

AT THE FOREFRONT OF  
THERAPIES FOR RARE DISEASES

# 2Q23 Results Conference Call & Webcast

August 8, 2023



# 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 presentation 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, including as they are impacted by COVID-19 related disruption, are based on current information. The potential impact on operations from the COVID-19 pandemic is inherently unknown and cannot be predicted with confidence and may cause actual results and performance to differ materially from the statements in this release, including without limitation, because of the impact on general political and economic conditions, including as a result of efforts by governmental authorities to mitigate COVID-19, such as travel bans, shelter in place orders and third-party business closures and resource allocations, manufacturing and supply chain disruptions and limitations on patient access to commercial or clinical product. In addition to the impact of the COVID-19 pandemic, 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, including the FDA, EMA, MHRA, and PMDA, 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 commercializing Galafold in Europe, Japan, the US and other geographies or AT-GAA if and when approved; 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 and manufacturing. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. Statements regarding corporate financial guidance and financial goals and the attainment of such goals. With respect to statements regarding projections of the Company's revenue 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, 2022, and on Form 10-Q for the quarter ended June 30, 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

Patient-dedicated, rare disease biotechnology company with sustained double-digit revenue growth, a global commercial infrastructure, and late-stage development capabilities



First Oral Precision  
Medicine for  
Fabry Disease

**GLOBAL  
COMMERCIAL  
ORGANIZATION**

**World-class  
Clinical  
Development  
Capabilities**



**Gene Therapy  
Platform**

Leveraging  
Experience in Protein  
Engineering  
& Glycobiology

**Non-GAAP  
PROFITABILITY**  
expected in  
**2H 2023**

**EMPLOYEES  
in 20 Countries**

**AT-GAA**

Under Global  
Regulatory Reviews for  
Pompe Disease

**14-18%**

FY23 Galafold  
Revenue Growth  
at CER

**GALAFOLD  
&  
POMBILITI +  
OPFOLDA**

**Cumulative \$1.5B-  
\$2B Peak Potential**

**\$266M**

Cash  
as of 6/30/23

# 2023 Strategic Priorities

- 1 Sustain double-digit Galafold revenue growth of 14-18% at CER<sup>1</sup>**
- 2 Secure FDA, EMA, and MHRA approvals for AT-GAA**
- 3 Initiate successful global launches of AT-GAA**
- 4 Advance best-in-class, next-generation Fabry and Pompe pipeline programs and capabilities**
- 5 Maintain strong financial position on path to profitability**



