

AT THE FOREFRONT OF
THERAPIES FOR RARE DISEASES

Corporate Overview

April 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 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 with >\$500M of sales projected in 2024



First Oral Precision
Medicine for
Fabry Disease

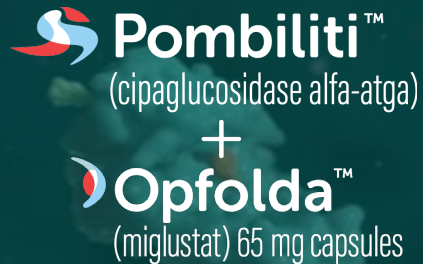
**LEVERAGEABLE
GLOBAL
COMMERCIAL
ORGANIZATION**

**2
APPROVED
THERAPIES**

**World Class
Clinical
Development
Capabilities**

**Non-GAAP
PROFITABILITY
Q4 2023
ACHIEVED**

**>500 EMPLOYEES
in 20+ Countries**



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

\$399M
in 2023 Revenue

21%
Increase Year-Over-Year

**Expect Full Year
2024
Non-GAAP
Profitability**

**Combined Peak
Revenue Potential
\$1.5B – \$2B**

2024 Strategic Priorities

A Transformative
Year Ahead for
Amicus

1

Drive double-digit Galafold[®] revenue growth of 11-16% at CER¹

2

Execute multiple successful launches of Pombiliti[™] + Opfoda[™]

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[®] *(migalastat)*

Continued Growth

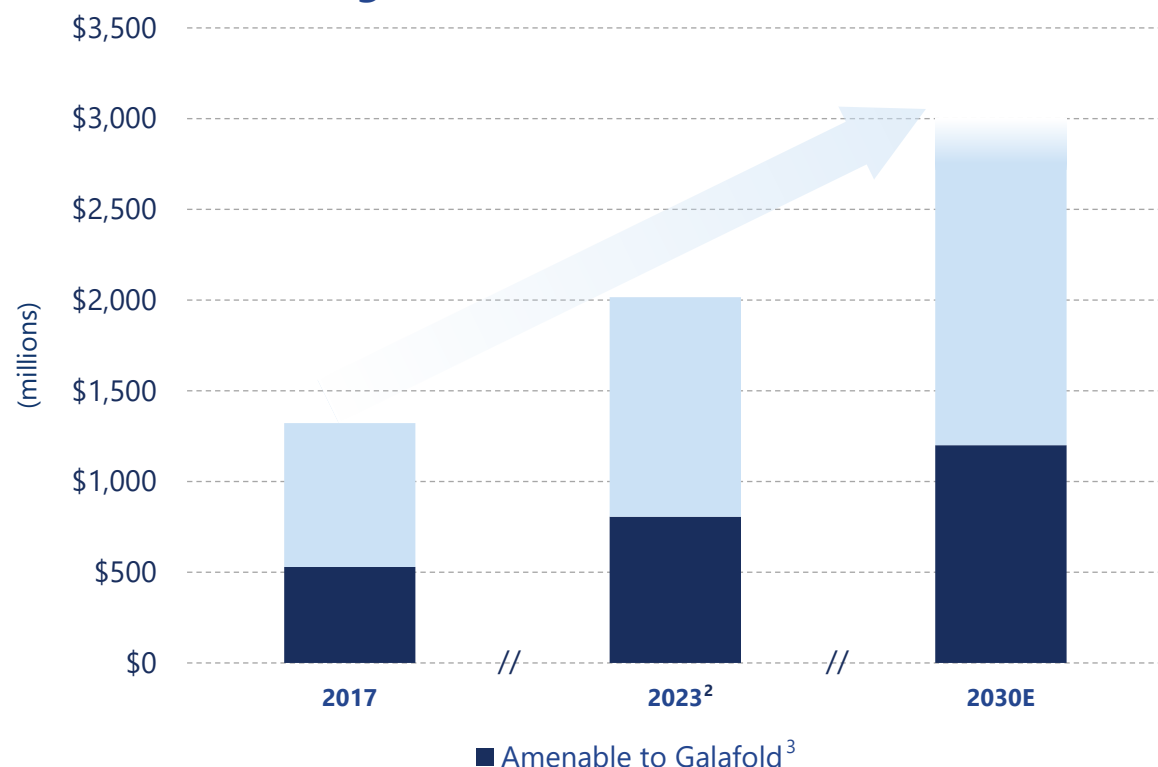
Building a leadership position in the treatment of Fabry disease



