AT THE FOREFRONT OF
THERAPIES FOR RARE DISEASES

42nd Annual J.P. Morgan Healthcare Conference

January 8, 2024

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 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, 2022, and on Form 10-Q for the quarter ended September 30, 2023. 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.

Our Passion is for Patients

Our Mission:

We seek to deliver the highest quality therapies for people living with rare diseases

Our Vision:

Be a leader in rare disease drug development and commercialization leveraging our global capabilities in bringing life-changing therapies to patients

Definition:

\əˈmēkəs (noun) *Latin* Friend

Amicus Therapeutics

A Rare Company

A leading biotech company with significant revenue growth and near-term profitability

Galafold® (migalastat)

First Oral Precision Medicine for Fabry Disease

LEVERAGEABLE GLOBAL COMMERCIAL ORGANIZATION

2 APPROVED THERAPIES

World Class Clinical Development Capabilities

Non-GAAP PROFITABILITY Q4 2023 ON-TRACK

>500 EMPLOYEES in 20+ Countries

Pombiliti™ (cipaglucosidase alfa-atga) + Opfolda™ (miglustat) 65 mg capsules

First Two-Component Therapy for Pompe Disease

~$399.4M in 2023 Revenue[1]

21% Increase Year-Over-Year

Combined Peak Revenue Potential

$1.5B - $2B

Line of Sight to Generating Positive Cashflow

[1] Preliminary and unaudited

Amicus Therapeutics

2023 Strategic Priorities Achieved

- Galafold® revenue growth of 12-17% at CER[1], raised to 16-18%
- Secure FDA, EMA, and MHRA approvals for Pombiliti™ + Opfolda™
- Initiate successful global launches of Pombiliti™ + Opfolda™
- Advance best-in-class, next-generation Fabry and Pompe pipeline programs and capabilities
- Maintain strong financial position on path to profitability

[1]CER: Constant Exchange Rates; 2023 Galafold revenue guidance utilized actual exchange rate as of December 31, 2022

2023 Key Milestones

Pombiliti + Opfolda Approved in Three Largest Pompe markets: U.S., E.U., U.K.

On Path to Non-GAAP Profitability

Galafold: Fastest Growing Treatment in Fabry Disease

Galafold Growth Driven by Patient Demand: Highest Growth Rate in Last Four Years

Successful Early Days of Pombiliti + Opfolda Launch

Strategic Financing with Blackstone Refinancing Debt at More Favorable Terms

Galafold Quarterly Revenue Surpasses $100 Million for First Time

Positive Long-term Data from OLE Study of Pombiliti + Opfolda

Amicus Therapeutics

2024 Strategic Priorities

A Transformative Year Ahead for Amicus

1. Double-digit Galafold® revenue growth of 11-16% at CER[1]
2. Successful multiple launches of Pombiliti™ + Opfolda™
3. Advance ongoing studies to support medical and scientific leadership in Fabry and Pompe diseases
4. Achieve non-GAAP profitability for the full year

[1]CER: Constant Exchange Rates; 2024 Galafold revenue guidance utilizes actual exchange rate as of December 31, 2023

Amicus Therapeutics

Amicus Therapeutics®

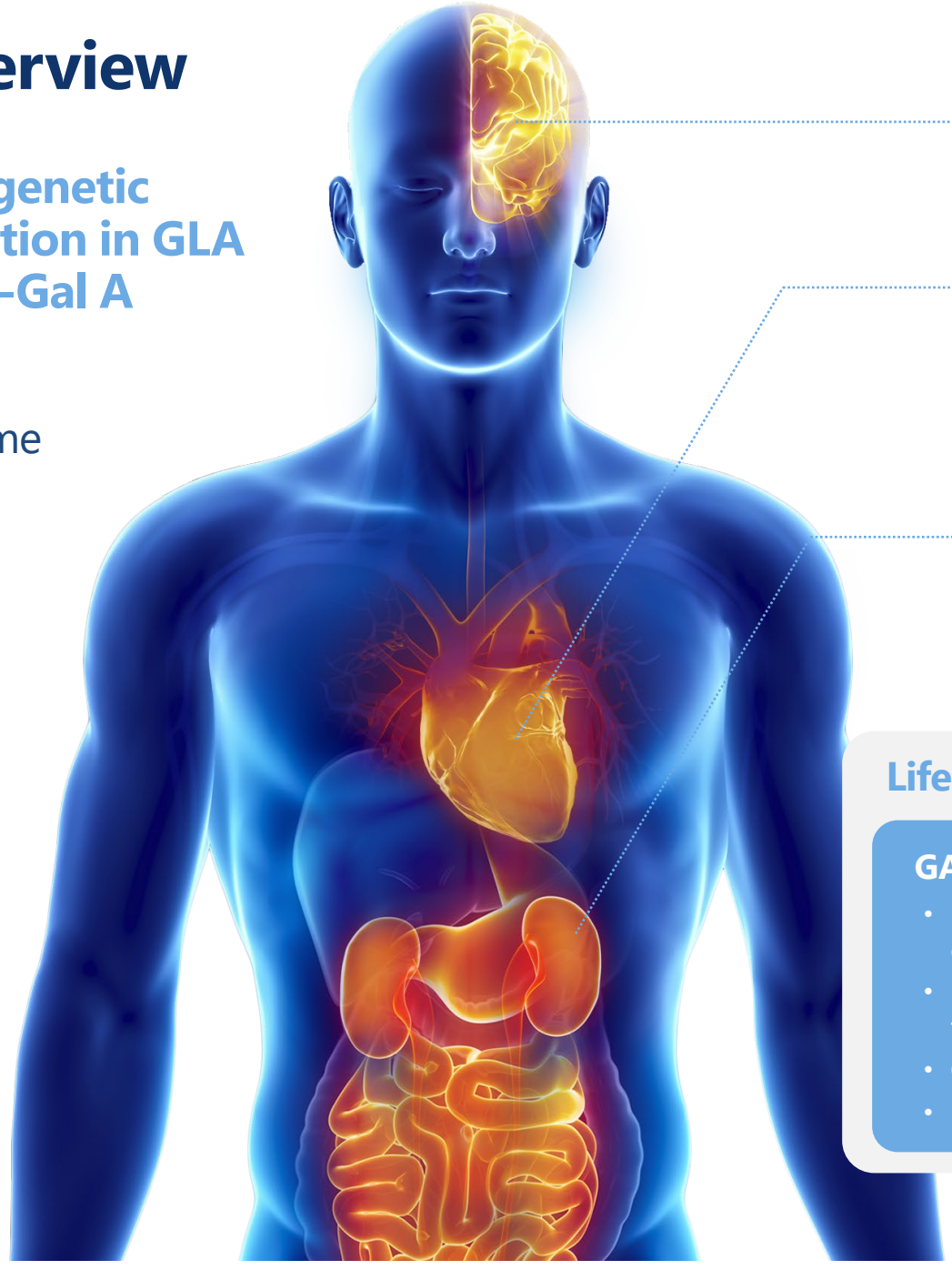
Continued Growth of Galafold® *(migalastat)*

