

AT THE FOREFRONT OF  
THERAPIES FOR RARE DISEASES

# Corporate Overview

December 2024



# Forward-Looking Statements

*This presentation contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to preclinical and clinical development of our product candidates, the timing and reporting of results from preclinical studies and clinical trials, the prospects and timing of the potential regulatory approval of our product candidates, commercialization plans, manufacturing and supply plans, financing plans, 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, with respect to 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 results of clinical or preclinical studies indicate that the product candidates are unsafe or ineffective; the potential that it may be difficult to enroll patients in our clinical trials; 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 preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; the potential that we may not be able to manufacture or supply sufficient clinical or commercial products; and the potential that we will need additional funding to complete all of our studies, 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, 2023, and on Form 10-Q for the quarter ended September 30, 2024, 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 leading biotech company projected to deliver 2024 total revenue growth of 30%-32%<sup>1</sup>



First Oral Precision  
Medicine for  
Fabry Disease

LEVERAGEABLE  
GLOBAL  
COMMERCIAL  
ORGANIZATION



2  
APPROVED  
THERAPIES

World-Class  
Clinical  
Development  
Capabilities

**\$69M-\$71M**

FY 2024  
Pombiliti + Opfolda  
Revenue<sup>1</sup>

~500 EMPLOYEES  
in 20+ Countries



First Two-Component Therapy  
for Pompe Disease

**16-18%**

FY 2024  
Galafold Revenue  
Growth<sup>1</sup>

Guiding to Full  
Year 2024  
Non-GAAP  
Profitability

Combined Peak  
Revenue Potential

**\$1.5B-\$2B**

# 2024 Strategic Priorities

A Transformative  
Year Ahead for  
Amicus

1

Galafold<sup>®</sup> revenue growth of 11-16% at CER<sup>1</sup>, now raised to 16-18%

2

Execute multiple successful launches of Pombiliti<sup>®</sup> + Opfolda<sup>®</sup>

3

Advance ongoing studies to support medical and scientific leadership in Fabry and Pompe diseases

4

Achieve non-GAAP profitability for the full year

# Galafold<sup>®</sup> (*migalastat*)

## Continued Growth

Building a leadership position  
in the treatment of Fabry disease

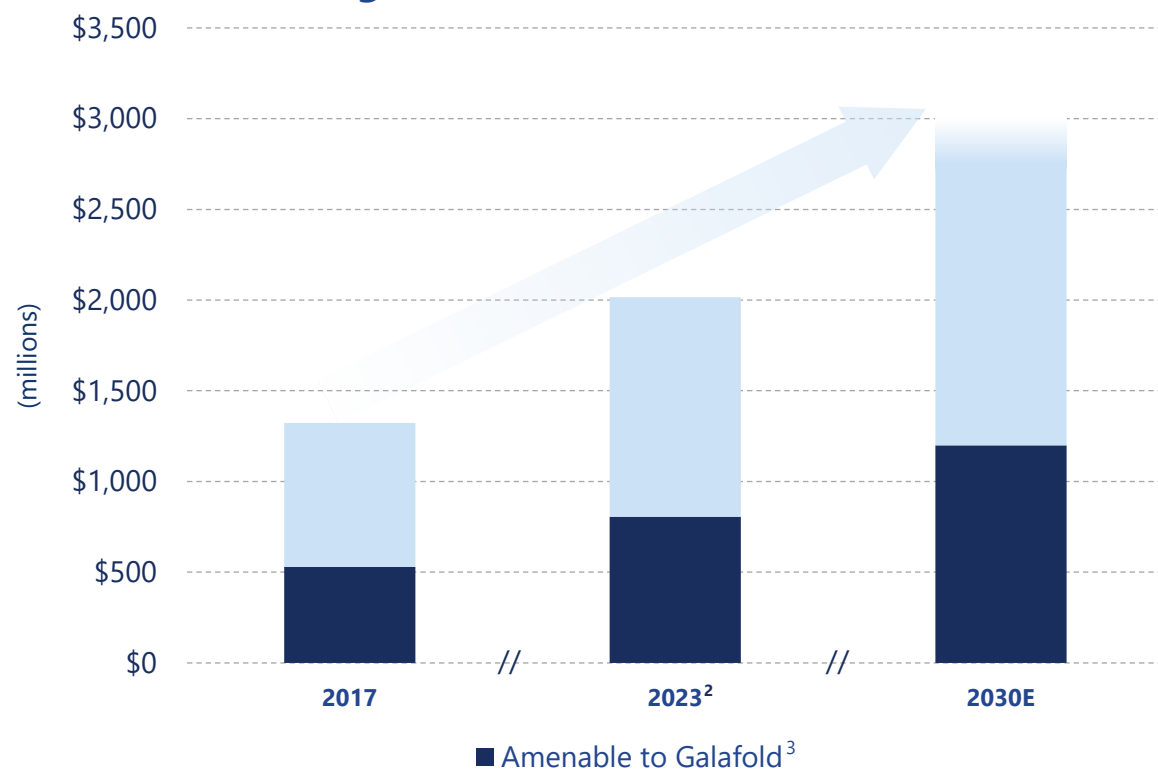




# Global Fabry Market

Fabry market expected to grow to ~\$3B by end of the decade

Global Fabry market of ~\$2B in 2023 and tracking toward ~\$3B+ the end of the decade<sup>1</sup>



- Significantly underdiagnosed
  - Newborn screening studies suggest Fabry is one of the more prevalent rare genetic diseases (~1:1,000 to ~1:4,000 incidence)
- Continued market growth driven by increased diagnosis
- Galafold continues to be the fastest growing Fabry treatment and the greatest contributor to market growth

