

AT THE FOREFRONT OF  
THERAPIES FOR RARE DISEASES

# FY23 Results Conference Call & Webcast

February 28, 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

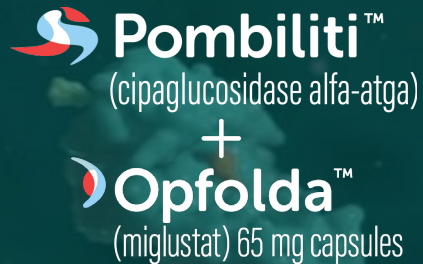


2  
APPROVED  
THERAPIES

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<sup>®</sup> revenue growth of 11-16% at CER<sup>1</sup>

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

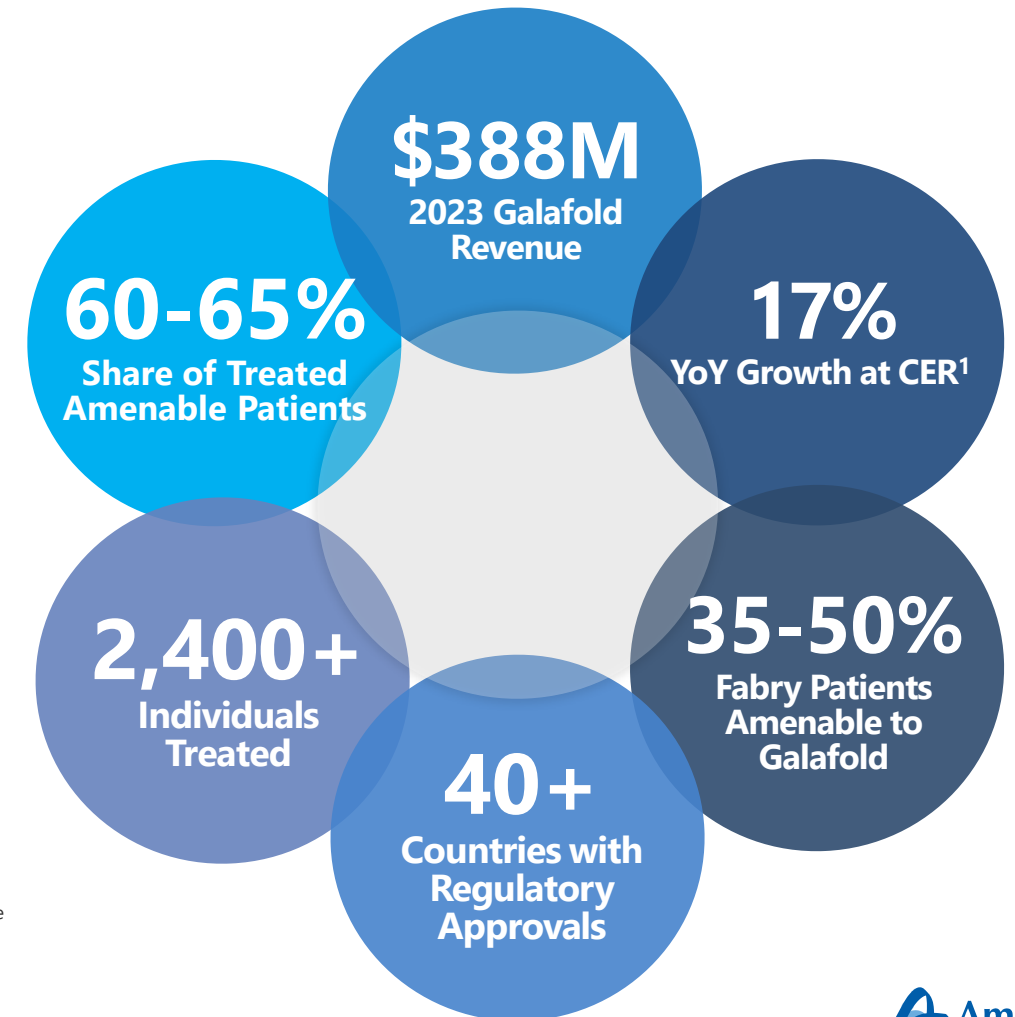
Building a leadership position in the  
treatment of Fabry disease



