AT THE FOREFRONT OF
THERAPIES FOR RARE DISEASES

43rd Annual J.P. Morgan Healthcare Conference

January 13, 2025

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 the prospects and timing of the potential regulatory and pricing and reimbursement approvals of our products, commercialization plans, manufacturing and supply plans, financing plans, and the projected revenues, profitability 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, 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 presentation due to the risks and uncertainties inherent in our business, including, without limitation: 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 U.K., the U.S. and other geographies; the potential that we will not be able to effectively compete in our approved markets: the potential that generic or new competitor products enter the market; 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 support 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 and 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. 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 presentation 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.

Definition:
\əˈmēkəs (noun) *Latin* Friend

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

Amicus Therapeutics

A Rare Company

A unique story in biotech with significant revenue growth and profitability

~$528M
2024 Total Revenue[1] (+32% Growth)[2]

First Oral Precision Medicine for Fabry Disease
Galafold® (migalastat)

10-15%
FY 2025 Galafold Revenue Growth[2]

First Two-Component Therapy for Pompe Disease
Pombiliti® (cipaglucosidase alfa-atga)
+
Opfolda® (miglustat) 65 mg capsules

65-85%
FY 2025 Pombiliti + Opfolda Revenue Growth[2]

FY 2024 Non-GAAP Profitability Achieved

Leverageable Global Commercial Organization

$1B+
Total Revenues Expected in 2028

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

2024 Strategic Priorities

Delivered

- Galafold® revenue growth of 11-16% at CER[1], raised to 16-18%
- Execute multiple successful launches of Pombiliti® + Opfolda®
- Advance ongoing studies to support medical and scientific leadership in Fabry and Pompe diseases
- Achieve non-GAAP profitability for the full year

[1]CER: Constant Exchange Rates;

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2024 Key Milestones

Highest Patient Demand for Galafold Since Early Launch

U.S. Galafold IP Settlement with Teva Provides Long Runway of Growth

Galafold: A Fast Growing Treatment in Fabry Disease & SoC in Amenable Population

Non-GAAP Profitability Achieved in 2024

Successful First Year of Launch of Pombiliti + Opfolda

Positive Feedback for Pombiliti + Opfolda Increasing Among Patients and Physicians

Reduced OPEX Guidance and Judiciously Managed Expenses

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2025 Strategic Priorities

1. Deliver total revenue growth of 17-24% at CER[1]
2. Double-digit Galafold® revenue growth of 10-15% at CER[1]
3. Pombiliti® + Opfolda® revenue growth of 65-85% at CER[1]
4. Advance ongoing studies to broaden labels and strengthen scientific leadership in Fabry and Pompe diseases
5. Deliver positive GAAP Net Income during H2 2025

[1]CER: Constant Exchange Rates

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Galafold® *(migalastat)* Continued Growth

Building a leadership position
in the treatment of Fabry disease

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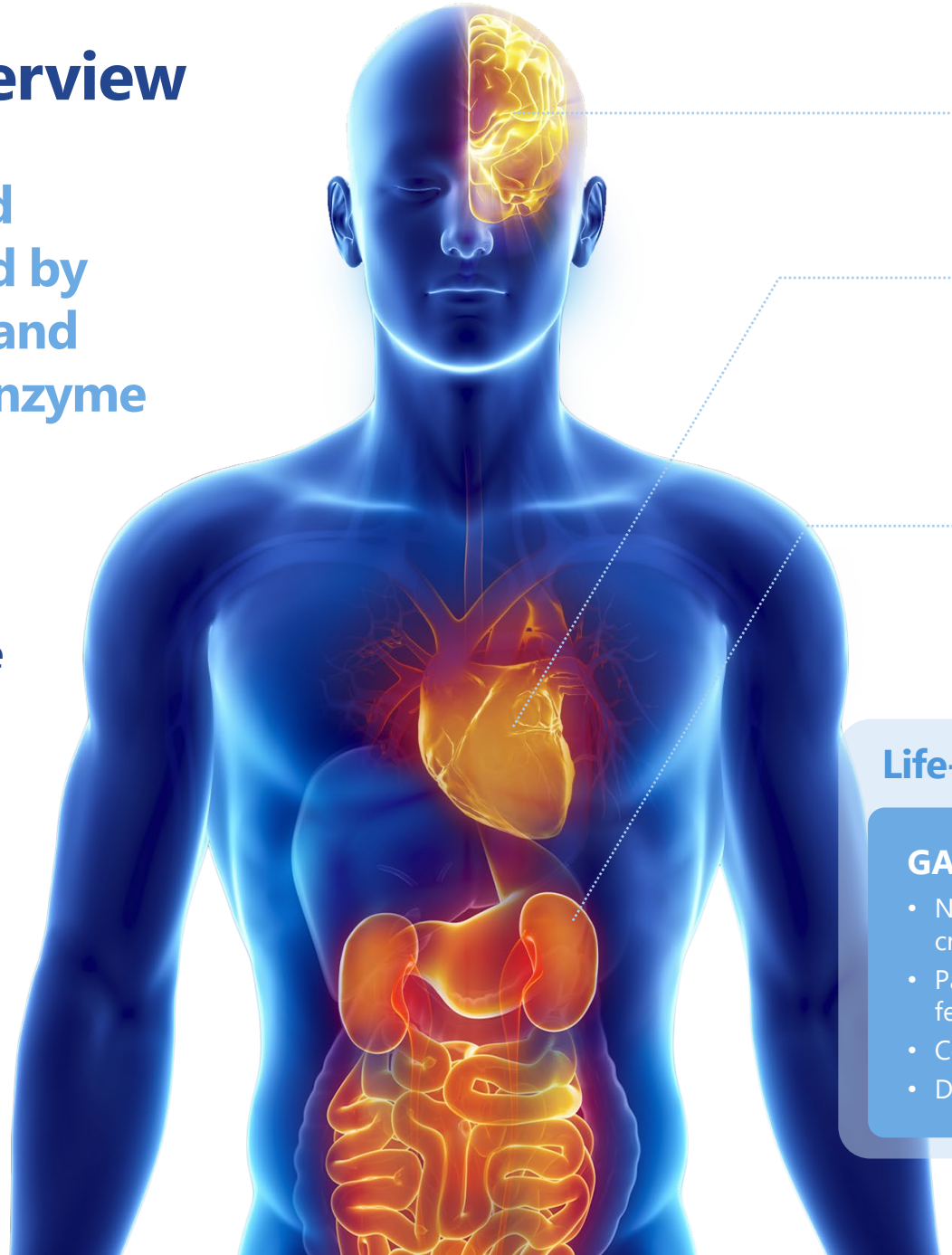
Fabry Disease Overview