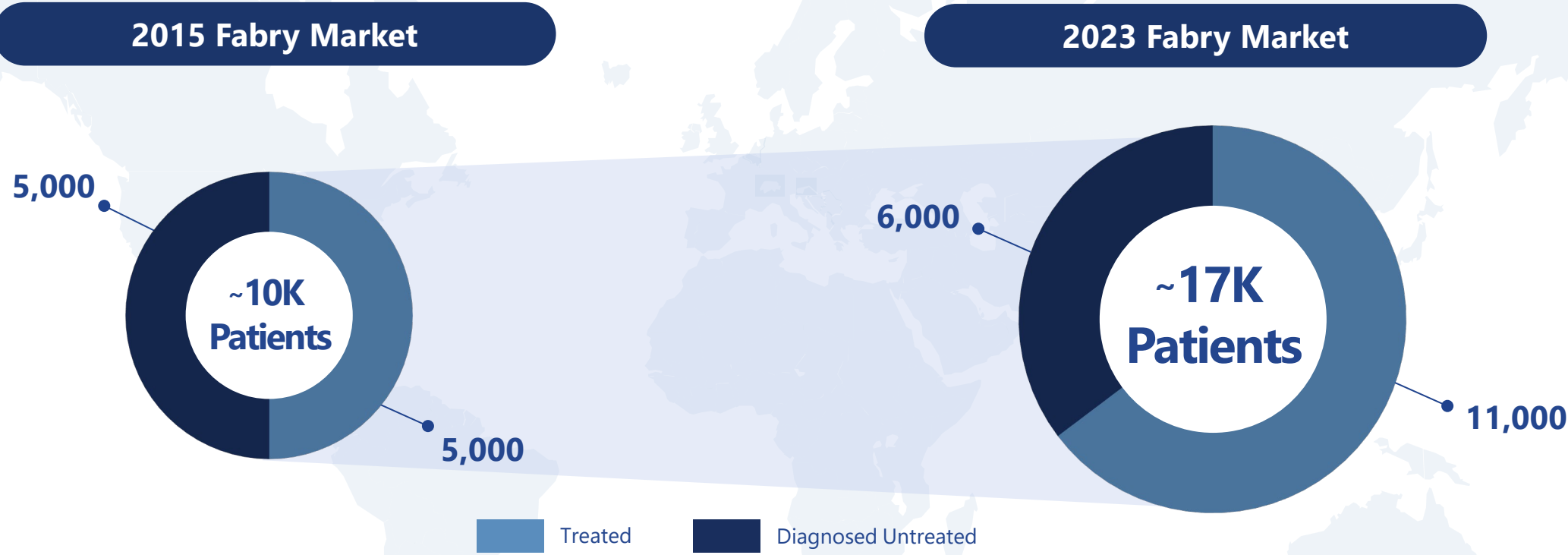
<sup>1</sup> Global market measured by reported sales of approved therapies for Fabry disease – 2030 sales projected using ~7% CAGR