# 2023 Galafold Success (as of December 31, 2023)

**Galafold is the only approved oral treatment option in Fabry disease**

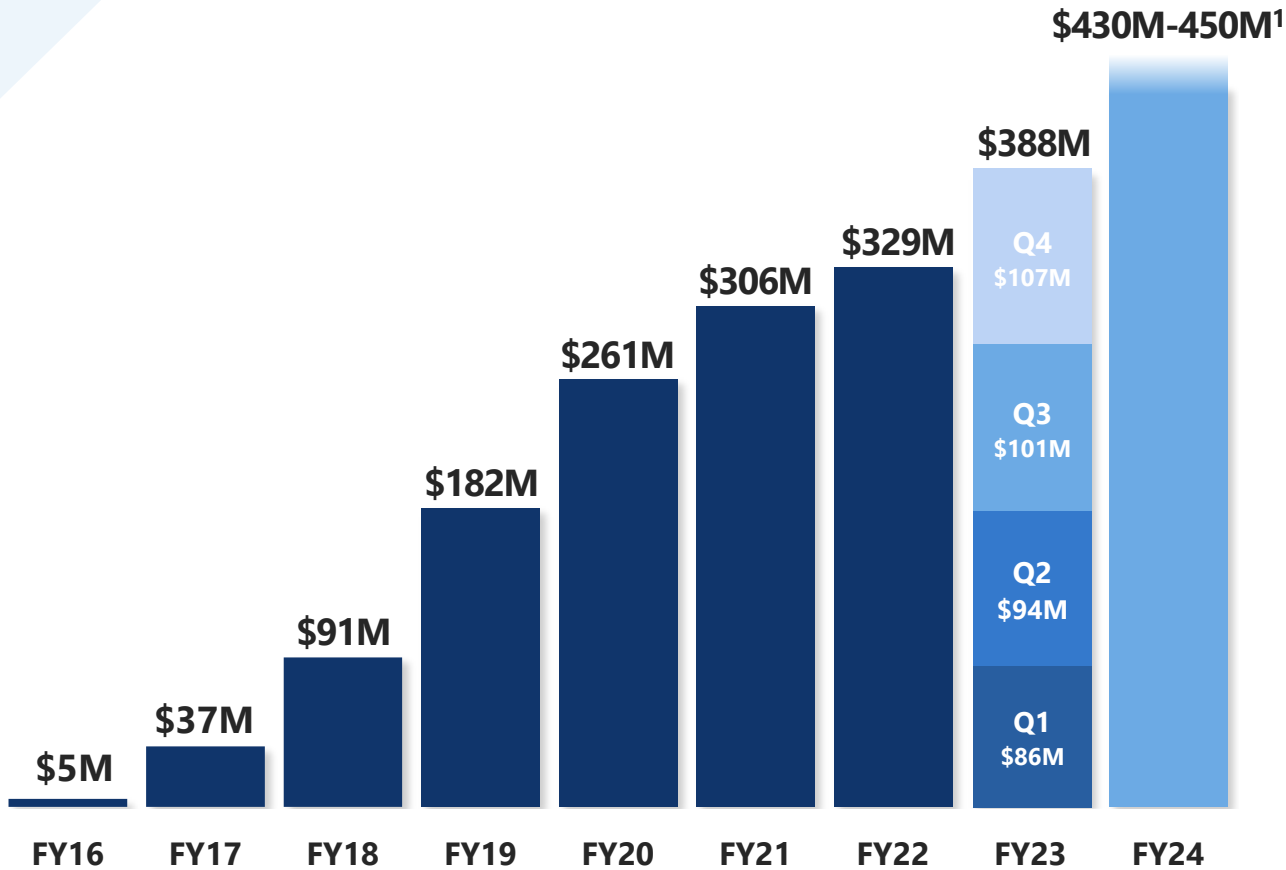
**A unique mechanism of action for Fabry patients with amenable variants**



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](http://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%)<sup>2</sup>
- 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



# 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



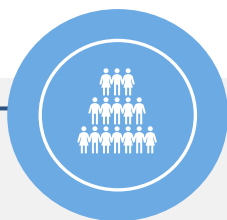
# FY 2023 Select Financial Results

2023 revenue of \$399.4M, up 20% at CER, and net loss significantly reduced

<i>(in thousands, except per share data)</i>	<b>Dec. 31, 2023</b>	<b>Dec. 31, 2022</b>
<b>Product Revenue</b>	\$399,356	\$329,233
<b>Cost of Goods Sold</b>	37,326	38,599
<b>R&amp;D Expense</b>	152,381	276,677
<b>SG&amp;A Expense</b>	275,270	213,041
<b>Changes in Fair Value of Contingent Consideration</b>	2,583	1,078
<b>Loss on Impairment of Assets</b>	1,134	6,616
<b>Depreciation and Amortization</b>	7,873	5,342
<b>Loss from Operations</b>	(77,211)	(212,120)
<b>Interest Income</b>	7,078	3,024
<b>Interest Expense</b>	(50,149)	(37,119)
<b>Loss on Extinguishment of Debt</b>	(13,933)	—
<b>Other (Expense) Income</b>	(15,886)	4,176
<b>Income Tax (Expense) Benefit</b>	(1,483)	5,471
<b>Net Loss</b>	(151,584)	(236,568)
<b>Net Loss Per Share</b>	(0.51)	(0.82)

# 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 profitability<sup>1</sup>

# 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



# Appendix





# Appendix I

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

	Years Ended December 31,		
	2023	2022	2021
<b>Total GAAP operating expenses</b>	<b>\$ 439,241</b>	<b>\$ 502,754</b>	<b>\$ 477,482</b>
<b>Research and development:</b>			
Share-based compensation	21,469	25,089	17,340
<b>Selling, general and administrative:</b>			
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
<b>Total Non-GAAP operating expense adjustments</b>	<b>97,667</b>	<b>89,548</b>	<b>70,561</b>
<b>Total Non-GAAP operating expenses</b>	<b>\$ 341,574</b>	<b>\$ 413,206</b>	<b>\$ 406,921</b>