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 decade¹

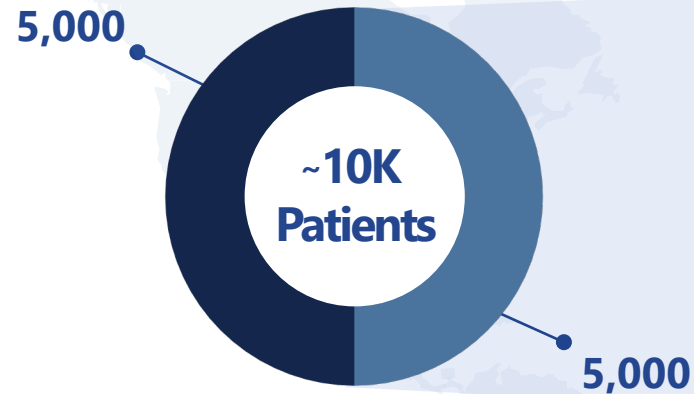


- Significantly underdiagnosed
 - Newborn screening studies suggest Fabry is one of the more prevalent rare genetic diseases (~1:1,000 to ~1:4,000 incidence)
- Continued market growth driven by increased diagnosis
- Galafold continues to be the fastest growing Fabry treatment and the greatest contributor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

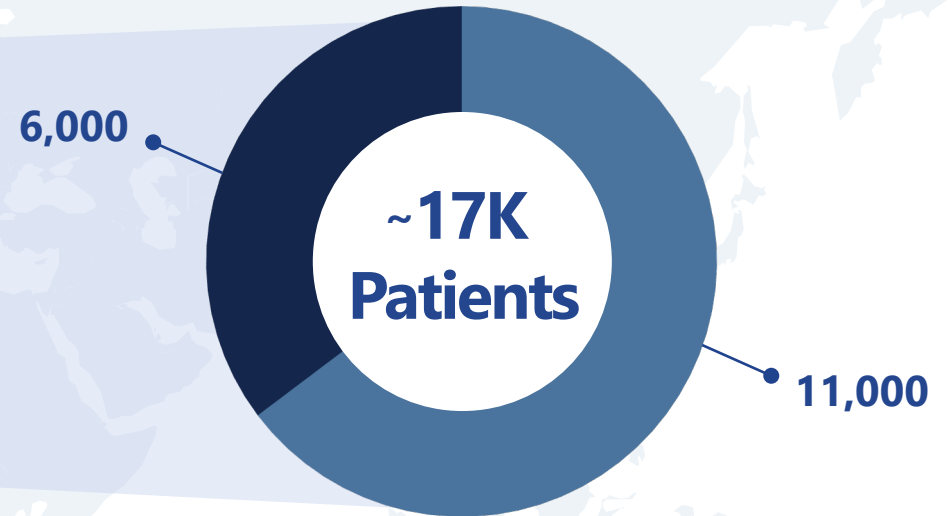
Fabry Patient Dynamics