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

- >1,000 known mutations
- >18,000 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

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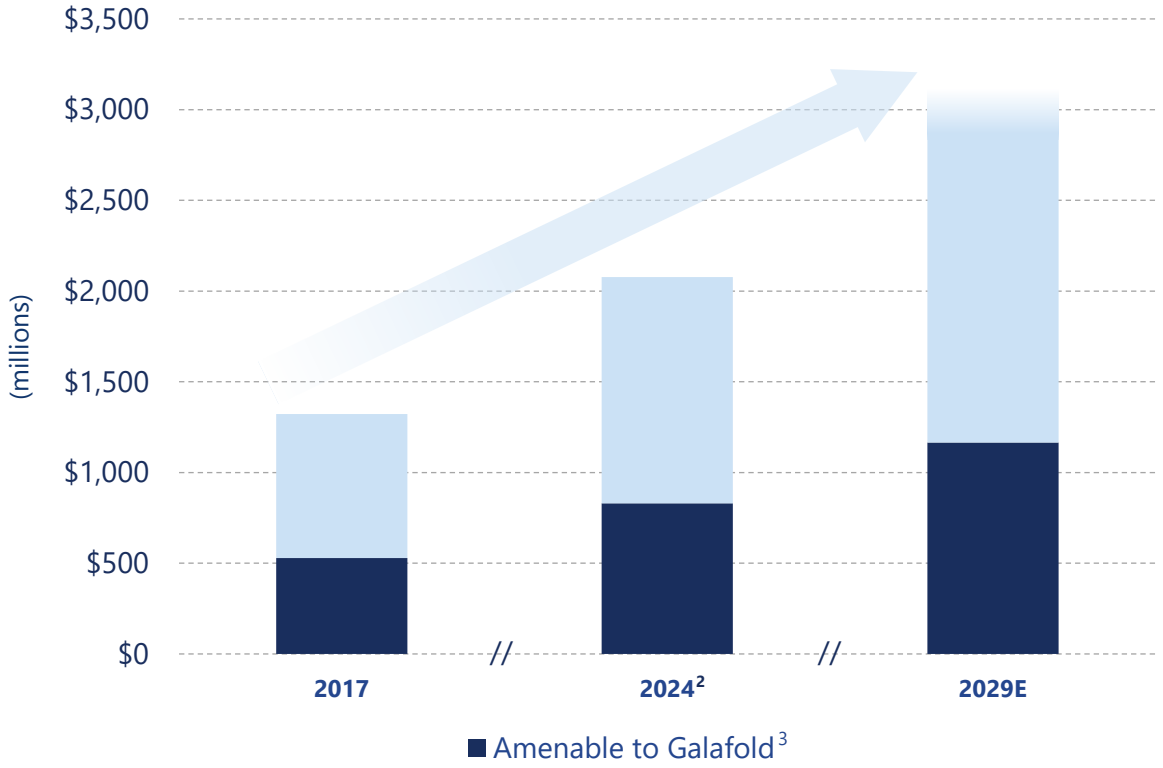
Global Fabry Market

Fabry market expected to grow to ~$3B by 2029

Global Fabry market of ~$2.1B in 2024 and tracking toward ~$3B+ by the end of the decade[1]



- Significantly underdiagnosed
  - Newborn screening studies suggest Fabry is one of the more prevalent rare genetic diseases (~1:1,500 to ~1:4,000 incidence)[4]
- Continued market growth driven by increased diagnosis
- Anticipate market size for amenable patients to surpass $1B in 2029
- Galafold continues to be the greatest contributor to market growth

[1] Global market measured by reported sales of approved therapies for Fabry disease – 2029 sales projected using ~7% CAGR
[2] LTM ended September 30, 2024
[3] Assumes ~40% amenability to Galafold
[4] Burton 2017 J Pediatr 2017;190:130-5 ; Mechtler *et al.*, The Lancet, 2011 Dec.; Hwu *et al.*, Hum Mutation, 2009 Jun; Spada *et al.*, Am J Human Genet., 2006 Jul