Expanding leadership in the treatment of Fabry disease

Fabry Disease Overview

Fabry is a rare inherited genetic disorder caused by mutation in GLA gene and deficiency of α-Gal A enzyme

- Deficiency of α-Gal A enzyme leads to GL-3 accumulation
- >1,000 known mutations
- >17,000 diagnosed WW
- X-linked dominant disease leading to diagnosis of multiple family members



Leading Causes of Death

TRANSIENT ISCHEMIC ATTACK (TIA) & STROKE[1]

HEART DISEASE[2]

- Irregular heartbeat (fast or slow)
- Heart attack or heart failure
- Enlarged heart

KIDNEY DISEASE[3]

- Protein in the urine
- Decreased kidney function
- Kidney failure

Life-limiting Symptoms

GASTROINTESTINAL[3]

- Nausea, vomiting, cramping, diarrhea
- Pain/bloating after eating, feeling full
- Constipation
- Difficulty managing weight

PAIN[3]

FATIGUE[3]

ANHIDROSIS[3]

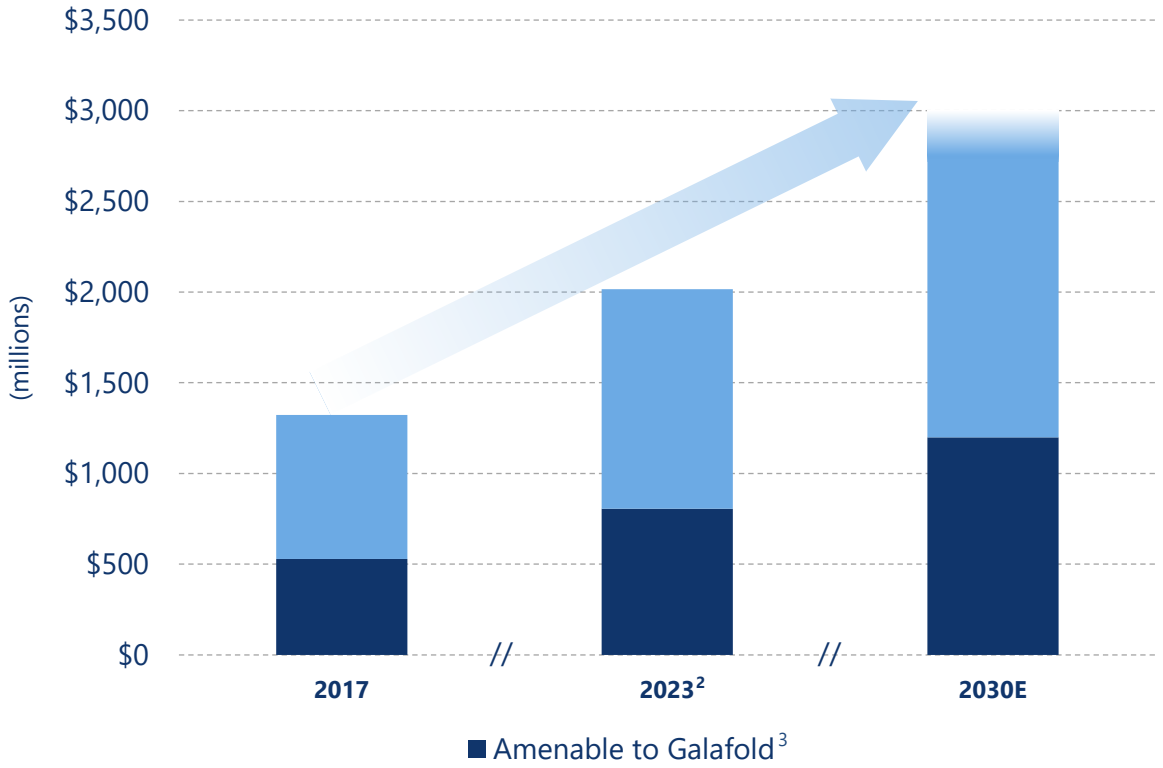
[1] Desnick R, *et al.* Ann Intern Med. 2003
[2] Yousef Z, *et al.* Eur Heart J. 2013
[3] Germain D. Orphanet J Rare Dis. 2010

Amicus Therapeutics

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[1]