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

2015 Fabry Market



2023 Fabry Market



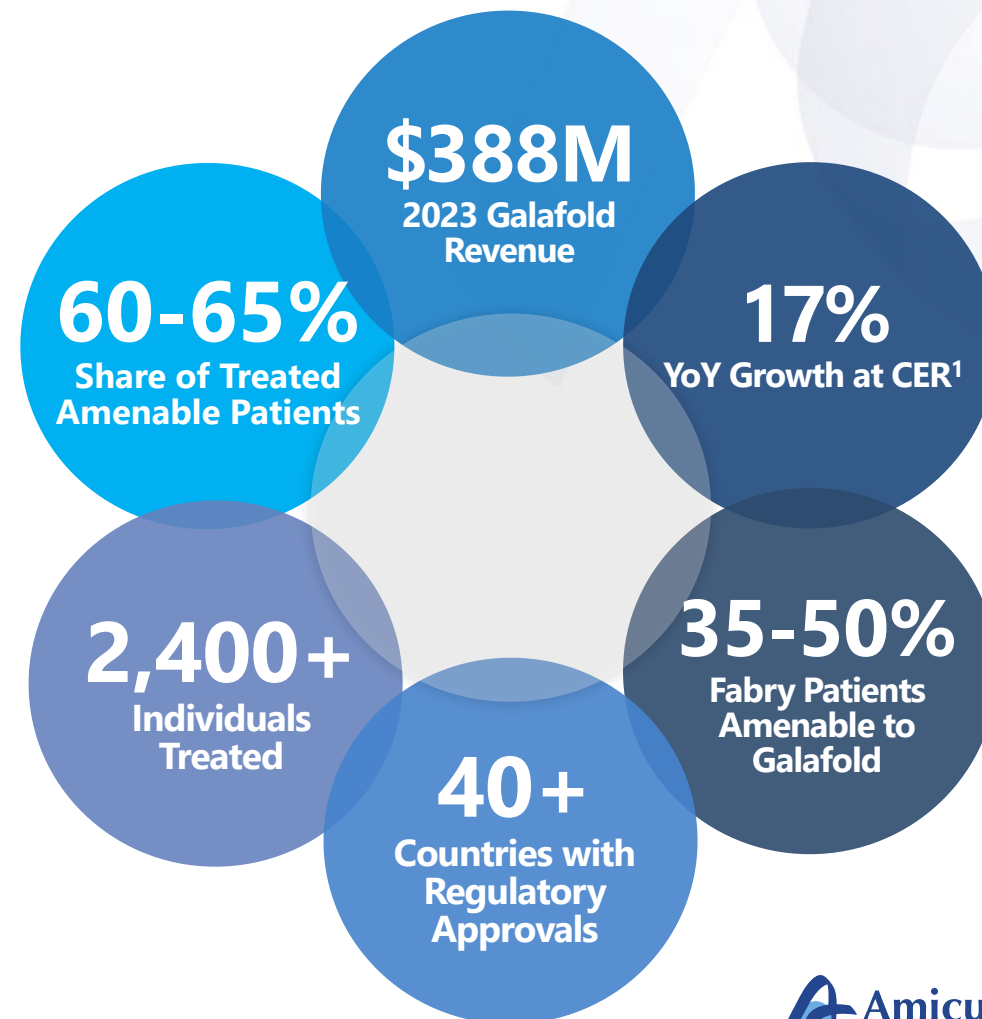
Treated Diagnosed Untreated

Significant pool of diagnosed untreated patients remain

2023 Galafold Success (as of December 31, 2023)

Galafold is the only approved oral treatment option in Fabry disease

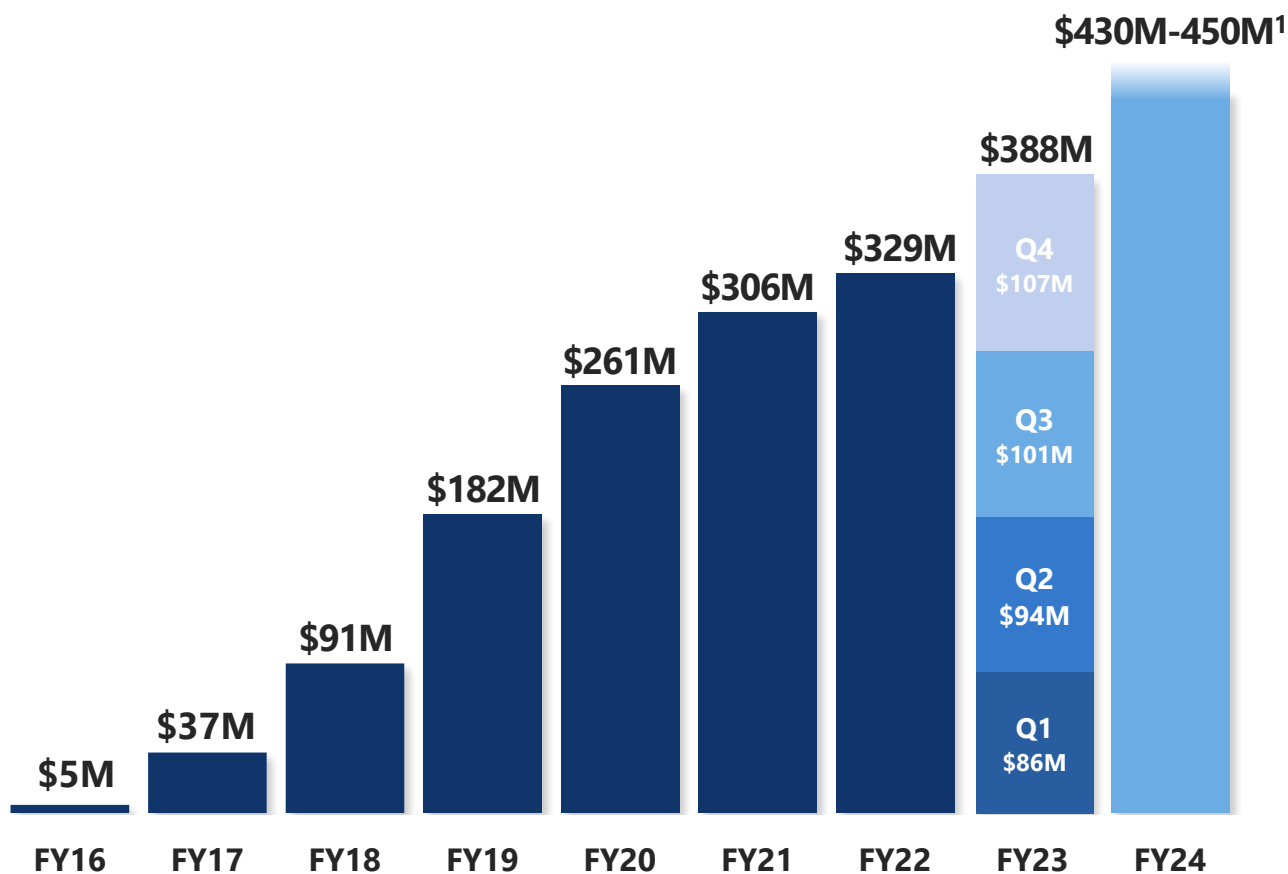
A unique mechanism of action for Fabry patients with amenable variants



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.