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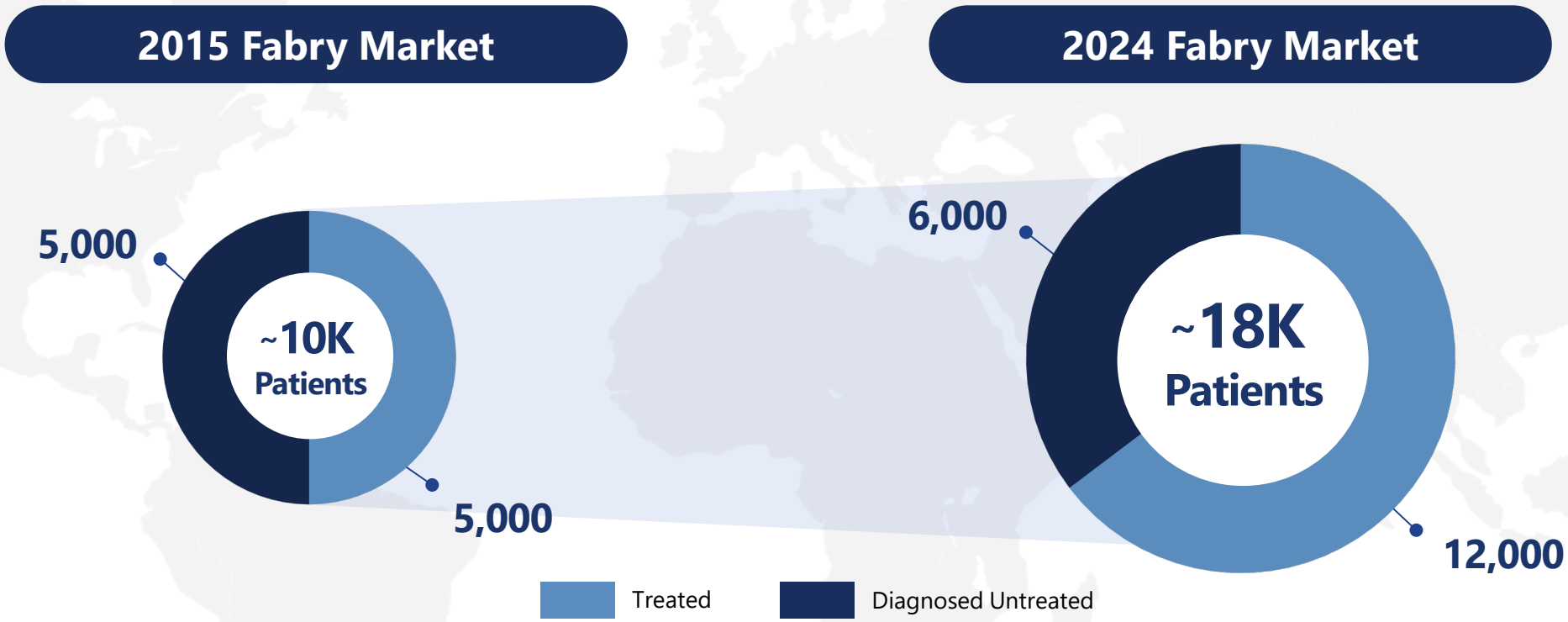
Fabry Market Dynamics

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



6,000 diagnosed untreated patients remain

Based on Amicus data on file

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2024 Galafold Success (as of December 31, 2024)

Only approved oral treatment in Fabry disease and standard of care for amenable patients

A unique mechanism of action for Fabry patients with amenable variants

~$458M
FY24 Galafold Revenue[1]

>60%
Share of Treated Amenable Patients

10-15%
2025 Galafold Growth at CER[2]

~2,730
Individuals Treated[3]

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://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
[3] As of YE 2024