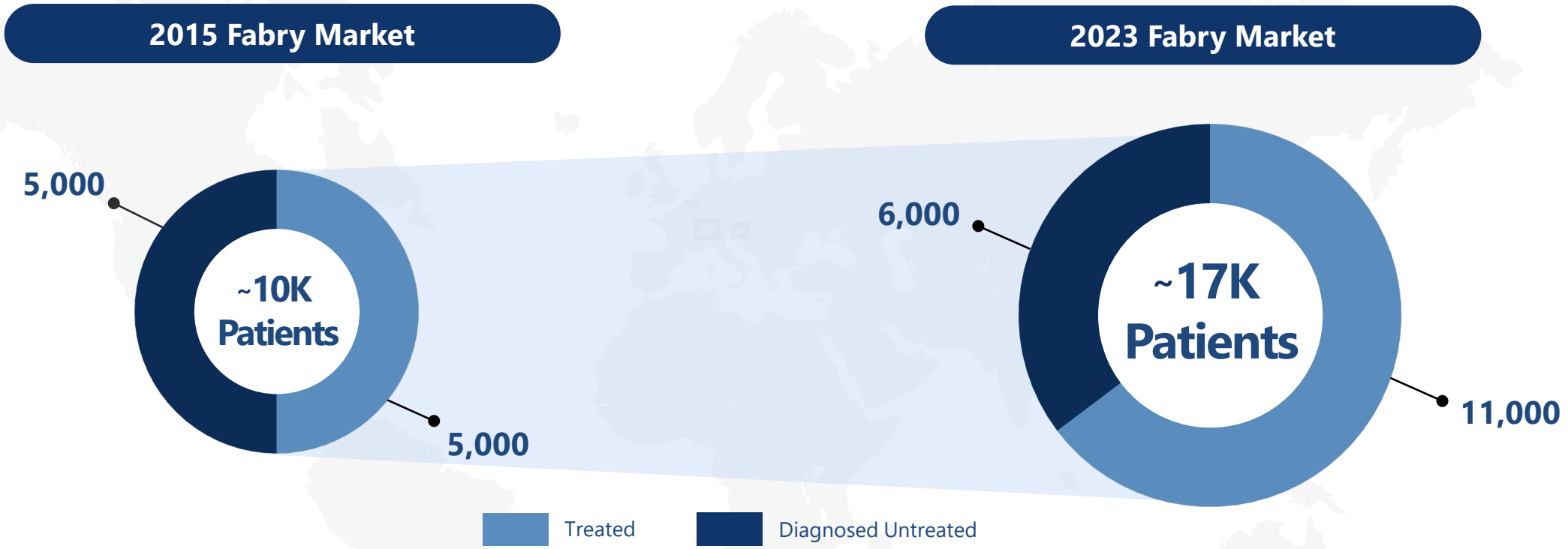
- Significantly underdiagnosed
  - Newborn screening studies suggest Fabry is one of the more prevalent rare genetic diseases (~1:1,000 to ~1:4,000 incidence)
- Continued market growth driven by increased diagnosis
- Galafold continues to be the fastest growing Fabry treatment and the greatest contributor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

[1] Global market measured by reported sales of approved therapies for Fabry disease – 2030 sales projected using ~7% CAGR
[2] LTM ended September 30, 2023
[3] Assumes ~40% amenability to Galafold

Amicus Therapeutics

Fabry Patient Dynamics

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



Significant pool of diagnosed untreated patients remain

Based on Amicus data on file

Amicus Therapeutics

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

~$388M
2023 Galafold Revenue[1]

60-65%
Share of Treated Amenable Patients

~17%
YoY Growth at CER[2]

2,400+
Individuals Treated

35-50%
Fabry Patients Amenable to Galafold

40+
Countries with Regulatory Approvals

Galafold is indicated for adults with a confirmed diagnosis of Fabry disease and an amenable variant. The most common adverse reactions reported with Galafold (≥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.

[1] Preliminary and unaudited
[2] CER: Constant Exchange Rates

Amicus Therapeutics

Key Growth Drivers for 2024

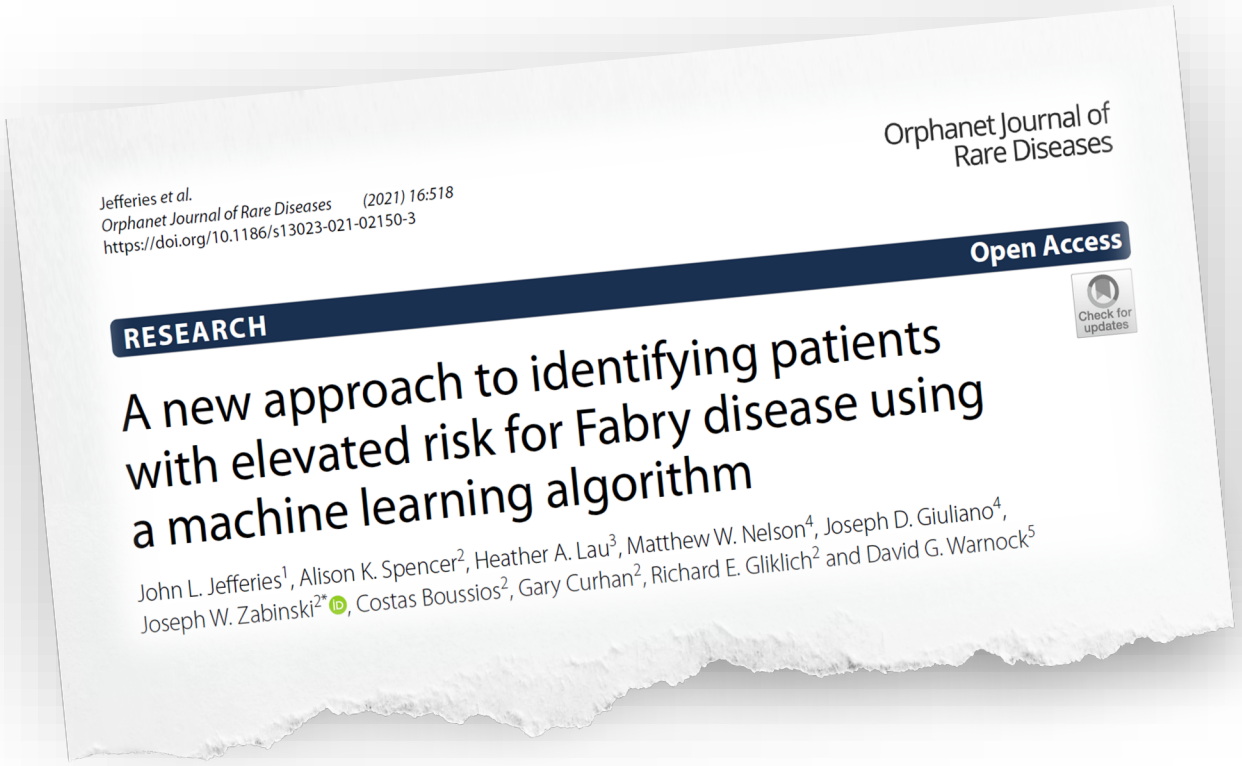
Strong patient demand laying groundwork for continued double-digit Galafold growth