Galafold Performance

Galafold YTD reported revenue growth of +18% to \$388M



- Global mix of switch (~43%) and previously untreated patients (~57%)²
- Expect non-linear quarterly growth to continue due to uneven ordering patterns and FX fluctuations

Distribution of Galafold revenue by quarter over previous 5 years:

	Q1	Q2	Q3	Q4
5 Year Avg.	22%	24%	26%	28%

FY24 revenue growth guidance to 11% to 16% at CER

Key Growth Drivers for 2024

Building off a strong year with highest patient demand seen in last four years to lay the groundwork for continued double-digit Galafold growth in 2024

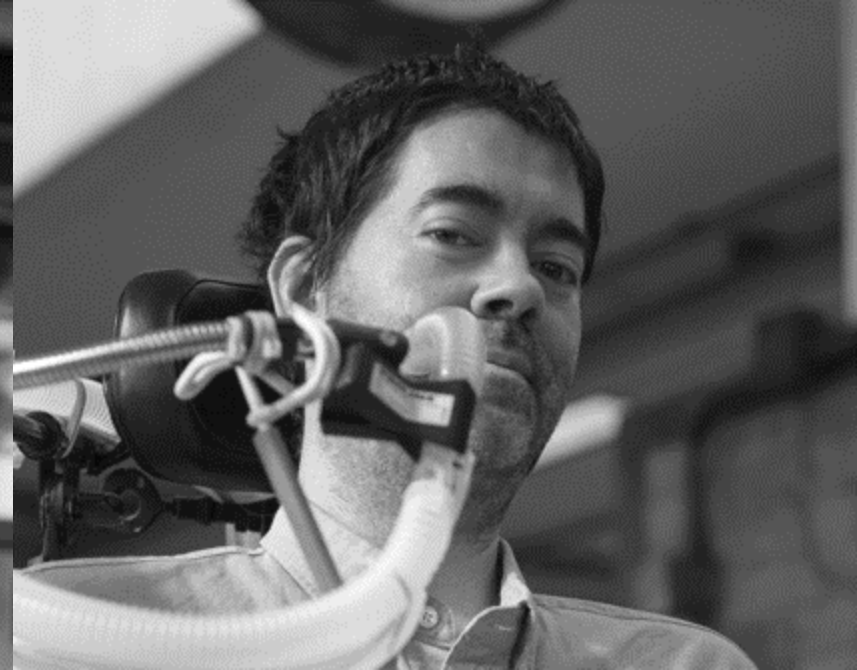
- Increasing patient identification through ongoing medical education, screening, and improved diagnostics
- Driving market share of treated amenable patients through excellent execution
- Expanding market through uptake in naïve population as well as geographic and label expansion
- Maintaining >90%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