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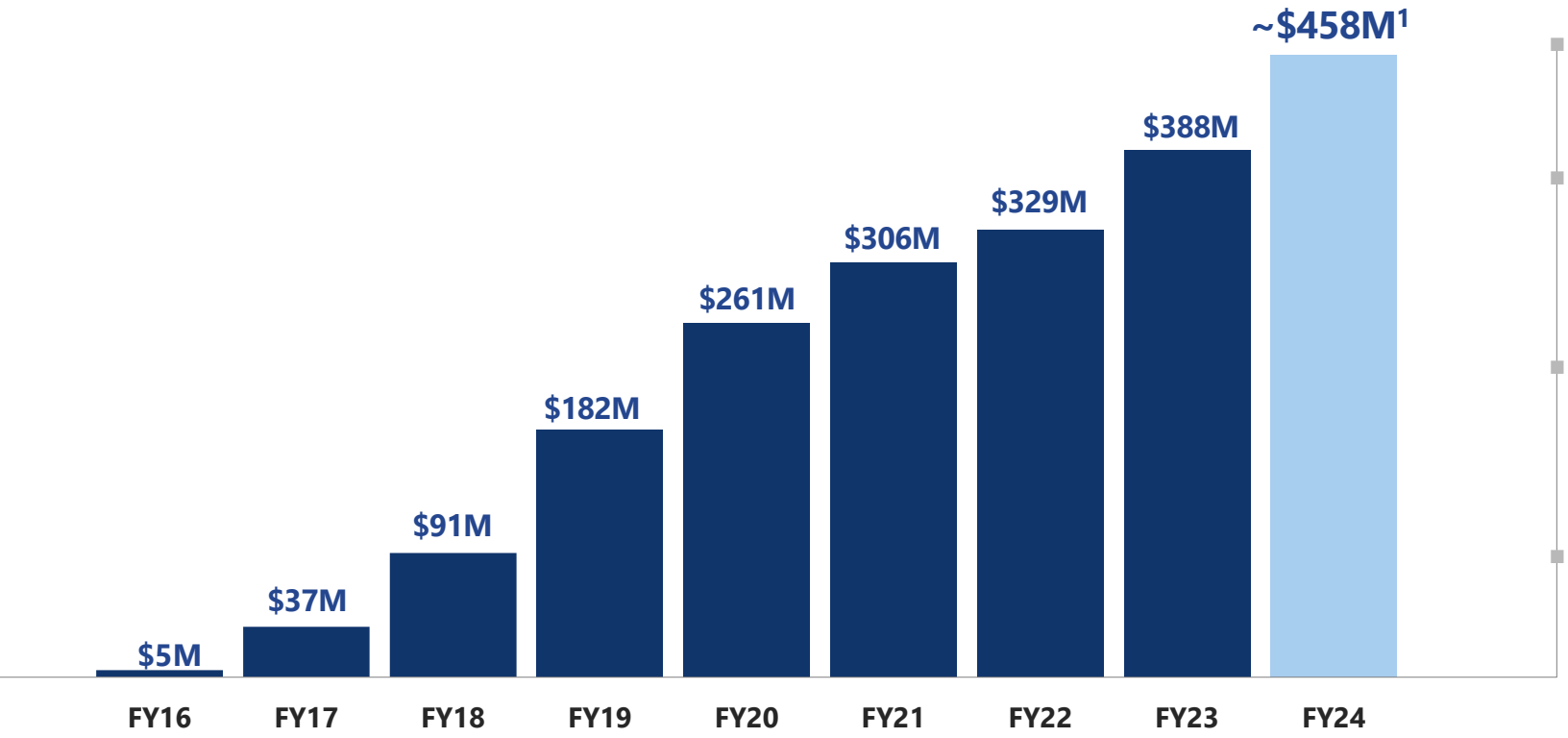
Galafold Performance

Growing patient demand in 2024 lays foundation for continued strong growth



- Global mix of naïve (~60%) and switch (~40%) patients[2]
- 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
- Expect non-linear quarterly growth to continue due to uneven ordering patterns and FX fluctuations

Strong FY 2025 Galafold growth guidance of 10-15% at CER[3]

[1] Preliminary and unaudited
[2] Data on file
[3] At CER: Constant Exchange Rates

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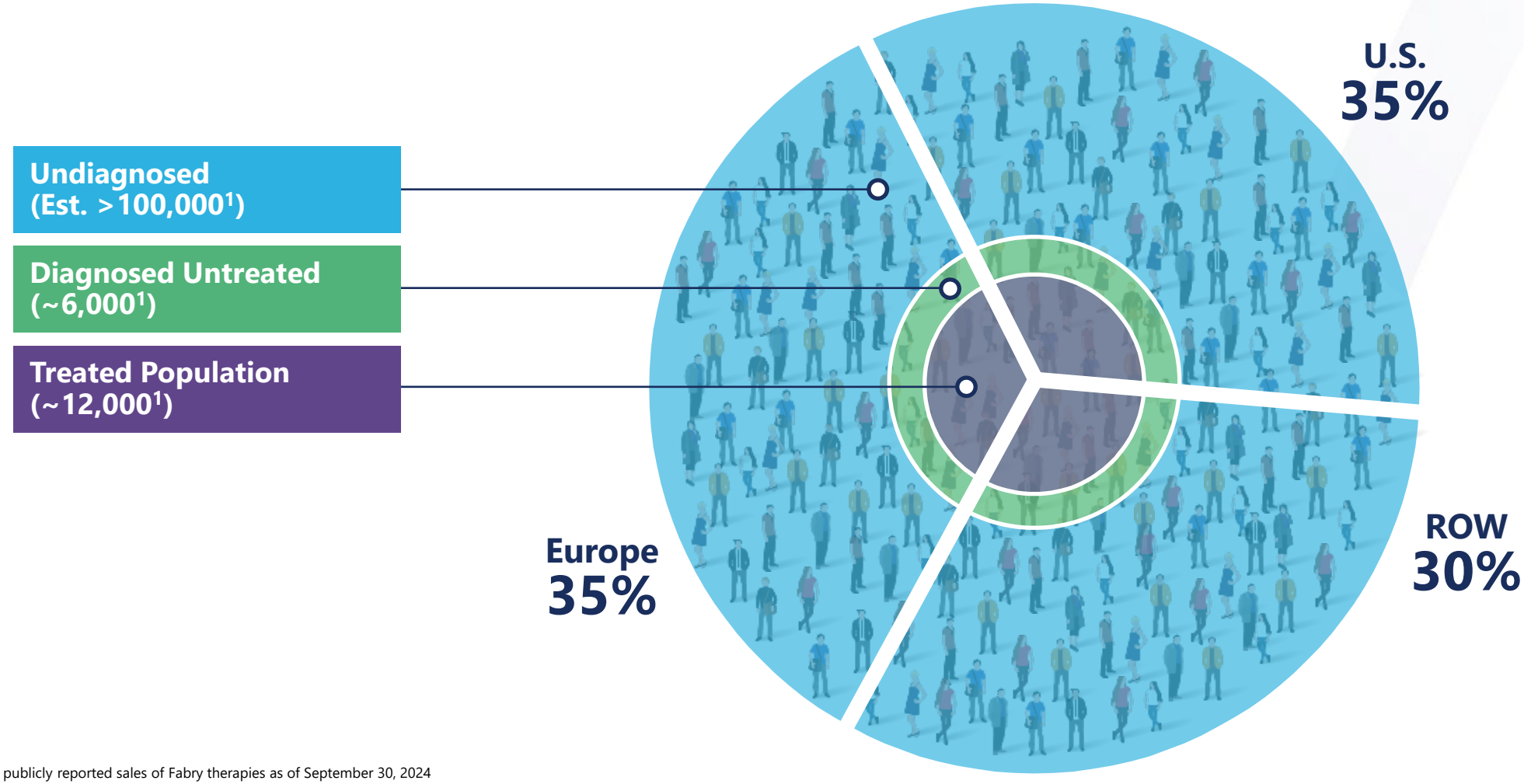
Fabry Market: Significant Remaining Unmet Need