- Improve diagnosis of patients through medical education, screening, and testing
- Drive market share of treated amenable patients through excellent execution
- Expand market through uptake in naïve population as well as geographic and label expansion
- Maintain >90% adherence and compliance through HCP and patient education and support

Amicus Therapeutics

Improving Diagnosis of Fabry Disease

Harnessing AI to improve diagnosis of people living with Fabry and predicting patient outcomes



- Partnership with OM1 and leading healthcare system in the U.S. to pilot algorithm
- Finding undiagnosed patients and variation in patient phenotype
- Predicting health outcomes including serious events
- Applying AI tools to additional Fabry markets

Launch Underway of Pombiliti™ + Opfolda™

(cipaglucosidase alfa-atga) ***(miglustat)***

Resetting expectations 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

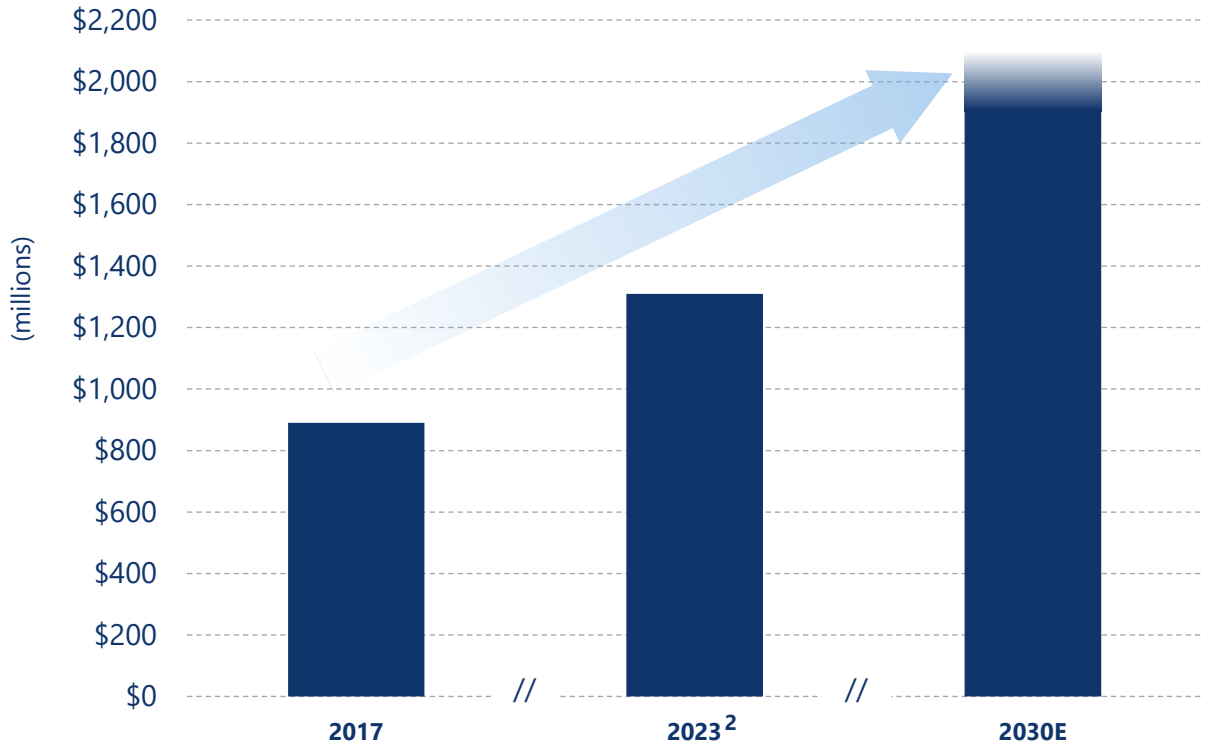
~$1.3B+ global Pompe ERT sales[1]

[1] Based on 12 months ended September 30, 2023.

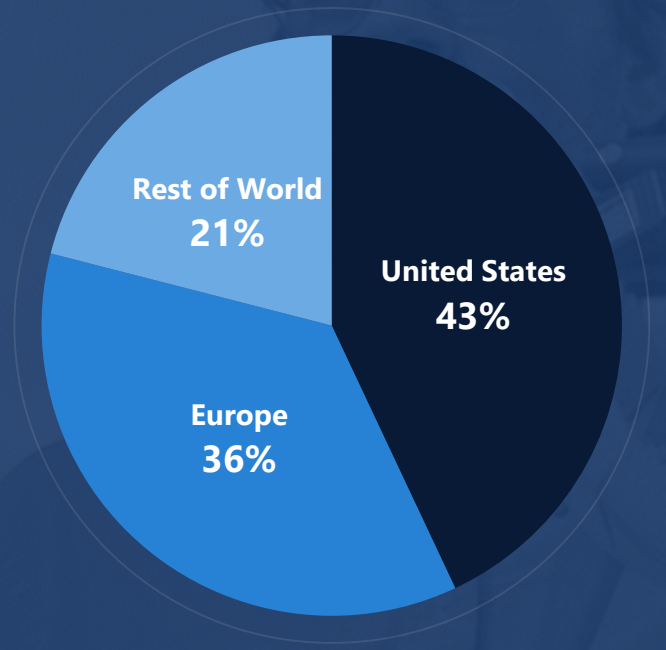
Global Pompe Market

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[1]