<sup>2</sup> LTM ended September 30, 2023

<sup>3</sup> Assumes ~40% amenability to Galafold

# Fabry Patient Dynamics

Number of people on a Fabry treatment has more than doubled since 2015

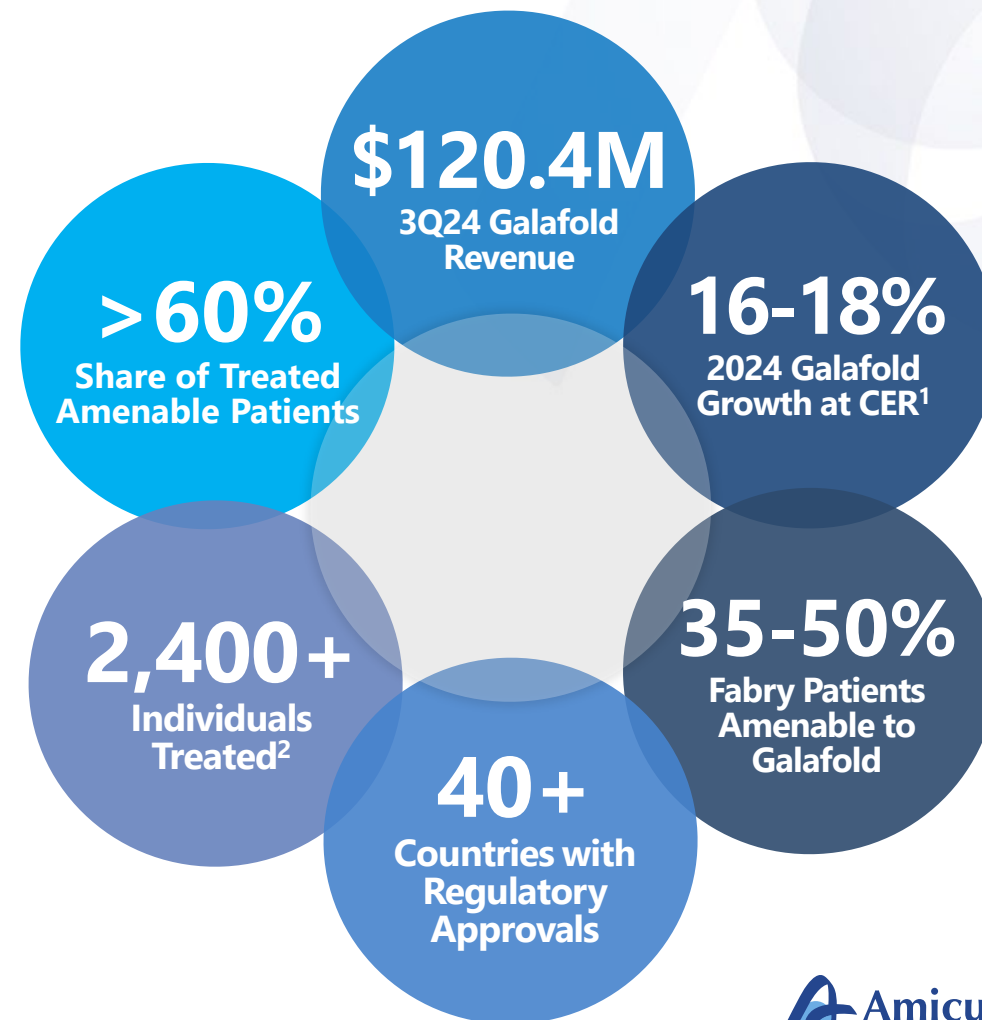
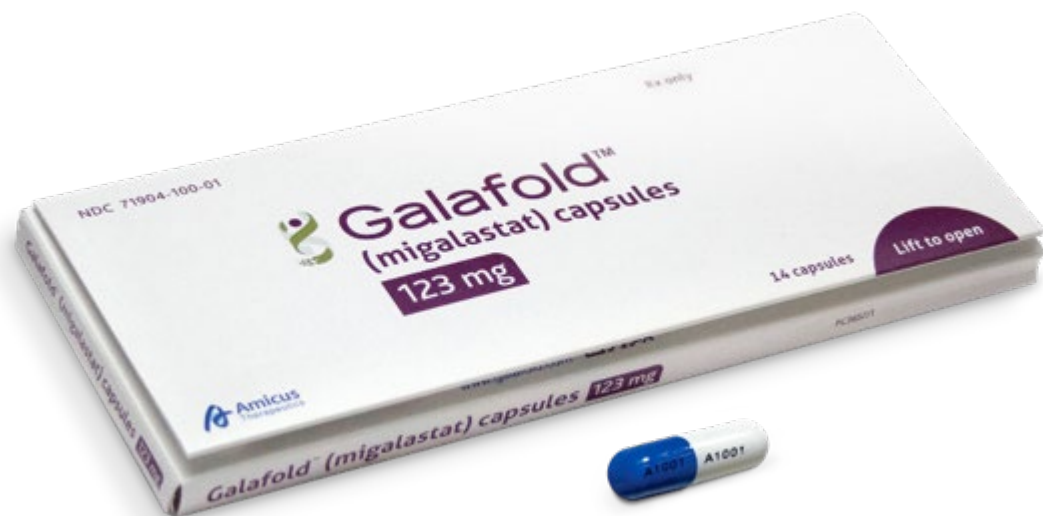