Research suggests there could be >100k people living with Fabry disease who remain undiagnosed



Based on publicly reported sales of Fabry therapies as of September 30, 2024

[1]Applying incidence of multiple newborn screening studies to population estimates in commercial markets:
Burton 2017 J Pediatr 2017;190:130-5 ; Mechtler *et al.*, The Lancet, 2011 Dec.; Hwu *et al.*, Hum Mutation, 2009 Jun; Spada *et al.*, Am J Human Genet., 2006 Jul

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Improving Diagnosis of Fabry Disease

Multiple initiatives leveraging AI and family screening to drive Dx and address health inequities

Collaboration using AI to diagnose Fabry

OM1

Penn Medicine


Jefferies et al.
Orphanet Journal of Rare Diseases (2021) 16:518
https://doi.org/10.1186/s13023-021-02150-3

Orphanet Journal of Rare Diseases

RESEARCH Open Access

A new approach to identifying patients with elevated risk for Fabry disease using a machine learning algorithm

John L. Jefferies[1], Alison K. Spencer[2], Heather A. Lau[3], Matthew W. Nelson[4], Joseph D. Giuliano[4], Joseph W. Zabinski[2*], Costas Boussios[2], Gary Curhan[2], Richard E. Gliklich[2] and David G. Warnock[5]

- 580K+ medical records screened
- 100 people with highest risk of Fabry identified
- Outreach ongoing to offer genetic testing

Collaboration for change in health inequity

NHS

- Initial findings from Fabry pilot programs in U.K.
  - Minority and low-income groups significantly under-represented
  - >90% of diagnosed Fabry population white
  - ~85% from the least deprived areas
- Initiative already identified low-income families who otherwise wouldn't have been diagnosed

Additional initiatives in several countries ongoing leveraging AI and/or targeted screening

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Long-Term Outlook:
Clear Path to $1B+ Galafold Revenue

Many Thousands of Patients and

$1B+

in Peak Sales

The Next 12 Years

Today

~2,730
Patients

~$458M
Revenue

Global IP protection provides Galafold long runway to becoming a $1B+ product

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

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

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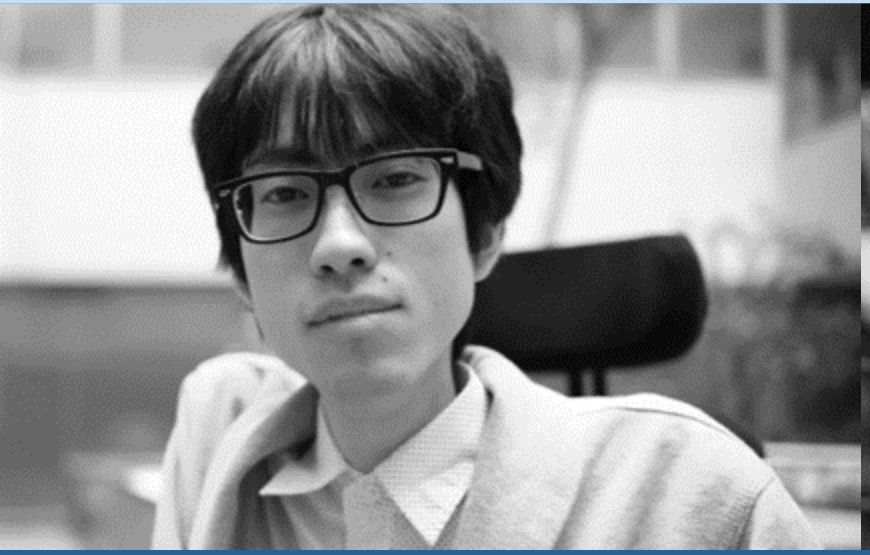
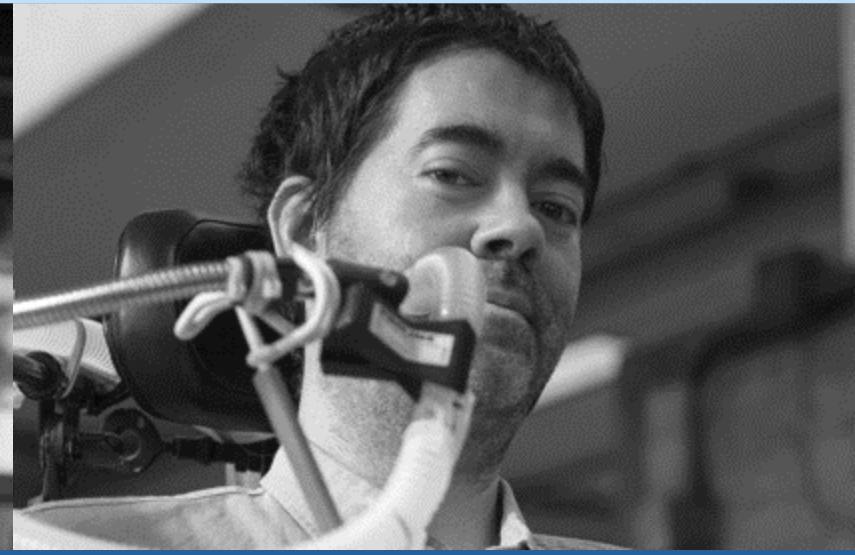
Pompe Disease Overview