Global Pompe Market Sales Split YTD 2023[2]



An estimated 3,500-4,000 Pompe patients globally are being treated by ERT[3]

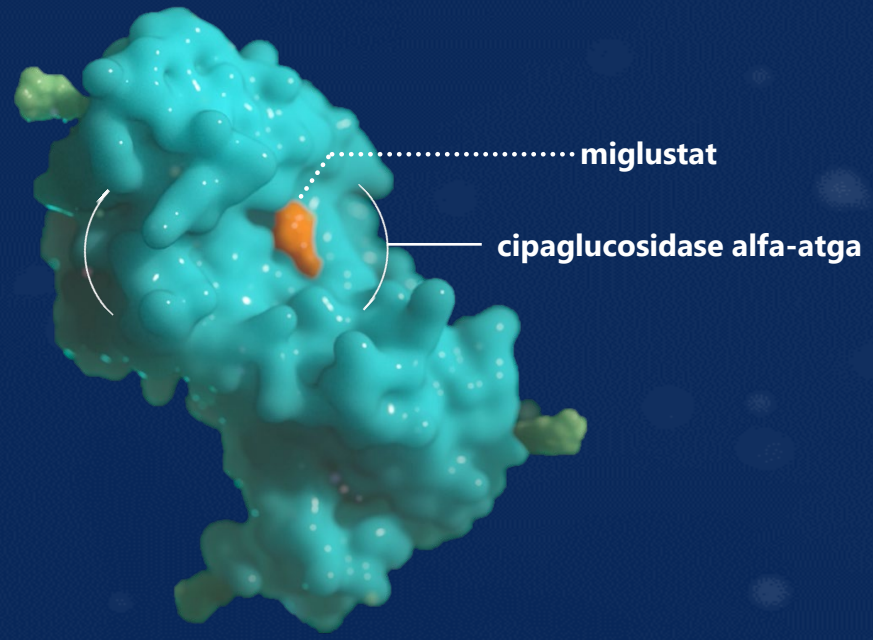
[1] Global market measured by reported sales of approved therapies for Pompe disease – 2029 sales projected using ~8% CAGR
[2] LTM ended September 30, 2023
[3] Amicus Data on File from Market Mapping

Amicus Therapeutics

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 activity[1]

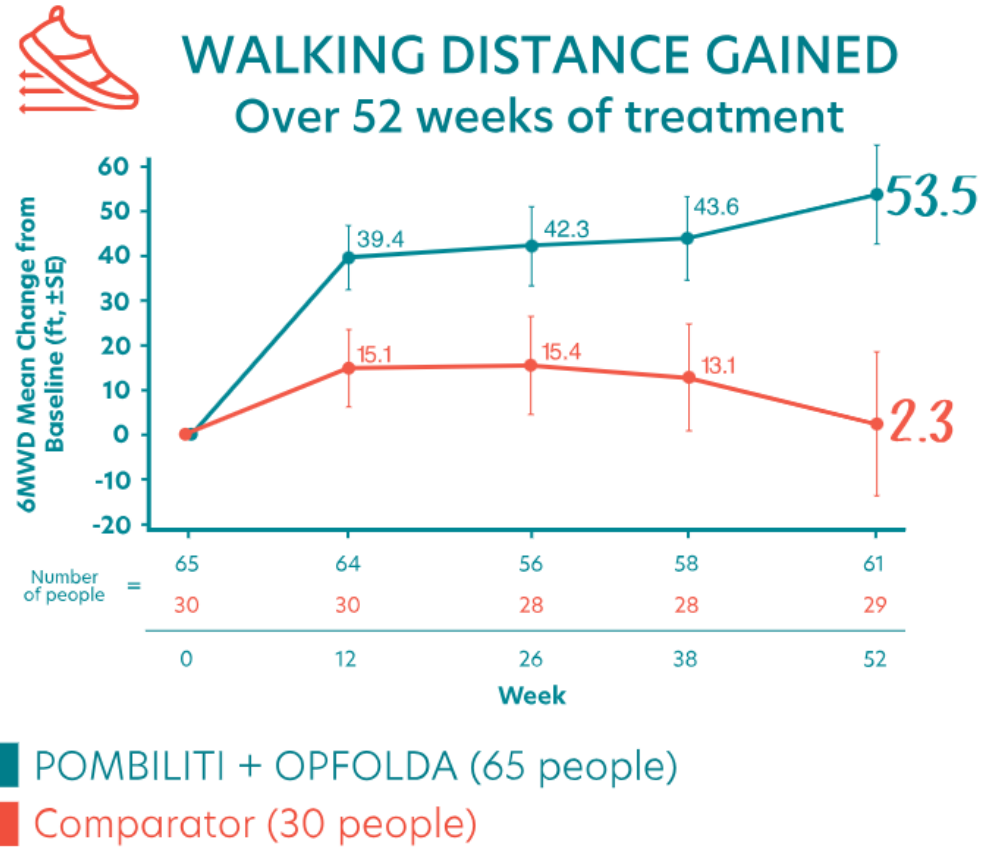


[1] Selvan et al. 2021, J Biol Chem 2021 Jan-Jun;296:100769
ERT: Enzyme Replacement Therapy

Amicus Therapeutics

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[1] in ERT-experienced adults

[1]An alglucosidase alfa product not approved in the US + placebo.
POMBILITI + OPFOLDA is not approved for use in ERT-naïve people.
Results of the 6MWT and FVC for ERT-experienced people were numerically favorable but were not tested to determine statistical superiority of POMBILITI + OPFOLDA vs the comparator.