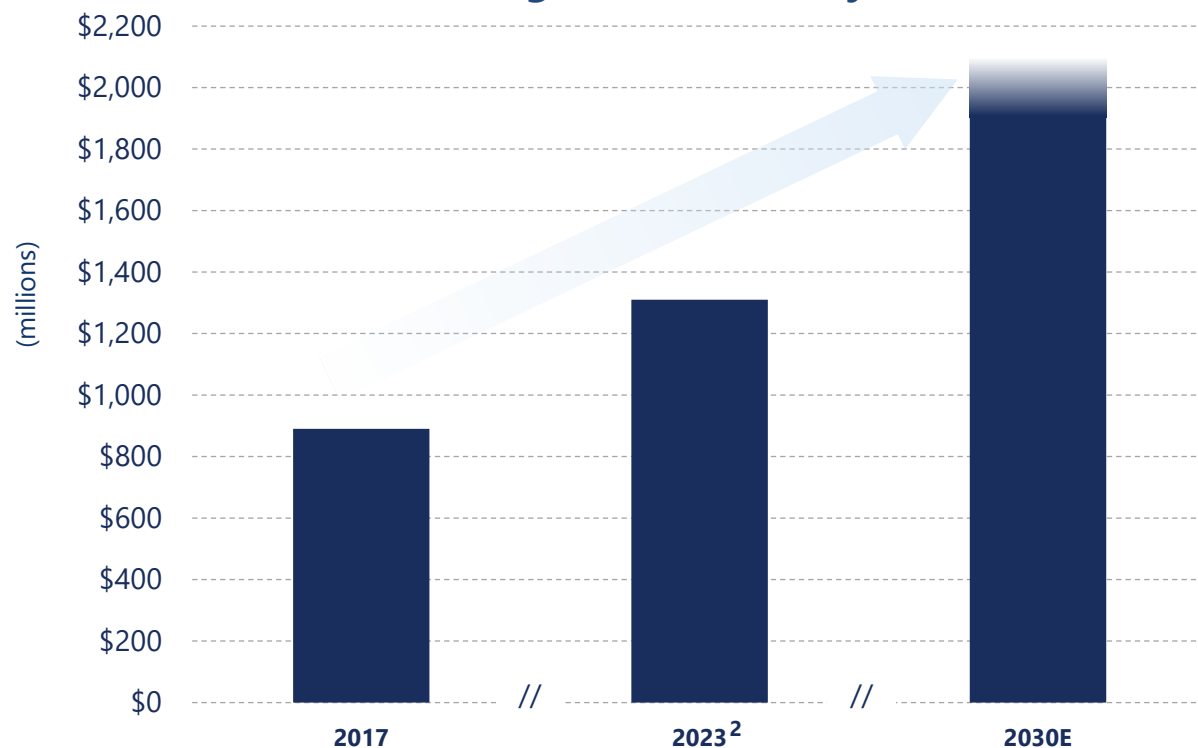
Symptoms include systemic muscle weakness that worsens over time