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

Symptoms include systemic muscle weakness that worsens over time

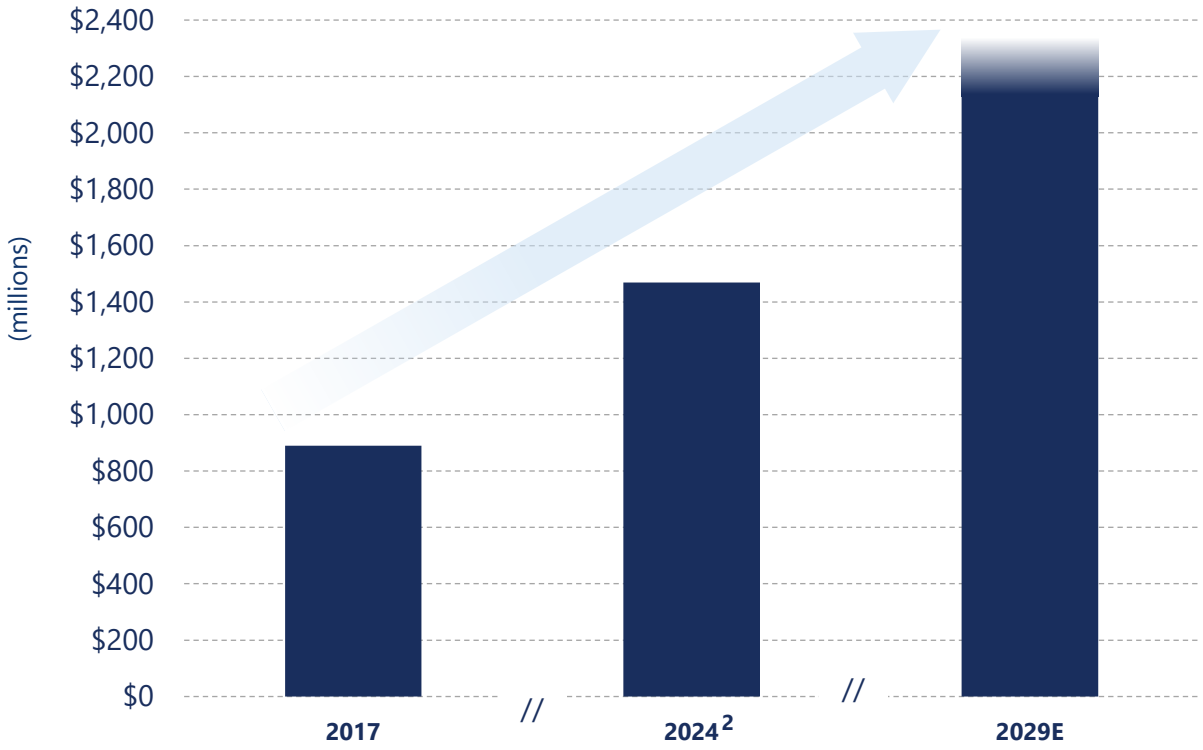
~$1.5B+ global Pompe ERT sales[1]

[1] Based on 12 months ended September 30, 2024

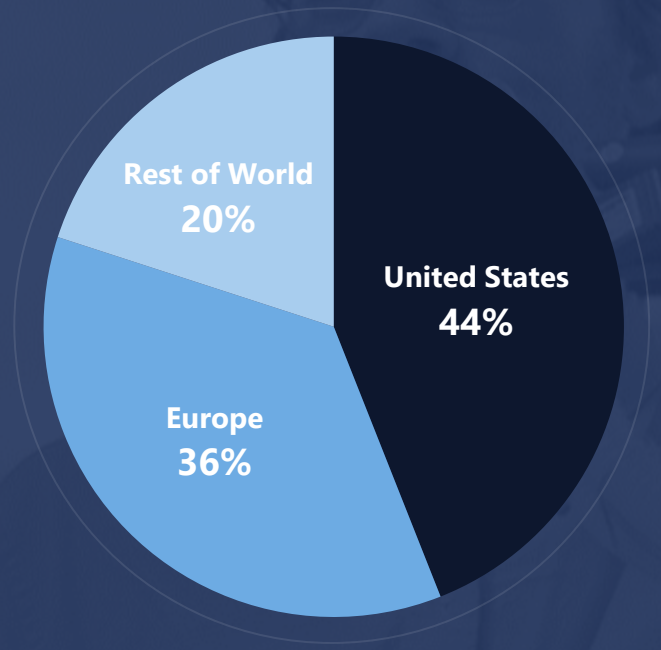
Global Pompe Market

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

Global Pompe Market of ~$1.5B in 2024 and Tracking toward $2B+ by 2029[1]



Global Pompe Market Sales Split YTD 2024[2]



An estimated >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, 2024
[3] Amicus Data on File from Market Mapping