# Galafold<sup>®</sup> (*migalastat*) Continued Growth

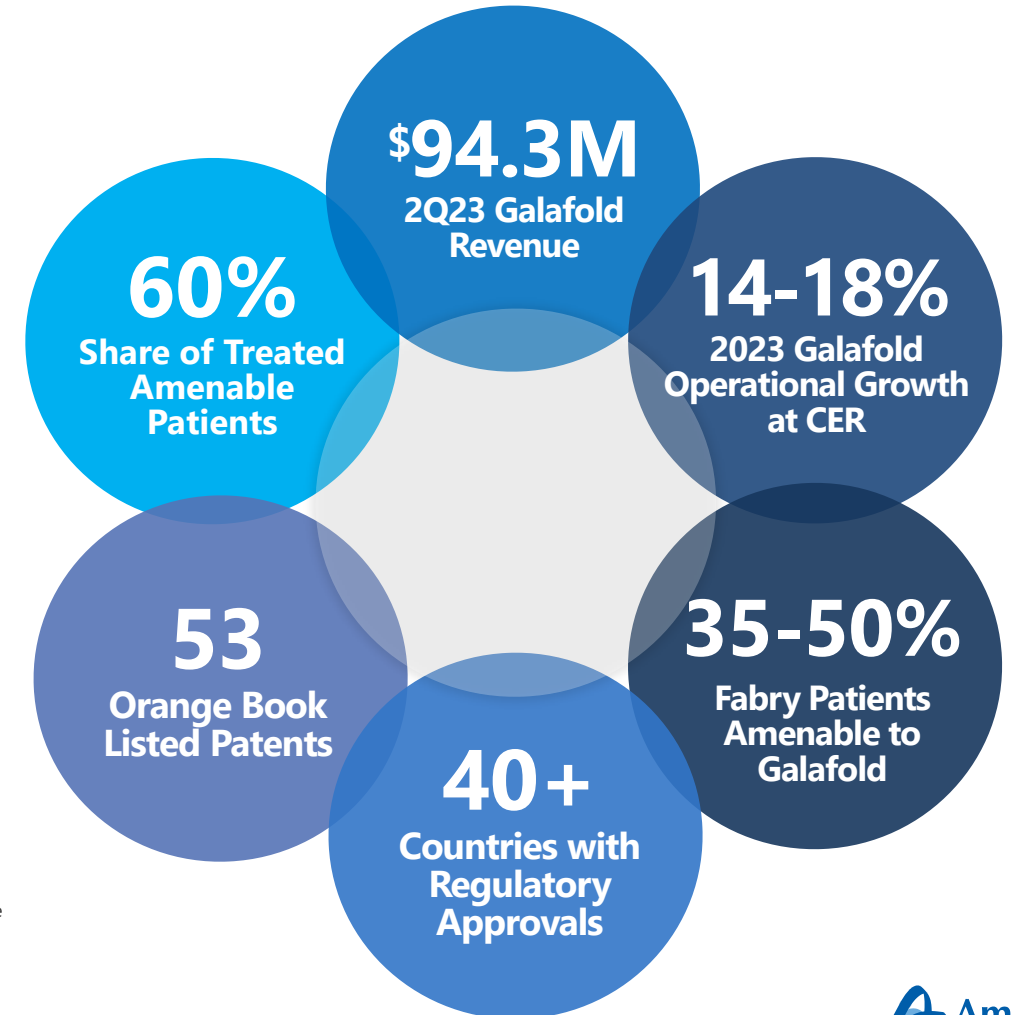
Building a leadership position in the  
treatment of Fabry disease



# 2023 Galafold Success (as of June 30, 2023)

Building on Galafold's success and leveraging leadership position to drive continued growth