~\$1.3B+ global Pompe ERT sales¹

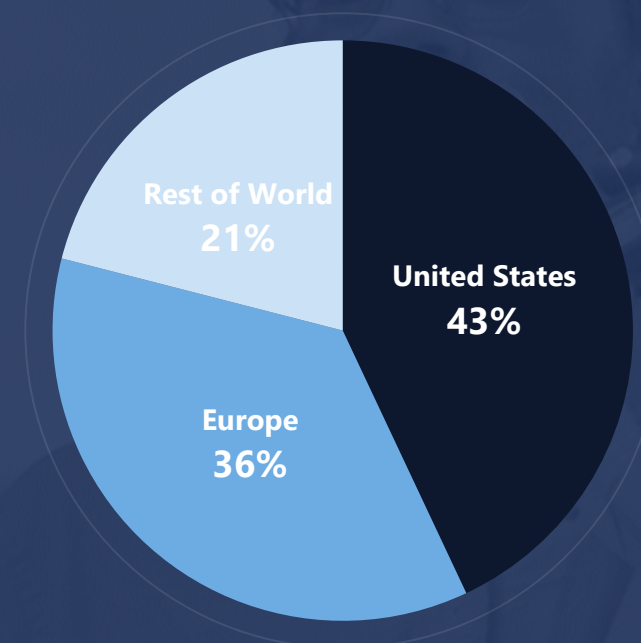
Global Pompe Market

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 2030¹**



**Global Pompe Market Sales Split
YTD 2023²**

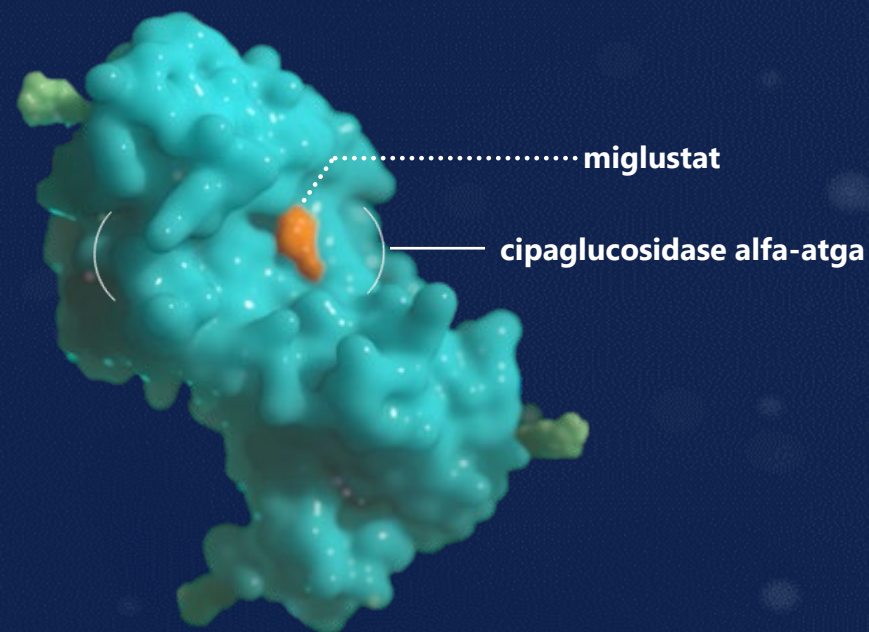


An estimated 3,500-4,000 Pompe patients globally are being treated by ERT³

Pombiliti + Opfolda Mechanism of Action

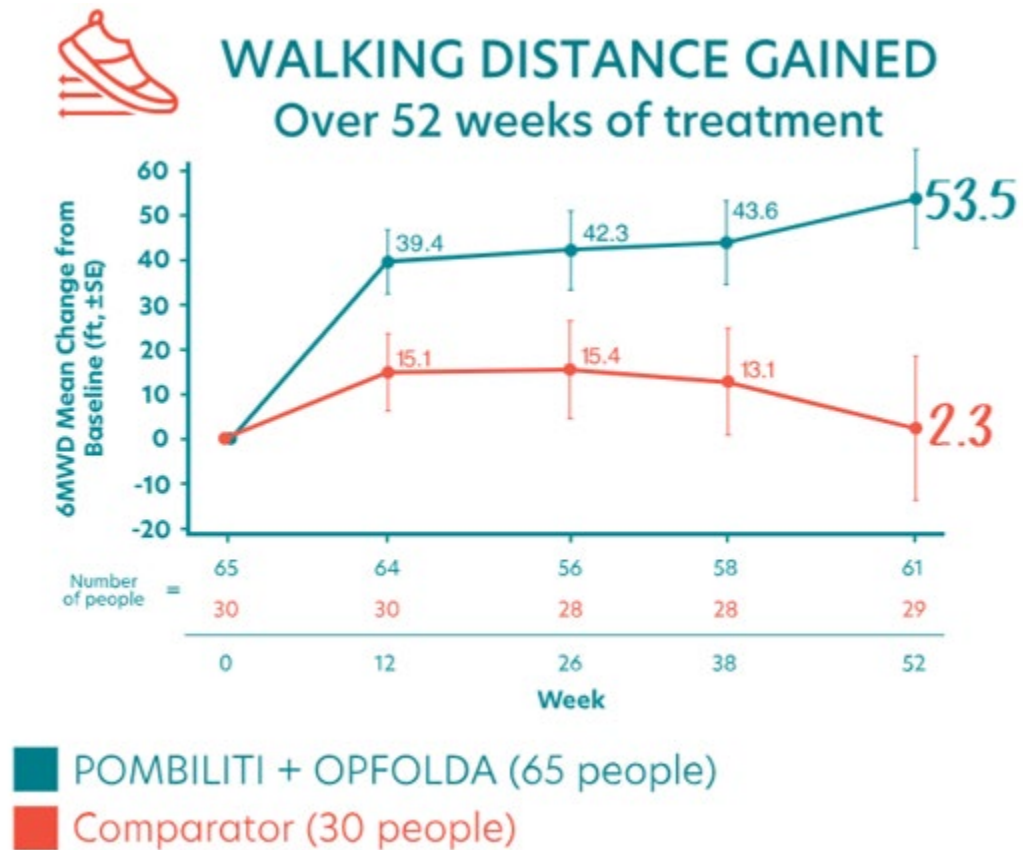
The only two-component therapy for the treatment of Pompe disease

- Pombiliti + Opfolda combines cipaglucosidase 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 activity¹



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 comparator¹ in ERT-experienced adults

Successful Global Launch of Pombiliti + Opfolda Underway

FY 2023 revenue of \$11.6M (\$8.5M in Q4 2023) provides strong foundation for 2024