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Pombiliti + Opfolda Profile

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

- Differentiated **mechanism of action** combining cipaglucosidase alfa-atga, an ERT, with miglustat, an orally administered enzyme stabilizer
- Only Pompe therapy with **Phase 3** study that included **ERT-experienced** patients
- Phase 3 PROPEL study demonstrated mean improvement in **6MWD** and stabilization in **FVC** in patients switching from SoC

ERT: Enzyme Replacement Therapy

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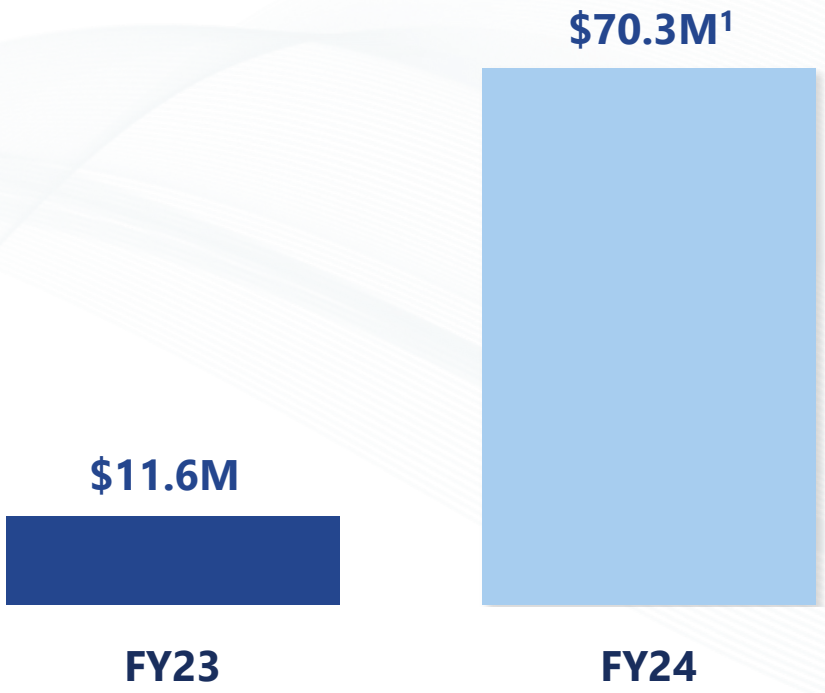
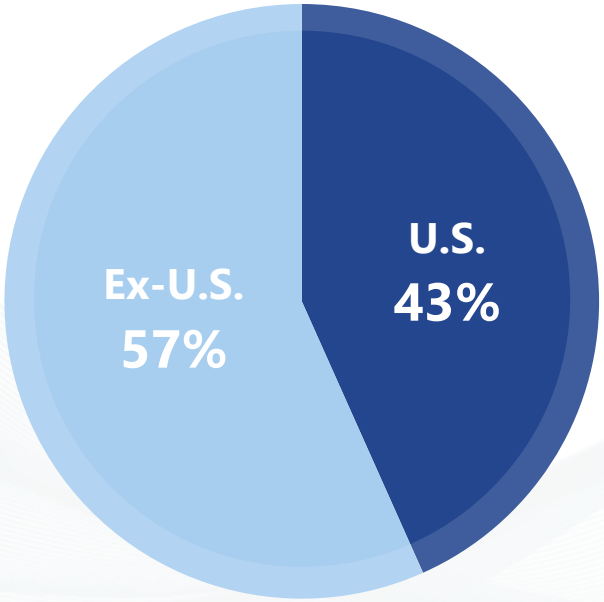
Pombiliti + Opfolda Performance

Successful first full year of launch with revenue of $70.3M sets foundation for 2025

Annual Revenue



2024 Geographic Mix



~50/50 revenue split between U.S. and ex-U.S. exiting 2024

[1] Preliminary and unaudited

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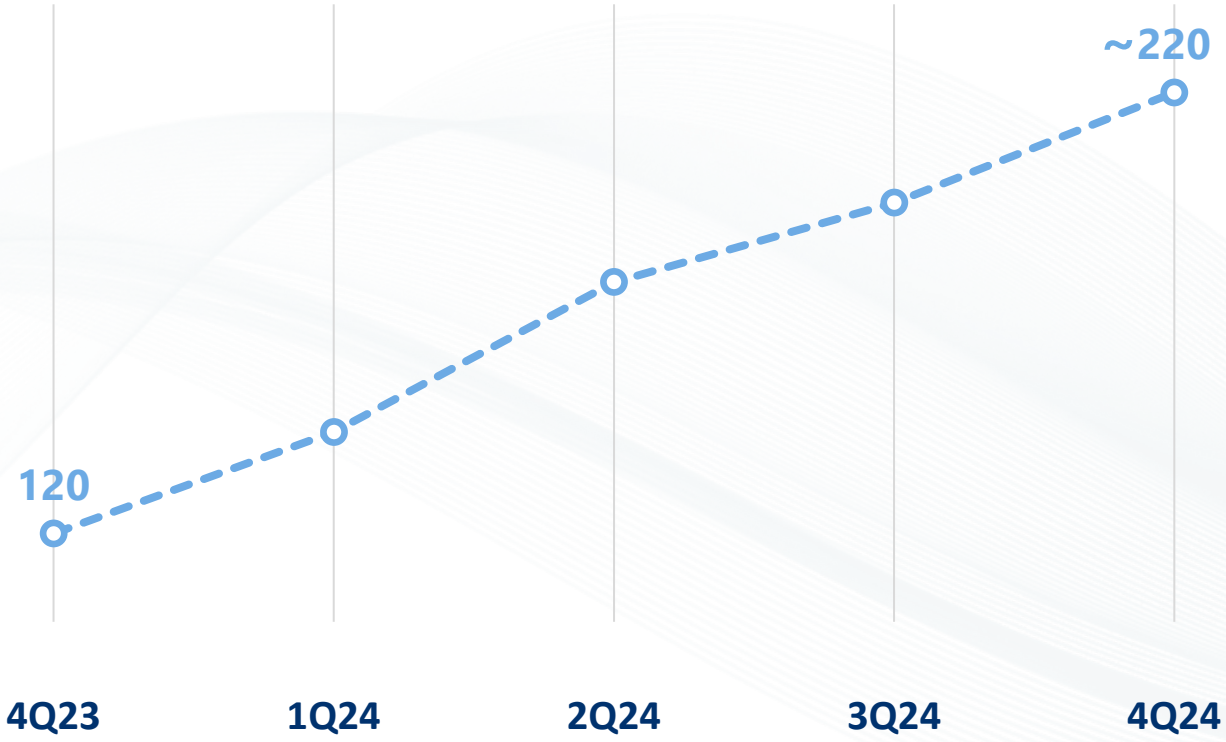
Pombiliti + Opfolda Global Launch Metrics