Amicus Therapeutics

Successful Global Launch of Pombiliti + Opfolda Underway

FY 2023 revenue of ~$11.6M[1] provides strong foundation for 2024

Patient Demand

~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 within first 30 days

Existing relationships with HCPs at key treatment centers

Ongoing disease education

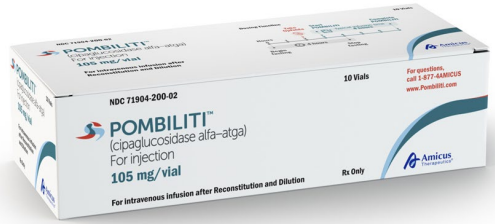
Access and Reimbursement

Positive interactions with US 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

[1] Preliminary and unaudited

Amicus Therapeutics

International: EU and U.K. Update and Market Opportunity

EU and U.K. Pompe markets collectively represent sizeable market opportunity

Strong indication statement:

Pombiliti™ (cipaglucosidase alfa) is a long-term enzyme replacement therapy used in combination with the enzyme stabiliser miglustat for the treatment of adults with late-onset Pompe disease (acid α glucosidase [GAA] deficiency)

- >1,300 patients are estimated to be treated in Europe[1]
- >200 patients are estimated to be treated in the U.K.[1]
- Broad experience from a wide set of KOLs through clinical trials and early access programs
- Leveraging EU label and regulatory outcome to extend into other geographies

LAUNCH DYNAMICS

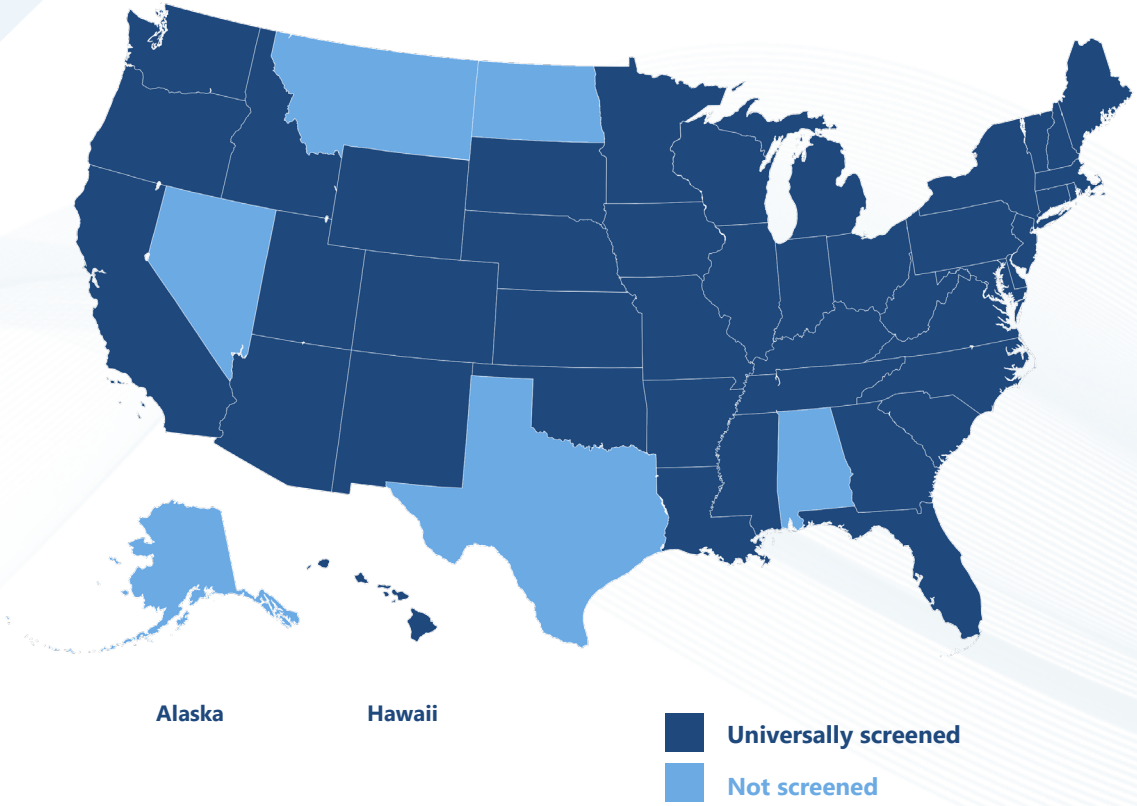
- All EAP and clinical trial patients transitioned
- Multiple new patient starts from both switch and naïve patients
- Robust interest from physician community and treatment centers

[1] Amicus Data on File from Market Mapping

U.S. Update and Market Opportunity

U.S. Pompe market currently represents a sizeable and growing market opportunity of >$500M[1]

Pompe Disease Newborn Screening in 44 U.S. States[2]



- ~80% of the 800 treated patients in the U.S. are adults[1]
- Based on reported revenues, ~45% of patients remain on legacy ERT (alglucosidase alfa)
- >25 clinical trial sites across the country with experience of Pombiliti + Opfolda

LAUNCH DYNAMICS

- All eligible clinical trial patients treated with commercial product or scheduled to be treated
- Majority of new commercial patients switched from newer ERT (avalglucosidase alfa-ngpt)
- Positive initial feedback from patient and physician communities

[1] Amicus Data on File from Market Mapping
[2] 2024, Jan 06. Newborn Screening Status for All Disorders. https://www.newsteps.org/resources/data-visualizations/newborn-screening-status-all-disorders

Regulatory and Clinical Updates

Building the body of evidence and expanding commercial access

- >10 reimbursement dossiers submitted and multiple regulatory submissions throughout 2024
- Ongoing clinical studies in children with late-onset Pompe disease and infantile-onset Pompe disease (IOPD)
- Amicus registry for Pompe disease expected to continue generating evidence on differentiated MOA and long-term effect

Amicus Therapeutics®

Corporate Outlook

Delivering on our mission for patients and shareholders

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[1] profitability

Clear line of sight to generating positive cashflow

[1]Non-GAAP Net Income defined as GAAP Net Income excluding the impact of stock-based compensation expense, changes in fair value of contingent consideration, loss on impairment of assets, depreciation and amortization, acquisition related income (expense), loss on extinguishment of debt, restructuring charges and income taxes.