Significant pool of diagnosed untreated patients remain

# 2024 Galafold Success (as of September 30, 2024)

Galafold is the only approved oral treatment option in Fabry disease

A unique mechanism of action for Fabry patients with amenable variants

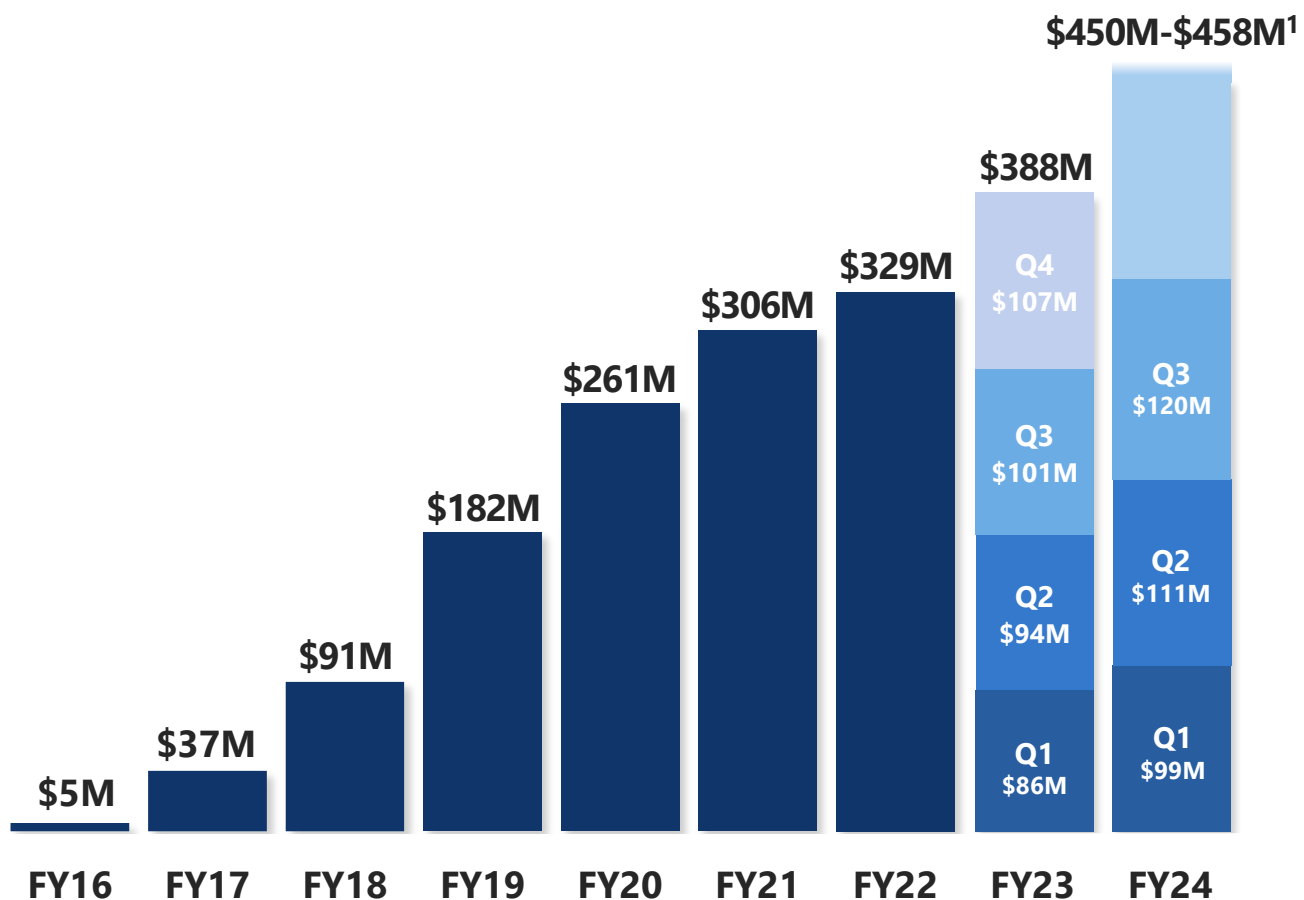


Galafold is indicated for adults with a confirmed diagnosis of Fabry disease and an amenable variant. The most common adverse reactions reported with Galafold ( $\geq 10\%$ ) were headache, nasopharyngitis, urinary tract infection, nausea, and pyrexia. For additional information about Galafold, including the full U.S. Prescribing Information, please visit <https://www.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

Q3 2024 Galafold reported revenue of \$120.4M (+19% growth at CER)



- Global mix of switch (~40%) and previously untreated patients (~60%)<sup>2</sup>
- Expect non-linear quarterly growth to continue due to uneven ordering patterns and FX fluctuations

FY 2024 Galafold growth guidance of 16-18% at CER

# Key Growth Drivers for 2024

Highest patient demand in last four years lays strong foundation for continued double-digit Galafold growth in 2024

- Expanding market through uptake in naïve population as well as geographic and label expansion
- Increasing patient identification through ongoing medical education, screening, and improved diagnostics
- Driving market share of treated amenable patients through excellent execution
- Maintaining >90% adherence and compliance through HCP and patient education and support

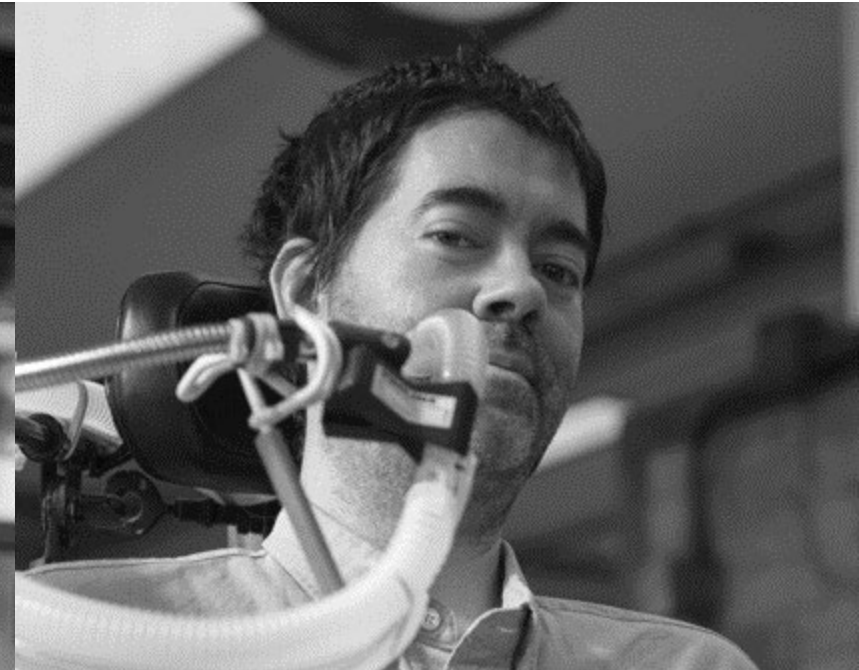
**Pombiliti™** (*cipaglucosidase alfa-atga*)  
+  
**Opfolda™** (*miglustat*)

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





# Late-onset Pompe Disease is a Rare, Inherited Genetic Disorder Caused by Mutation in GAA Gene and Deficiency of $\alpha$ -Glucosidase Enzyme



~5,000-10,000 people diagnosed globally

Deficiency of GAA leading to lysosomal glycogen accumulation and cellular dysfunction