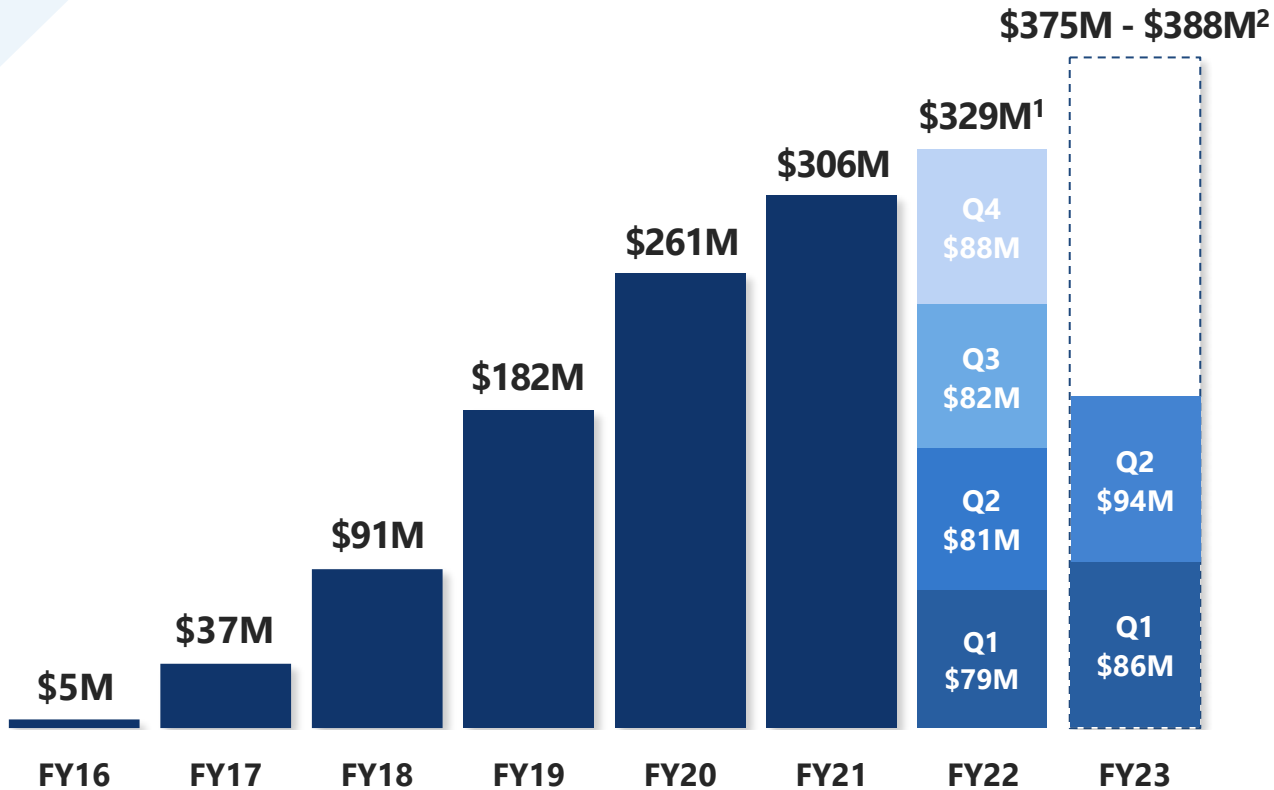
Galafold is the first and only approved oral treatment option with a unique mechanism of action for Fabry patients with amenable variants



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

Raising FY23 revenue growth guidance to 14% to 18% at CER



- Global mix of switch (~45%) and previously untreated patients (~55%)<sup>3</sup>
- Compliance and adherence over 90%+
- Expect non-linear quarterly growth to continue due to uneven ordering patterns and FX fluctuations

1H23 reported revenue growth of +13% to \$180M with strong operational growth of +16%

<sup>1</sup> FY22 reported revenue growth of +8% to \$329M with strong operational growth of +16% at CER – FY22 negative currency impact YoY of ~\$26M

<sup>2</sup> At constant exchange rate (CER)

<sup>3</sup> Data on file

# Galafold Global Commercial Momentum (as of June 30, 2023)

**Strong patient demand and performance against key metrics lay the foundation for continued double-digit growth in 2023**

## **Sustained Growth in 2023 Driven by:**

- **Continued penetration into existing markets**
- **Further uptake in diagnosed untreated population**
- **Continued geographic expansion and label extensions**
- **Maintaining compliance and adherence**
- **Driving reimbursement and access**





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

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



# Late-Onset Pompe Disease (LOPD) Overview

Late-onset Pompe disease is a rare, debilitating, and life-threatening lysosomal disorder caused by a deficiency of the enzyme acid alpha-glucosidase (GAA)



~5,000-10,000 people diagnosed globally; Significant underdiagnosis

Diagnosed at different stages of life, from childhood to adulthood

Majority of patients on current standard of care decline after ~2 years

Respiratory failure is a major cause of mortality

Deficiency of GAA leading to lysosomal glycogen accumulation and cellular dysfunction

Symptoms include progressive muscle weakness, particularly skeletal and respiratory muscles, that worsens over time

~\$1.2B+ global Pompe ERT sales<sup>1</sup>

# Global Regulatory Status

Expect regulatory approvals and launch into the three largest Pompe markets in 2023