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

>350 Patients*

>2,600 Patients*

Many Thousands of Patients*

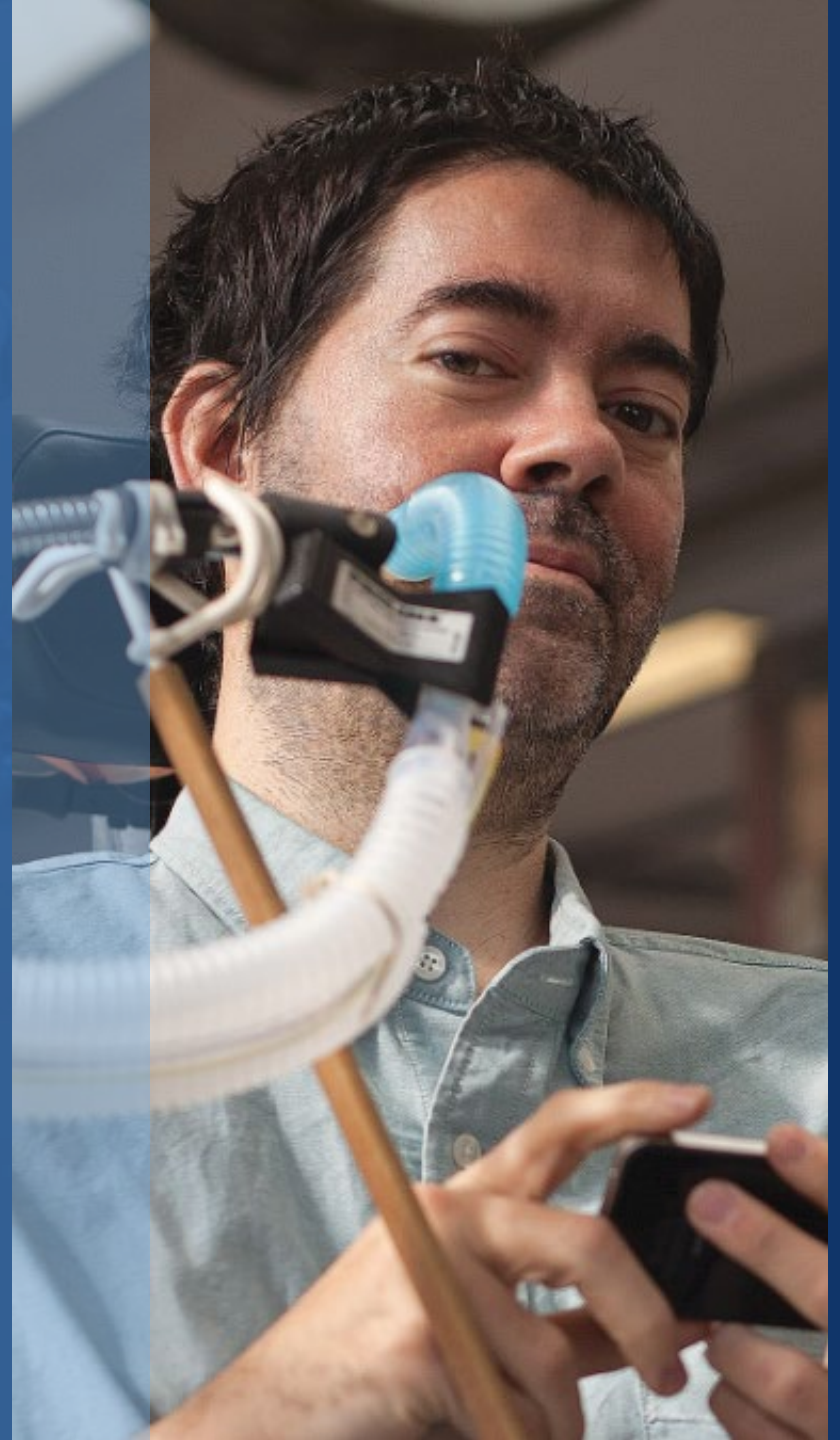
YE17 → YE23 → 2024+

*Clinical and commercial, all figures approximate

Amicus Therapeutics

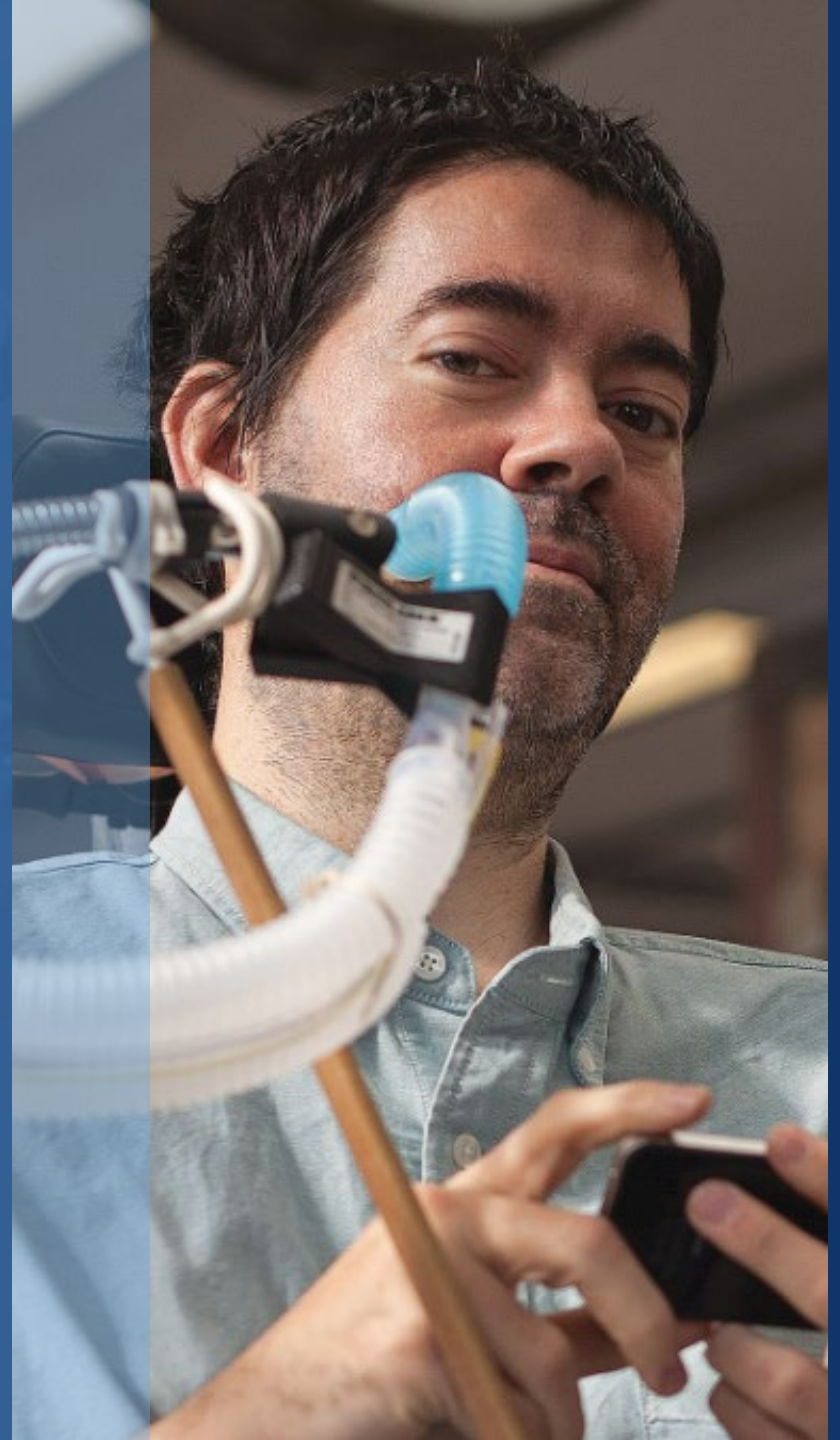
Amicus Therapeutics®

Thank You



Amicus Therapeutics®

Appendix



FX Sensitivity and Galafold Distribution of Quarterly Sales

Impact from Foreign Currency Q4 2023[1]

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[1]

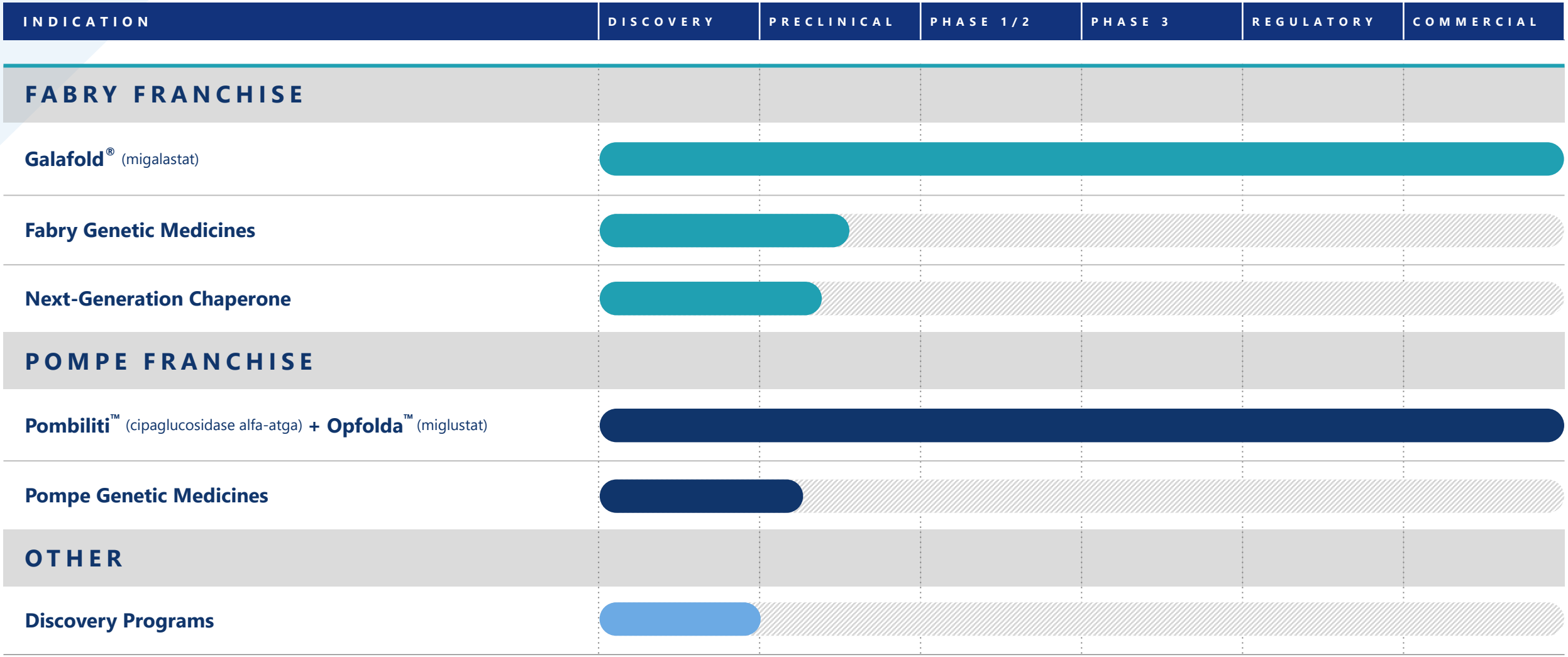
Given the high proportion of Amicus revenue Ex-US (~60%), a change in exchange rates of +/- 5% compared to year-end 2023 rates could lead to a $15M move in global reported revenues in 2024.

Distribution of Galafold Revenue by Quarter over Past 5 years:

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

[1] Preliminary and unaudited

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



Amicus Therapeutics

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 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

484

% Female Employees

57%

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**.

Amicus Therapeutics