Significantly underdiagnosed

Respiratory failure is major cause of mortality

Significant unmet need

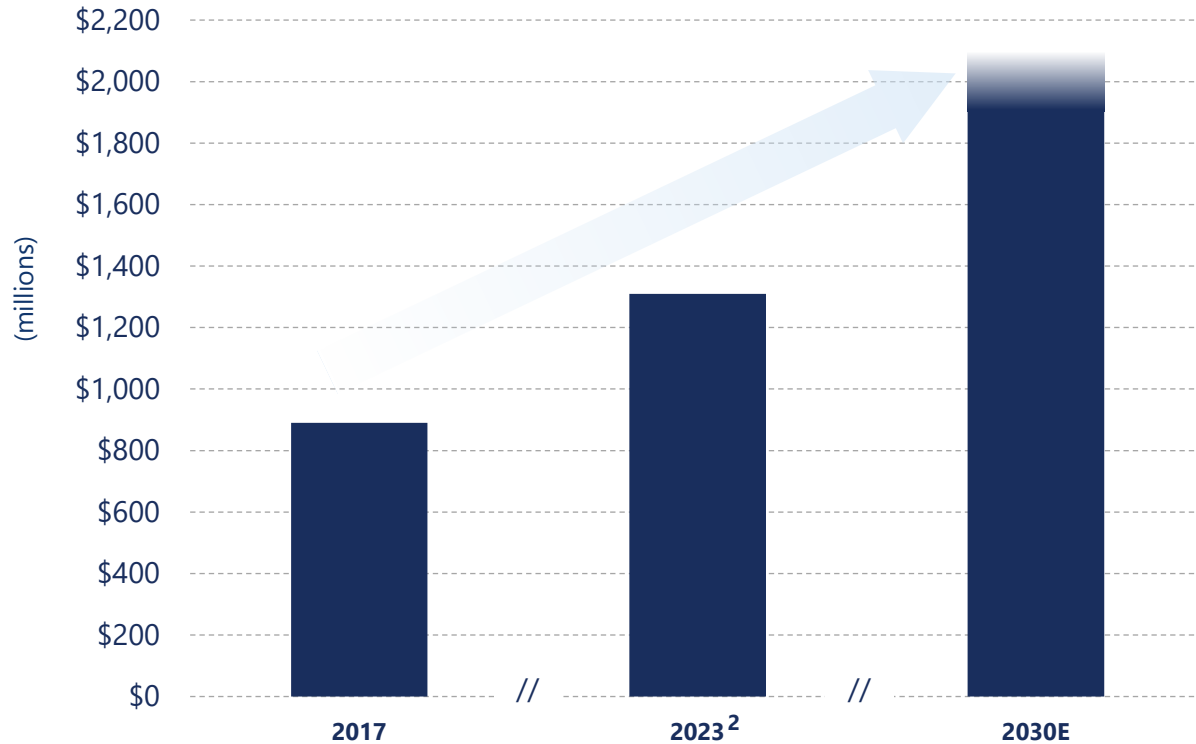
Symptoms include systemic muscle weakness that worsens over time

~\$1.3B+ global Pompe ERT sales<sup>1</sup>

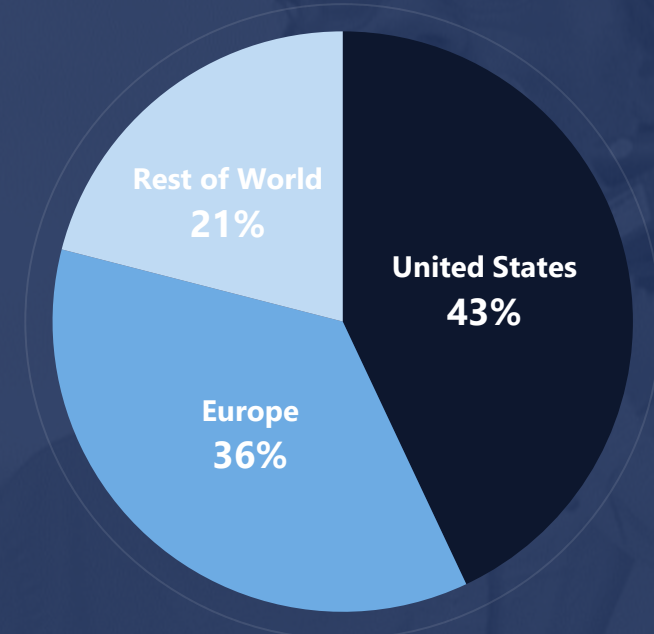
# Global Pompe Market

Global Pompe disease market growth continues to be driven by the diagnosis of new patients

Global Pompe Market of ~\$1.3B in 2023 and Tracking toward \$2B+ by 2030<sup>1</sup>



Global Pompe Market Sales Split YTD 2023<sup>2</sup>



An estimated 3,500-4,000 Pompe patients globally are being treated by ERT<sup>3</sup>

<sup>1</sup> Global market measured by reported sales of approved therapies for Pompe disease – 2029 sales projected using ~8% CAGR

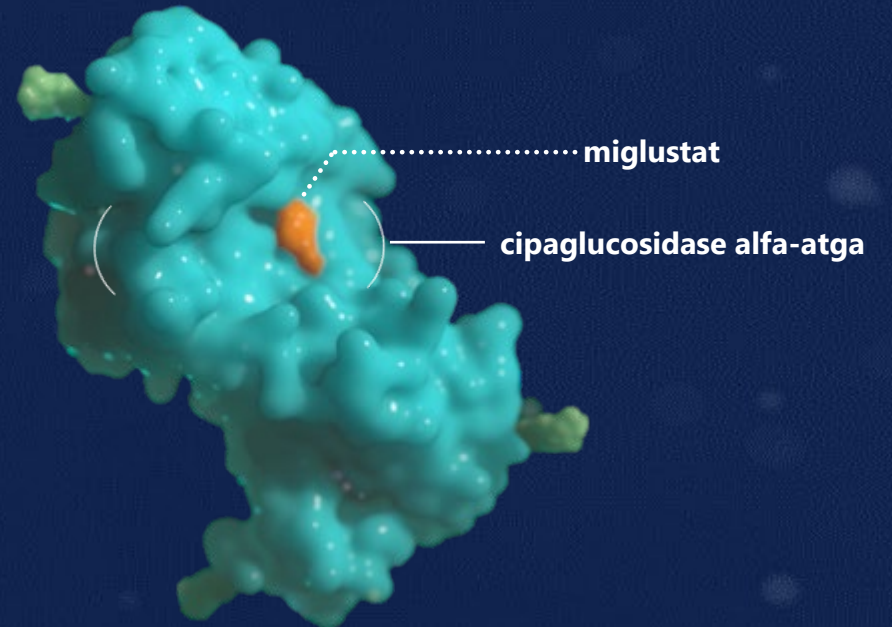
<sup>2</sup> LTM ended September 30, 2023