- **Pombiliti<sup>®</sup> + Opfolda<sup>®</sup> now approved in the EU**



- **U.S. FDA approval expected 3Q 2023**



- **U.K. MAA submitted via recognition procedure based on CHMP opinion**
- **MHRA approval expected 3Q 2023**



# Ongoing Clinical Studies and Expanded Access Mechanisms

Advancing science through ongoing clinical studies and providing expanded access through multiple mechanisms

- Ongoing clinical studies in children and adolescents<sup>1</sup> with LOPD and infantile-onset Pompe disease (IOPD)
- Multiple expanded access mechanisms in place, including in the U.S., U.K., Germany, France, Japan, and others
- At time of first regulatory approval, ~200 people living with Pompe disease on AT-GAA across extension studies and expanded access programs
- ~75 centers worldwide currently participating in clinical trials and access programs



# Pombiliti + Opfolda EU Opportunity

EU Pompe market currently represents a sizeable market opportunity of \$450M+

- Strong indication statement:
  - *Pombiliti<sup>®</sup> (cipaglucosidase alfa) is a long-term enzyme replacement therapy used in combination with the enzyme stabiliser Opfolda<sup>®</sup> (miglustat) for the treatment of adults with late-onset Pompe disease (acid  $\alpha$  glucosidase [GAA] deficiency)*
- > 1,300 patients are estimated to be treated in Europe<sup>1</sup>
  - ~60 Patients throughout EU currently on Pombiliti + Opfolda, including ~20 in Germany and Austria
- Launch underway in Germany
  - 6 month “free pricing” period and AMNOG reimbursement process
  - First patients dosed and additional patients scheduled to start infusions



# Launch of Pombiliti + Opfolda Underway in the EU

Experienced and passionate rare disease commercial and medical organization supporting early days of launch



## Performance

### Patient Demand

Initial focus on clinical trial and expanded access patients

First patients dosed; Multiple scheduled for infusion

On-track to transition all trial and expanded access patients in Germany within 90 days



## KOL and Patient Outreach

### Promotion and Education Efforts

Existing relationships with HCPs at key treatment centers

Engaging top prescribers within first 30 days

Ongoing disease education



## Access and Reimbursement

### Positive Interactions with Payors

Focus on broad patient access

Country-by-country reimbursement process

Active discussions to demonstrate value



# Corporate Outlook

Delivering on our mission for patients  
and shareholders



# 2Q 2023 Select Financial Results

2Q23 revenue of \$94.5M and growth rate of 17% at CER

<i>(in thousands, except per share data)</i>	<b>Jun. 30, 2023</b>	<b>Jun. 30, 2022</b>
<b>Product Revenue</b>	\$94,503	\$80,731
<b>Cost of Goods Sold</b>	9,114	8,197
<b>R&amp;D Expense</b>	35,149	78,319
<b>SG&amp;A Expense</b>	65,423	53,379
<b>Changes in Fair Value of Contingent Consideration</b>	337	115
<b>Loss on Impairment of Assets</b>	1,134	—
<b>Depreciation and Amortization</b>	2,206	1,334
<b>Loss from Operations</b>	(18,860)	(60,613)
<b>Income Tax Expense</b>	(2,715)	(911)
<b>Net Loss</b>	(43,232)	(62,157)
<b>Net Loss Per Share</b>	(0.15)	(0.21)



# Financial Outlook and Path to Profitability

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



## Sustain Revenue Growth

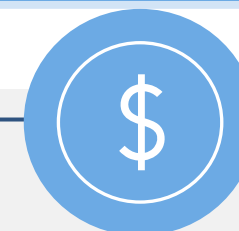
**\$180.8M** 1H 2023 revenue,  
**+16% YoY**  
operational growth

2023 Galafold revenue  
growth guidance of  
**+14-18% YoY at CER**



## Secure Approvals of AT-GAA

Galafold and  
AT-GAA expected to  
drive strong double-  
digit growth long term