Patient Demand

As of early January 2024

~**120** patients treated with commercial product or scheduled to be treated

~105 patients from clinical trials and early access

~15 new patients from competitor ERTs or naïve

Very positive early feedback from real-world experience



KOL Outreach

Successfully engaged with top prescribers in each approved country

Existing relationships with HCPs at key treatment centers

Ongoing disease education



Access and Reimbursement

Positive interactions with US, UK, and EU payors

Focus on broad patient access

Country-by-country reimbursement process underway

Multiple launches expected in 2H 2024



 **Pombiliti™**
(cipaglucosidase alfa-atga)

 **Opfolda™**
(miglustat) 65 mg capsules



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

Regulatory and Clinical Updates

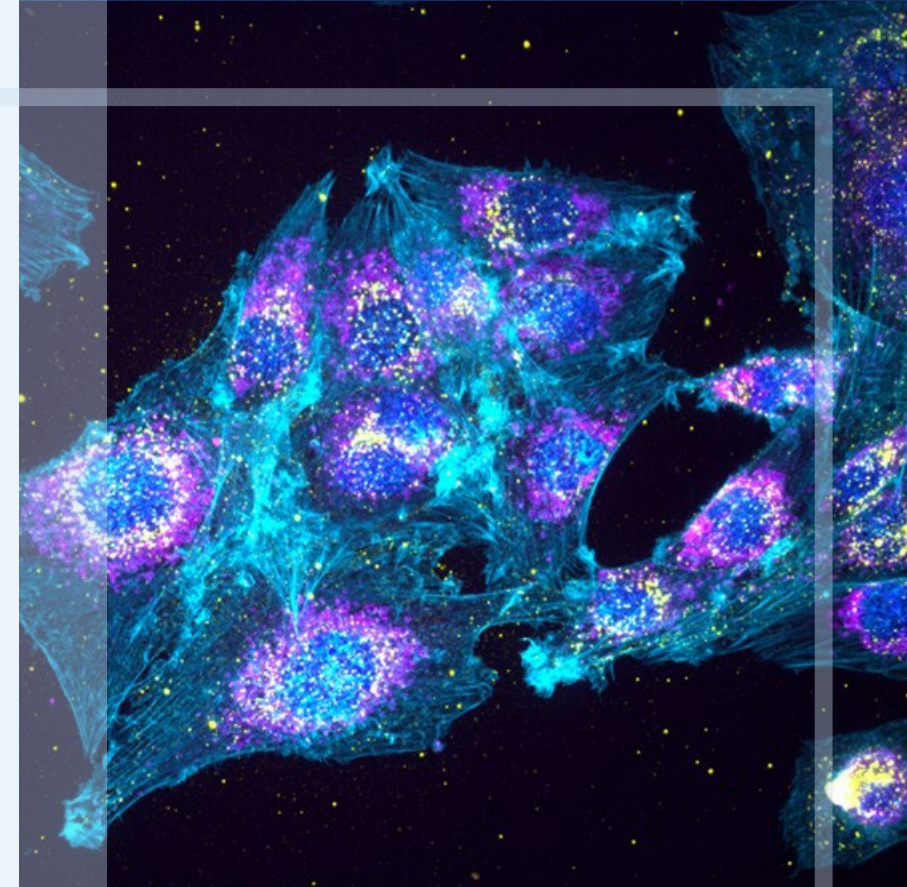
Continuing to build the body of evidence and expand commercial access

- >10 reimbursement dossiers and multiple regulatory submissions throughout 2024
- 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
- Significant presence at *WORLDSymposium™* 2024 with 11 posters and an oral presentation highlighting work in Fabry and Pompe



Corporate Outlook

Delivering on our mission for patients and shareholders



Financial Outlook and Path to Profitability

Clear strategy to build our business, advance our portfolio, and achieve profitability



Sustain Revenue Growth

\$399M FY23 revenue,
+21% YoY growth

>\$500M in total
revenue in FY24



Successfully Launch Pombiliti + Opfolda

Galafold and
Pombiliti + Opfolda
expected to drive
strong **double-digit
growth long term**