<sup>3</sup> Amicus Data on File from Market Mapping

# Pombiliti + Opfolda Mechanism of Action

The only two-component therapy for the treatment of Pompe disease

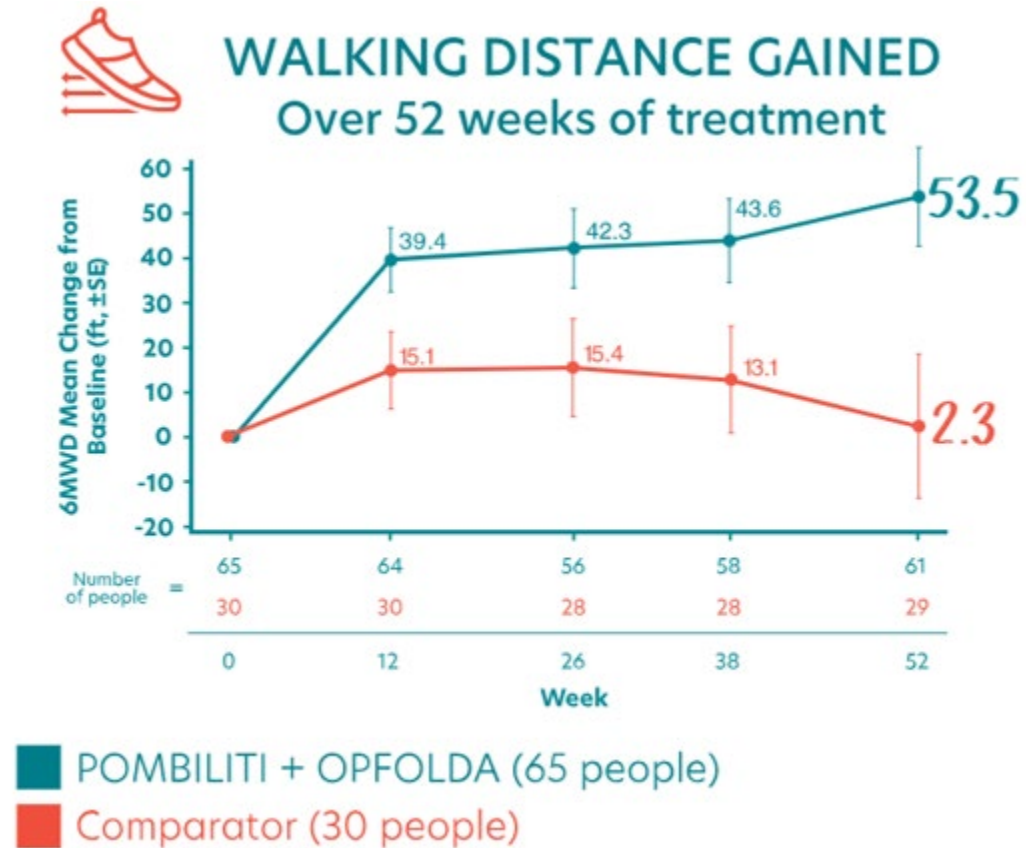
- Pombiliti + Opfolda combines cipaglicosidase alfa-atga, an ERT, with miglustat, an orally administered enzyme stabilizer
- Pombiliti is expressed in a unique cell line producing a naturally glycosylated and highly phosphorylated M6P that can be properly processed to its mature form, which is required for greater lysosomal GAA activity<sup>1</sup>





# PROPEL Phase 3 Data

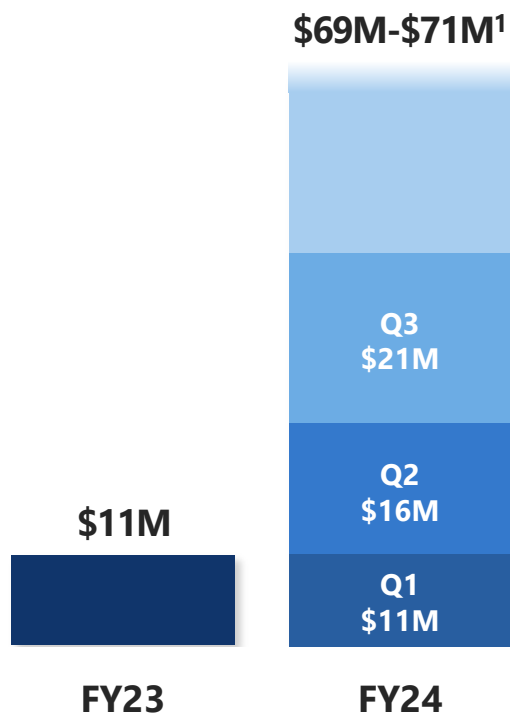
## Resetting expectations for Pompe treatment with Pombiliti + Opfolda – Improvement is Possible





- Pombiliti + Opfolda was evaluated in adults with late-onset Pompe disease (LOPD) in a randomized, controlled clinical study over 52 weeks
- ERT-experienced people were on treatment for an average of 7.4 years before the study
- Pombiliti + Opfolda was shown to improve walking distance and breathing function vs the comparator<sup>1</sup> in ERT-experienced adults

# Pombiliti + Opfolda Performance

Pombiliti + Opfolda continues to build momentum with Q3 2024 revenue of \$21.1M, up +33% from Q2



