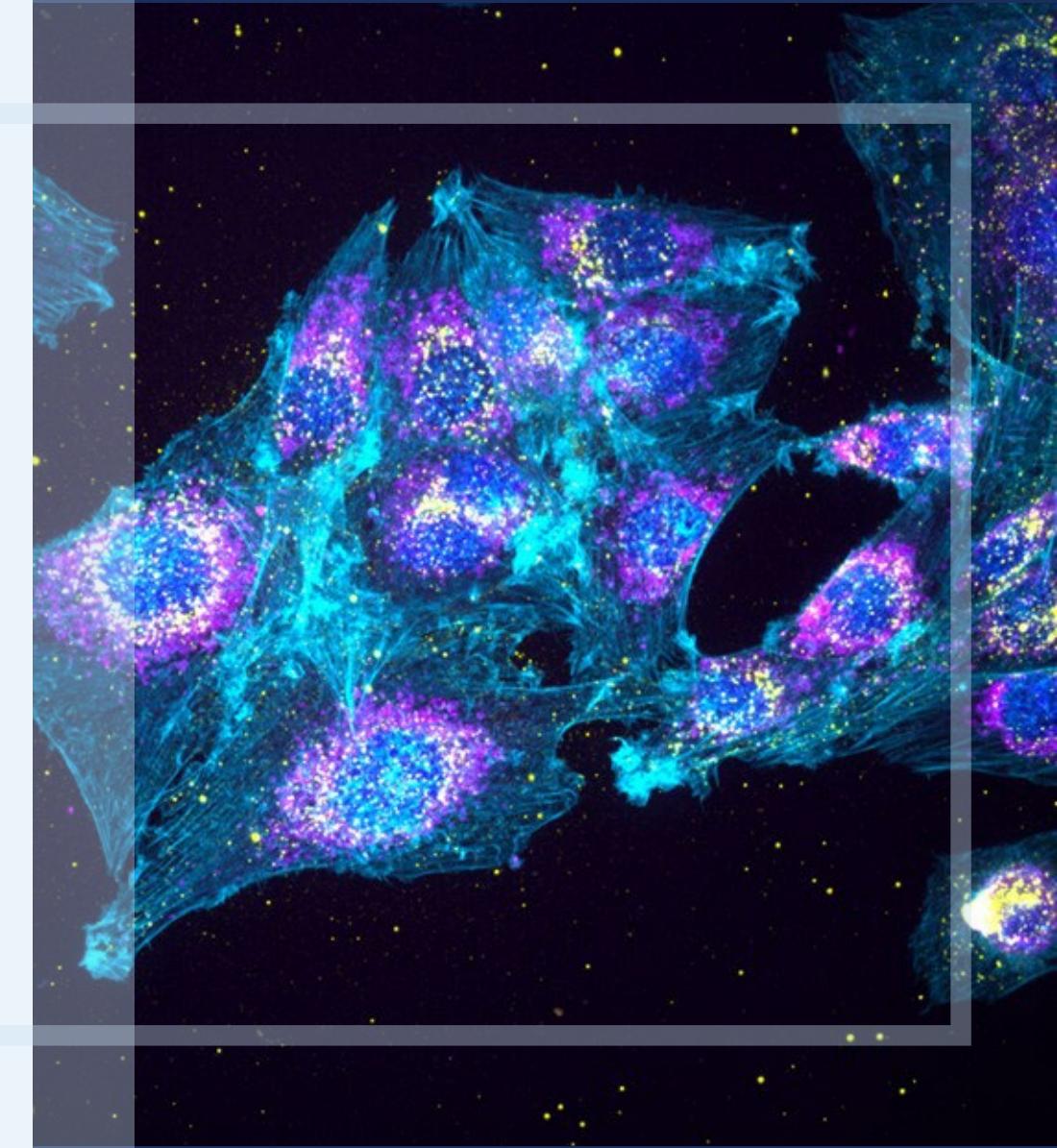


**ACTION3 Part 3
Final Analysis**

2-year proteinuria (primary) and eGFR (secondary) data serves as basis for Full Approval (n=286)