Deliver on Financial Goals

Focused on disciplined
expense management

FY24 non-GAAP operating
expense guidance of
\$345M-\$365M

Achieve FY24
non-GAAP profitability¹

Positioned for Significant Value Creation in 2024

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



Accelerating
total revenue
growth

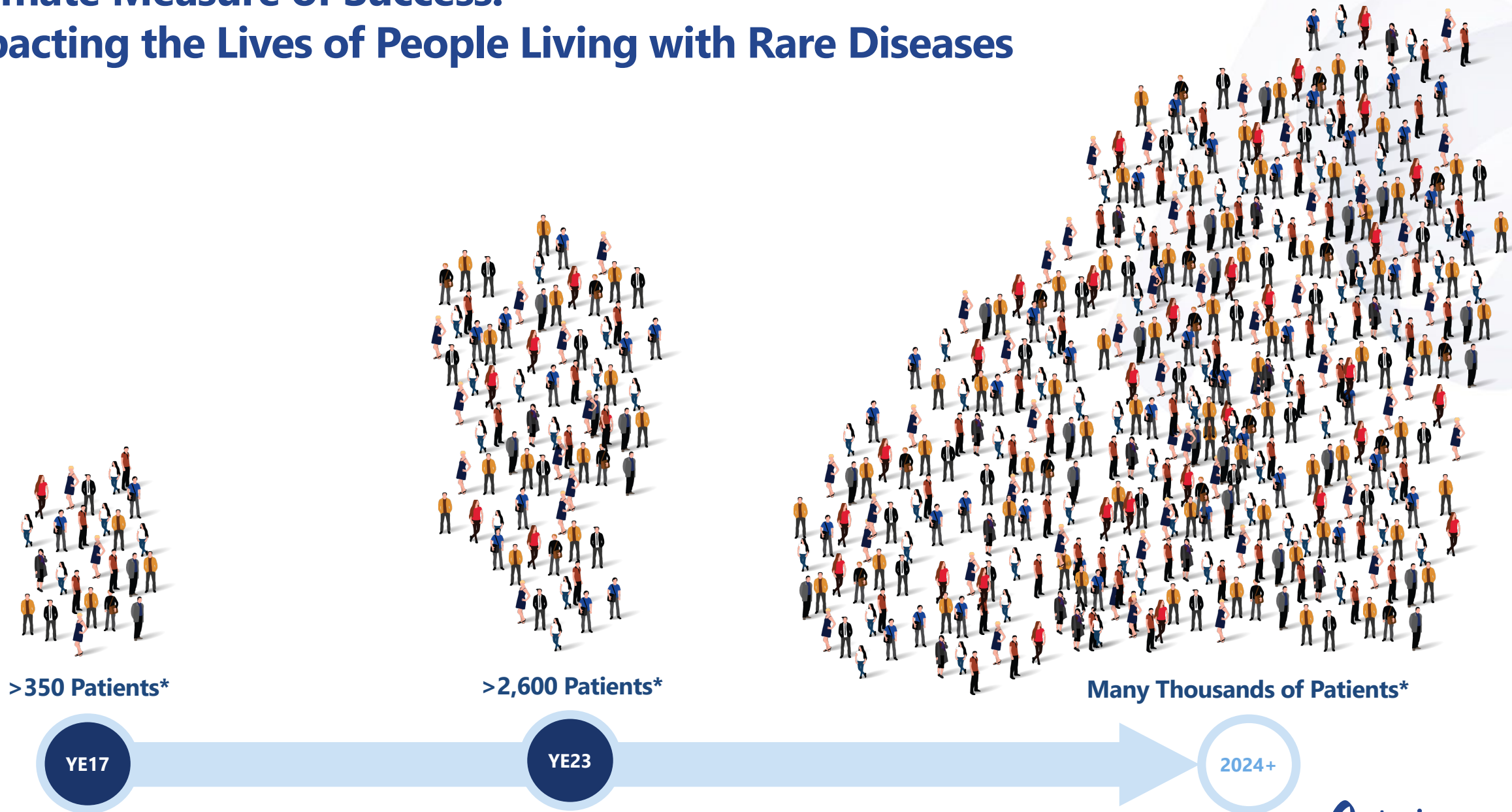


Delivering
full-year
non-GAAP¹
profitability



Clear line of
sight to
generating
positive
cashflow

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



Appendix



Appendix I

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

	Years Ended December 31,		
	2023	2022	2021
Total GAAP operating expenses	\$ 439,241	\$ 502,754	\$ 477,482
Research and development:			
Share-based compensation	21,469	25,089	17,340
Selling, general and administrative:			
Share-based compensation	64,608	51,423	40,498
Loss on impairment of assets	1,134	6,616	—
Changes in fair value of contingent consideration payable	2,583	1,078	6,514
Depreciation and amortization	7,873	5,342	6,209
Total Non-GAAP operating expense adjustments	97,667	89,548	70,561
Total Non-GAAP operating expenses	\$ 341,574	\$ 413,206	\$ 406,921

Appendix II

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

	Three Months Ended December 31,		Years Ended December 31,	
	2023	2022	2023	2022
GAAP net loss	\$ (33,843)	\$ (55,865)	\$ (151,584)	\$ (236,568)
Share-based compensation	18,095	18,626	86,077	76,512
Loss on impairment of assets	—	—	1,134	6,616
Changes in fair value of contingent consideration payable	—	1,584	2,583	1,078
Depreciation and amortization	2,182	1,311	7,873	5,342
Loss on extinguishment of debt	13,933	—	13,933	—
Income tax expense (benefit)	2,183	(14,214)	1,483	(5,471)
Non-GAAP net income (loss)	<u>\$ 2,550</u>	<u>\$ (48,558)</u>	<u>\$ (38,501)</u>	<u>\$ (152,491)</u>
Non-GAAP net income (loss) attributable to common stockholders per common share — basic and diluted	\$ 0.01	\$ (0.17)	\$ (0.13)	\$ (0.53)
Weighted-average common shares outstanding — basic and diluted	300,648,503	289,602,648	295,164,515	289,057,198

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 through Feb 2024:

32 patients / **24** 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

Global Employees

517

% Female Employees

58%

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 Q4 2023:

Currency Variances: USD/	Q4 2022	Q4 2023	YoY Variance
EUR	1.021	1.076	5.4%
GBP	1.174	1.241	5.7%
JPY	0.007	0.007	(4.4%)

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
5 Year Avg.	22%	24%	26%	28%

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