 **Pombiliti**<sup>®</sup>  
(cipaglicosidase alfa-atga)  
+  
 **Opfolda**<sup>®</sup>  
(miglustat) 65 mg capsules



Guiding to \$69M-\$71M in FY 2024 Pombiliti + Opfolda Revenue at CER

# Successful Global Launch of Pombiliti + Opfolda Underway

Focus in 2024 is on maximizing the number of patients on therapy by year end



## Patient Demand

*As of end of October 2024*

**203** patients have been treated or scheduled to be treated with commercial product

~196 treated patients

Very positive feedback from real-world experience



## KOL Outreach

Increasing depth and breadth of prescribers

Ongoing disease education

Building the body of real-world evidence



## Access and Reimbursement

Positive interactions with global payors

Time through U.S. insurance process improving

Country-by-country reimbursement process underway

Anticipate multiple reimbursement agreements over next 6-9 months



# Regulatory and Clinical Updates

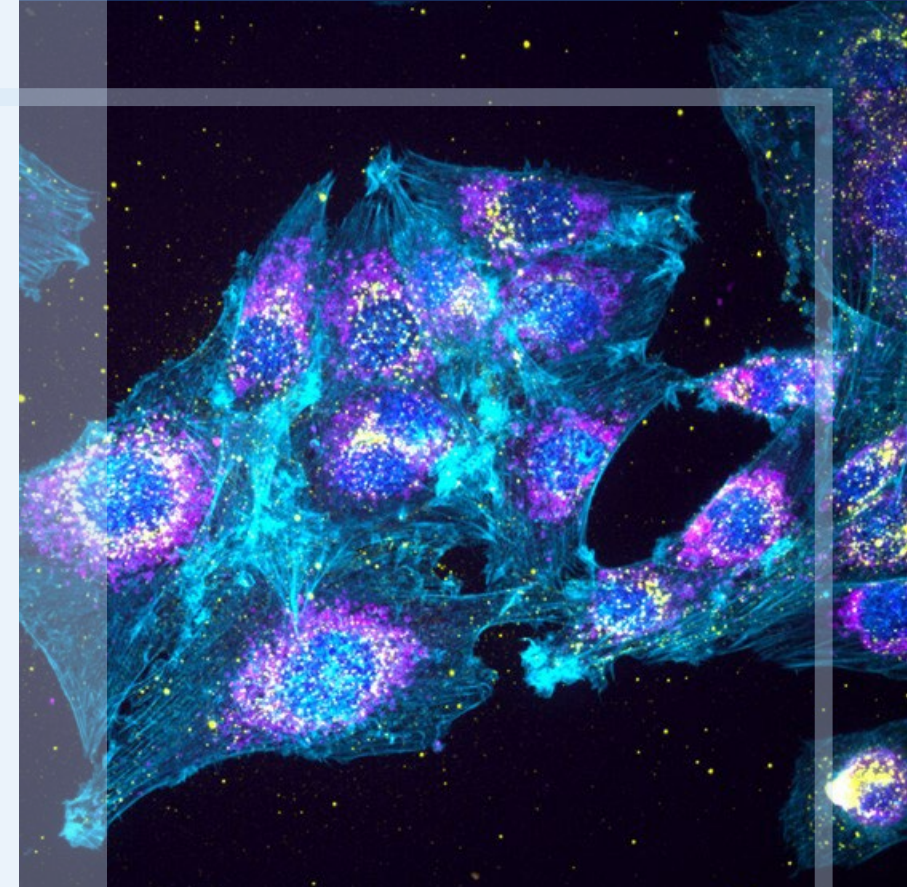
Continuing to build the body of evidence and expand commercial access

- >10 reimbursement dossiers and multiple regulatory submissions throughout 2024
- Japan new drug application (JNDA) submitted to the Ministry of Health, Labor and Welfare (MHLW)
- Ongoing clinical studies in children with late-onset Pompe disease (LOPD) and infantile-onset Pompe disease (IOPD)
- Amicus registry for Pompe disease to continue generating evidence on differentiated MOA and long-term effect



# Corporate Outlook

Delivering on our mission for patients and shareholders



# Q3 2024 Select Financial Results

Q3 2024 revenue of \$141.5M, up 37% and non-GAAP net income of \$30.8M

	Q3'24		YTD'24	
	Sep. 30, 2024	Sep. 30, 2023	Sep. 30, 2024	Sep. 30, 2023
<i>(in thousands, except per share data)</i>				
<b>GAAP net product sales</b>	\$ 141,517	\$ 103,501	\$ 378,589	\$ 284,274
<b>GAAP cost of goods sold</b>	13,279	9,946	38,107	26,002
<b>GAAP operating expenses</b>	106,579	110,578	331,577	331,791
<b>Non-GAAP operating expenses</b>	82,578	89,844	250,195	254,401
<b>GAAP net loss</b>	(6,729)	(21,577)	(70,845)	(117,741)
<b>Non-GAAP net income (loss)</b>	30,786	(3,971)	44,692	(41,051)
<b>GAAP net loss per share</b>	\$ (0.02)	\$ (0.07)	\$ (0.23)	\$ (0.40)
<b>Non-GAAP net income (loss) per share</b>	\$ 0.10	\$ (0.01)	\$ 0.15	\$ (0.16)



# Updated Full-Year 2024 Guidance

	Updated Guidance	Previous Guidance
<b>Total Revenue Growth<sup>1</sup></b>	30% to 32%	26% to 31%
<b>Galafold Revenue Growth<sup>1</sup></b>	16% to 18%	14% to 18%
<b>Pombiliti + Opfolda Revenue<sup>1</sup></b>	\$69M to \$71M	\$62M to \$67M
<b>Non-GAAP Operating Expense</b>	\$340M to \$350M	\$345M to \$360M