Corporate Outlook

Delivering on our mission for patients
and shareholders



Q3 2025 Select Financial Results

Q3 2025 revenue of \$169M, up 17% at CER and non-GAAP net income of \$54.2M

<i>(in thousands, except per share data)</i>	Q3'25		YTD'25	
	Sep. 30, 2025	Sep. 30, 2024	Sep. 30, 2025	Sep. 30, 2024
Net product sales	\$ 169,061	\$ 141,517	\$ 448,998	\$ 378,589
Cost of goods sold	19,467	13,279	46,382	38,107
GAAP operating expenses	115,325	106,579	385,773	331,577
Non-GAAP operating expenses	95,362	82,577	317,688	250,195
GAAP net income (loss)	17,306	(6,729)	(28,800)	(70,845)
Non-GAAP net income	54,237	30,786	65,133	44,692
GAAP net income (loss) per share – basic and diluted	\$ 0.06	\$ (0.02)	\$ (0.09)	\$ (0.23)
Non-GAAP net income per share – basic	\$ 0.18	\$ 0.10	\$ 0.21	\$ 0.15
Non-GAAP net income per share – diluted	\$ 0.17	\$ 0.10	\$ 0.21	\$ 0.15

QTD September 30, 2025 basic and diluted weighted-average common shares outstanding: 308,468,423 and 310,433,494 respectively; QTD September 30, 2024 basic and diluted weighted-average common shares outstanding: 304,690,596 and 304,690,596 respectively
 YTD September 30, 2025, basic and diluted weighted-average common shares outstanding: 308,139,134 and 308,139,134 respectively; YTD September 30, 2024, basic and diluted weighted-average common shares outstanding: 303,792,479 and 303,792,479 respectively
 Second quarter 2025 operating expenses reflect the previously announced \$30M upfront payment for the U.S. licensing agreement of DMX-200

FY 2025 Financial Guidance Reiterated

FY 2025 Financial Guidance¹

Total Revenue Growth¹	15% to 22%
Galafold Revenue Growth¹	10% to 15%
Pombiliti + Opfolda Revenue Growth¹	50% to 65%
Gross Margin	Mid 80%
Non-GAAP Operating Expense	\$380M to \$400M ²
GAAP Net Income	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 CER¹**
- 2 **Double-digit Galafold® revenue growth of 10-15% at CER¹**
- 3 **Pombiliti® + Opfolda® revenue growth of 50-65% at CER¹**
- 4 **Advance ongoing studies in Fabry, Pompe and FSGS**
- 5 **Deliver positive GAAP net income during H2 2025**

A Rare Company

A unique story in biotech with significant revenue growth and profitability

Two Approved Therapies

One Late Stage Phase 3 Program

Double-digit Revenue Growth

Surpassing \$1B in Total Sales in 2028

Leverageable Rare Disease Infrastructure

Self-Sustainable Company and Growing Free Cash Flow



Appendix



Reconciliation of Non-GAAP Financial Measures

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
	\$ 115,325	\$ 106,579	\$ 385,773	\$ 331,577
Total operating expenses - as reported GAAP				
Research and development:				
Share-based compensation	2,368	4,397	8,765	12,329
Selling, general and administrative:				
Share-based compensation	15,721	14,291	52,055	53,359
Loss on impairment of assets	—	—	1,702	—
Restructuring Charges	—	3,143	—	9,188
Depreciation and amortization	1,874	2,170	5,563	6,506
Total operating expense adjustments to reported GAAP	<u>19,963</u>	<u>24,002</u>	<u>68,085</u>	<u>81,382</u>
Total operating expenses - as adjusted	<u><u>\$ 95,362</u></u>	<u><u>\$ 82,577</u></u>	<u><u>\$ 317,688</u></u>	<u><u>\$ 250,195</u></u>

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 September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
GAAP net income (loss)	\$ 17,306	\$ (6,729)	\$ (28,800)	\$ (70,845)
Share-based compensation	18,089	18,688	60,820	65,688
Depreciation and amortization	1,874	2,170	5,563	6,506
Loss on impairment of assets	—	—	1,702	—
Restructuring charges	—	3,143	—	9,188
Income tax expense	16,968	13,514	25,848	34,155
Non-GAAP net income	<u>\$ 54,237</u>	<u>\$ 30,786</u>	<u>\$ 65,133</u>	<u>\$ 44,692</u>
Non-GAAP net income attributable to common stockholders per common share — basic	\$ 0.18	\$ 0.10	\$ 0.21	\$ 0.15
Non-GAAP net income attributable to common stockholders per common share — diluted	\$ 0.17	\$ 0.10	\$ 0.21	\$ 0.15
Weighted-average common shares outstanding — basic	308,468,423	304,690,596	308,139,134	303,792,479
Weighted-average common shares outstanding — diluted	310,433,494	304,690,596	308,139,134	303,792,479

Exchange Rates

Currency Average Rates

FX Rates	Q3 2024	Q3 2025	Variance
USD/EUR	1.099	1.169	6.4%
USD/GBP	1.301	1.349	3.7%
USD/JPY	0.007	0.007	0.9%

Rare Disease Pipeline