## Deliver on Financial Goals

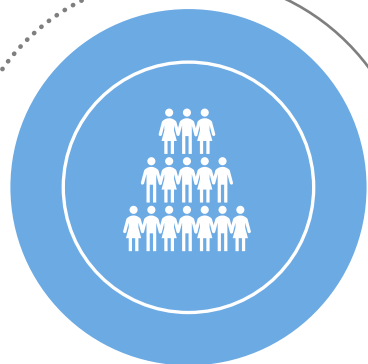
Focused on prudent  
expense management

2023 non-GAAP operating  
expense guidance of  
**\$330M-\$350M**

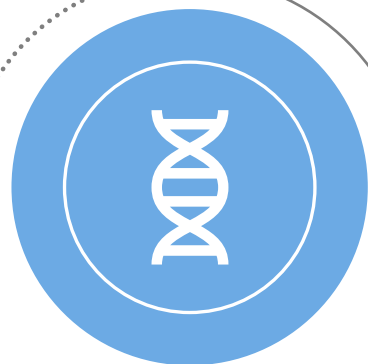
Achieve profitability<sup>1</sup>  
in 2H 2023

# Positioned for Significant Value Growth

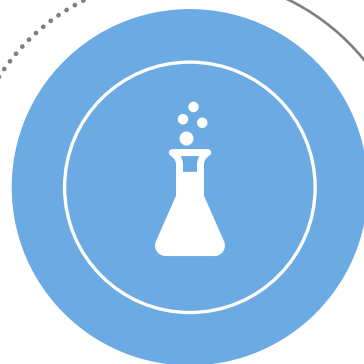
Focused on execution and driving sustainable double-digit revenue growth on path to profitability



Continue to bring Galafold<sup>®</sup> to as many patients as possible, sustain double-digit operational revenue growth



Successful launch of AT-GAA for people living with Pompe disease



Advance next-generation gene therapies in Fabry and Pompe diseases



Fully leverage global capabilities and infrastructure as a leader in rare diseases



Achieve non-GAAP profitability in 2H 2023<sup>1</sup>



# Appendix



# Appendix

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

	<u>Three Months Ended June 30,</u>		<u>Six Months Ended June 30,</u>	
	<u>2023</u>	<u>2022</u>	<u>2023</u>	<u>2022</u>
<b>Total operating expenses - as reported GAAP</b>	<b>\$104,249</b>	<b>\$133,147</b>	<b>\$221,213</b>	<b>\$279,619</b>
<b>Research and development:</b>				
Stock-based compensation	4,117	4,379	12,607	13,744
<b>Selling, general and administrative:</b>				
Stock-based compensation	12,460	8,084	38,864	29,370
<b>Loss on impairment of assets</b>	1,134	-	1,134	6,616
<b>Changes in fair value of contingent consideration payable</b>	337	115	588	(1,073)
<b>Depreciation and amortization</b>	2,206	1,334	3,463	2,745
<b>Total operating expense adjustments to reported GAAP</b>	<b>20,254</b>	<b>13,912</b>	<b>56,656</b>	<b>51,402</b>
<b>Total operating expenses - as adjusted</b>	<b>\$83,995</b>	<b>\$119,235</b>	<b>\$164,557</b>	<b>\$228,217</b>

# 2022 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

Expanded Access through Feb 2023:

79 patients / 19 countries

Contributions allocated:

\$2,288,998 U.S.

\$954,349 Intl.