**Guiding to full-year 2024 non-GAAP profitability**

# Positioned for Significant Value Creation in 2024

Unlocking the value of two unique commercial therapies in sizeable and growing markets



Accelerating  
total revenue  
growth



Delivering  
full-year  
non-GAAP<sup>1</sup>  
profitability



Clear line of  
sight to  
generating  
positive  
cashflow

# Ultimate Measure of Success: Impacting the Lives of People Living with Rare Diseases



>350 Patients\*

YE17



>2,600 Patients\*

YE23



Many Thousands of Patients\*

2024+



# Appendix



# Appendix I

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
<b>Total operating expenses - as reported GAAP</b>	<b>\$ 106,579</b>	<b>\$ 110,578</b>	<b>\$ 331,577</b>	<b>\$ 331,791</b>
<b>Research and development:</b>				
Stock-based compensation	4,397	4,380	12,329	16,987
<b>Selling, general and administrative:</b>				
Stock-based compensation	14,291	12,131	53,359	50,995
<b>Loss on impairment of assets</b>	—	—	—	1,134
<b>Changes in fair value of contingent consideration payable</b>	—	1,995	—	2,583
<b>Restructuring Charges</b>	3,143	—	9,188	—
<b>Depreciation and amortization</b>	2,170	2,228	6,506	5,691
<b>Total operating expense adjustments to reported GAAP</b>	<b>24,001</b>	<b>20,734</b>	<b>81,382</b>	<b>77,390</b>
<b>Total operating expenses - as adjusted</b>	<b>\$ 82,578</b>	<b>\$ 89,844</b>	<b>\$ 250,195</b>	<b>\$ 254,401</b>

# Appendix II

**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,	
	2024	2023	2024	2023
<b>GAAP net loss</b>	<b>\$ (6,729)</b>	<b>\$ (21,577)</b>	<b>\$ (70,845)</b>	<b>\$ (117,741)</b>
Share-based compensation	18,688	16,511	65,688	67,982
Changes in fair value of contingent consideration payable	—	1,995	—	2,583
Depreciation and amortization	2,170	2,228	6,506	5,691
Loss on impairment of assets	—	—	—	1,134
Restructuring charges	3,143	—	9,188	—
Income tax expense (benefit)	13,514	(3,128)	34,155	(700)
Non-GAAP net income (loss)	<u>\$ 30,786</u>	<u>\$ (3,971)</u>	<u>\$ 44,692</u>	<u>\$ (41,051)</u>
Non-GAAP net income (loss) attributable to common stockholders per common share — basic and diluted	\$ 0.10	\$ (0.01)	\$ 0.15	\$ (0.14)
Weighted-average common shares outstanding — basic and diluted	304,690,596	295,759,435	303,792,479	293,314,167



# Environmental, Social, & Governance (ESG) Snapshot

## Who We Serve

Programs we invest in have 3 key characteristics:

- Address a rare genetic disease
- First-in-class or best-in-class
- Impart meaningful benefit for patients

## Pledge for a Cure

Designate a portion of product revenue back into R&D for that specific disease until there is a cure.

## Pricing PROMISE

Committed to never raising the annual price of our products more than consumer inflation.

## Charitable Giving

(as of December 31, 2023)

Contributions allocated:

**\$1,980,516** U.S.

**\$706,417** Intl.

Expanded Access as of Nov. 2024:

**40** patients / **16** countries

Amicus-supported community programs: **37** Volunteer hours (U.S.): **511**

## Environmental Management

Committed to producing transformative medicines for people living with rare diseases while practicing environmental responsibility and adhering to sustainability best practices in our operations.

*Our mission is to drive sustainability with our partners by incorporating environmental and sustainability principles into all our commercial relationships*

**0%** Amicus-owned Direct Manufacturing and Related Scope 1 and Scope 2 Emissions

(as of December 31, 2023)

Global Employees

**517**

% Female Employees

**58%**

(as of September 30, 2024)

## Board of Directors

Committed to ongoing Board refreshment and diversity of background, gender, skills, and experience:

Director Diversity

3 Female  
1 Veteran Status  
1 African American

**89%**

Board Independence

**56%**

Overall Board Diversity

## Diversity, Equity, & Inclusion (DEI)

Pledge to support a more inclusive culture to impact our employees, our communities, and society.

We have embedded DEI into our business units, our Belief Statement, and Mission-Focused Behaviors

## Employee Recruitment, Engagement, & Retention

Leverage employee capabilities and expertise to provide a culture that drives performance and ultimately attracts, energizes, and retains critical talent.

Amicus is Certified as a **Great Place to Work** in the U.S., U.K., Italy, Germany, Spain, France, and Japan

## Career Development

**90%** Employees say Amicus is a great place to work compared to 57% of employees at a typical U.S.-based company

# FX Sensitivity and Galafold Distribution of Quarterly Sales

## Impact from Foreign Currency Q3 2024:

Currency Variances: USD/	Q3 2023	Q3 2024	YoY Variance
EUR	1.088	1.099	1.0%
GBP	1.266	1.301	2.7%
JPY	0.007	0.007	(2.9%)

## Full-year 2024 Revenue Sensitivity

Given the high proportion of Amicus revenue Ex-US (>60%), a change in exchange rates of +/- 5% compared to year-end 2023 rates could lead to a ~\$15M move in global reported revenues in 2024.

## Distribution of Galafold Revenue by Quarter over Past 5 Years:

	Q1	Q2	Q3	Q4
<b>5 Year Avg.</b>	22%	24%	26%	28%

# Streamlined Rare Disease Pipeline with Focus on Fabry Disease and Pompe Disease Franchises