# Appendix II

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

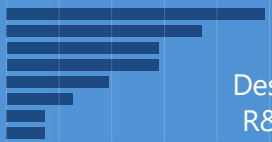
	Three Months Ended December 31,		Years Ended December 31,	
	2023	2022	2023	2022
<b>GAAP net loss</b>	\$ (33,843)	\$ (55,865)	\$ (151,584)	\$ (236,568)
Share-based compensation	18,095	18,626	86,077	76,512
Loss on impairment of assets	—	—	1,134	6,616
Changes in fair value of contingent consideration payable	—	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

Contributions allocated:

**\$2,288,998** U.S.

**\$954,349** Intl.

Expanded Access through Jan 2024:

**32** patients / **24** countries

Amicus-supported community programs:

**22**

Volunteer hours (U.S.):

**580**

## 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 GHG 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
- 2 Veteran Status
- 1 African American

**80%** Board Independence

**60%** Overall Board Diversity

## Diversity, Equity, & Inclusion (DEI)

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

Goal of maintaining gender diversity and increasing overall diversity throughout our global workforce.

## Employee Recruitment, Engagement, & Retention

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

*Pulse surveys reveal employees feel **high personal satisfaction** in their job, are **proud of their work** and what they contribute to the community*

## Career Development

Reimagined performance management process to measure the *what* and the *how*, rewarding those who role-model our **Mission-focused Behaviors**.

# 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%)

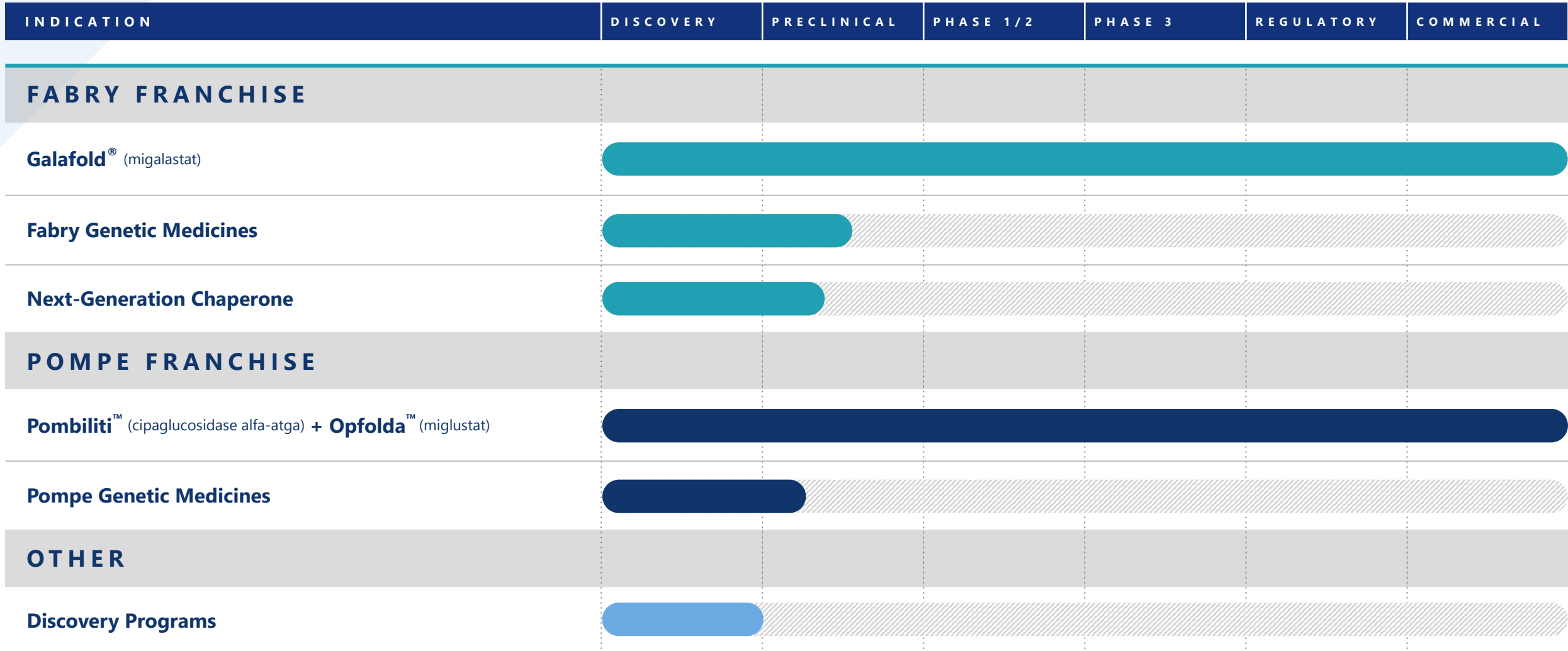
## Full-year 2024 Revenue Sensitivity

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





**Thank you**