Amicus supported community programs:

22

Volunteer hours (U.S.):

580

## Environmental Management

Committed to producing transformative medicines for patients 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 % Female Employees

484

57%

% Hiring Slate Diversity 97%

## 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 Q2 2023

Currency Variances: USD/	Q2 2022	Q2 2023	YoY Variance
EUR	1.066	1.089	2.2%
GBP	1.257	1.251	(0.5%)
JPY	0.008	0.007	(5.6%)

## Full Year 2023 Revenue Sensitivity

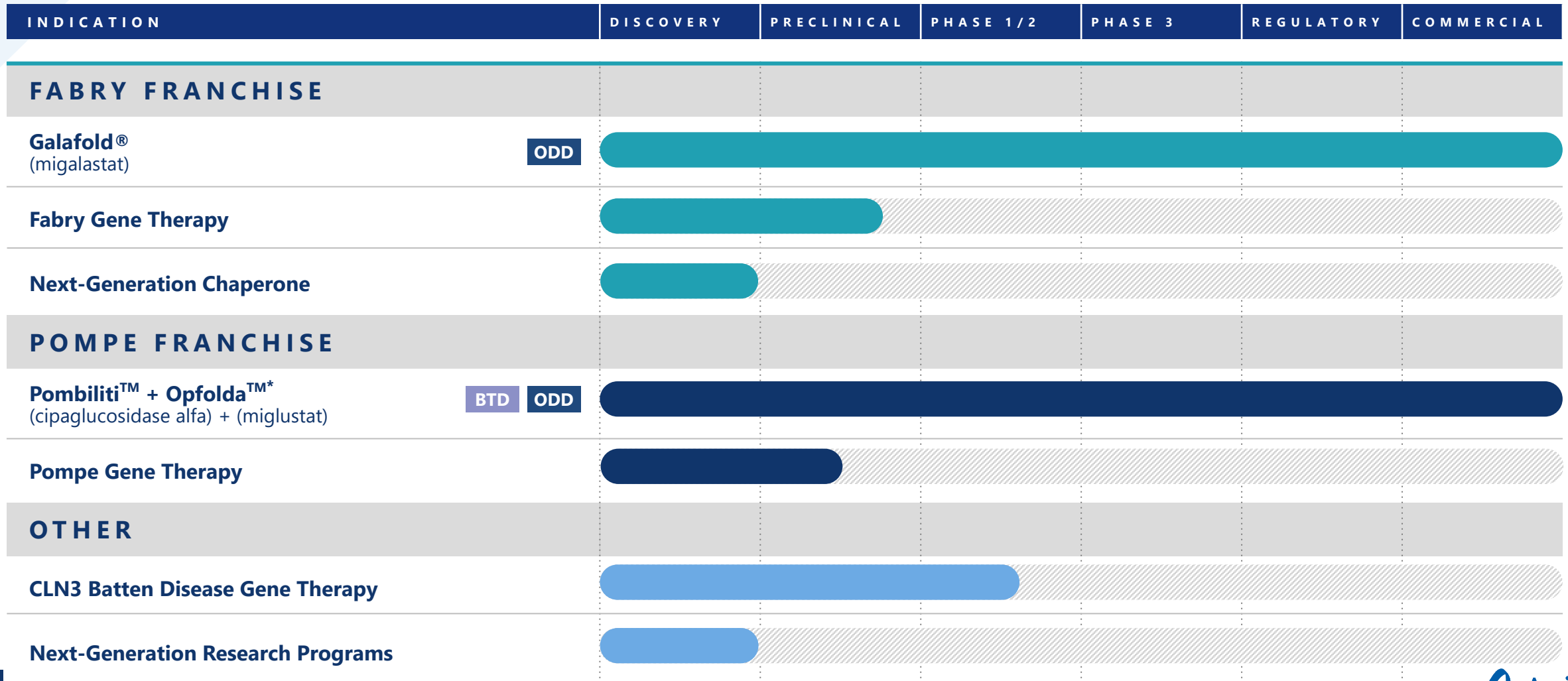
Given the high proportion of Amicus revenue is Ex-US, a change in exchange rates of +/- 5% compared to year end 2022 rates could lead to a \$11M-\$12M change in global reported revenues in 2023.

## Distribution of Galafold Revenue by Quarter in Past 5 years:

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

# Amicus Pipeline

Streamlined rare disease pipeline with focus on Fabry disease and Pompe disease franchises





**Thank you**