~220 Individuals Treated or Scheduled Provides Strong Foundation for 2025

Global Launch: Individuals Treated or Scheduled



- **~220** patients have been treated or scheduled to be treated with commercial product
  - ~209 treated patients
  - ~25 new prescriptions in Q4
- All eligible clinical trial patients from launched markets on commercial therapy by end of 1H24
- New commercial patients time through U.S. insurance process optimized to <30 days
- Patients starting Pombiliti + Opfolda at proportional rate to the respective market share

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Geographic Expansion

3 new regulatory approvals and up to 10 new launch countries in 2025

Regulatory

- 3 additional regulatory approvals anticipated in 2025

Reimbursement

- In 2025, expect to launch in up to 10 new countries, including 4 recent agreements
  - >650 LOPD patients 18+ in those 10 countries
- First commercial patients from those new launch countries anticipated over H1 2025
- Anticipate >20 individuals switching from clinical trials or early access programs in new countries in 2025

Regulatory approvals anticipated in 2025:


JAPAN


AUSTRALIA


CANADA

Combined ~150-200 people 18+ living with LOPD and being treated with a Pompe therapy

New reimbursement agreements completed in:


ITALY


CZECH REPUBLIC


SWITZERLAND


SWEDEN

Combined ~200-250 people 18+ living with LOPD and being treated with a Pompe therapy

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Ongoing Clinical Studies

Continuing to build the body of evidence to support planned label expansion

ZIP

Clinical study in children with late-onset Pompe disease (LOPD)

ROSSELLA

Clinical study in children with infantile-onset Pompe disease (IOPD)

followME Pompe Journey

Amicus registry adding to evidence on differentiated MOA and long-term effect

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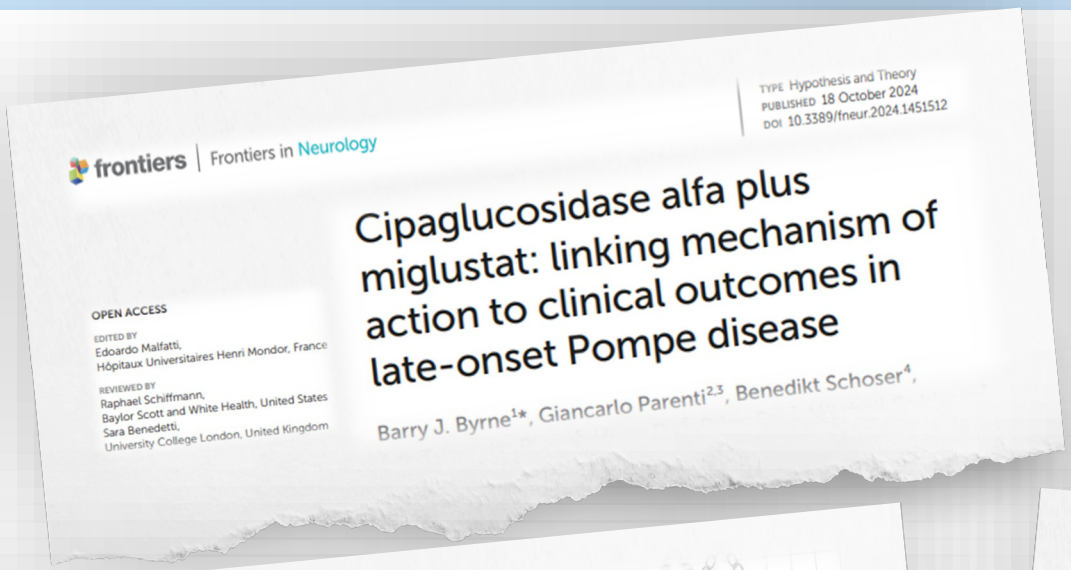
Pombiliti + Opfolda Real-World Evidence

Ongoing publications demonstrate the impact of Pombiliti + Opfolda's differentiated MOA on long-term outcomes


frontiers | Frontiers in Neurology

TYPE Hypothesis and Theory
PUBLISHED 18 October 2024
DOI 10.3389/fneur.2024.1451512

OPEN ACCESS

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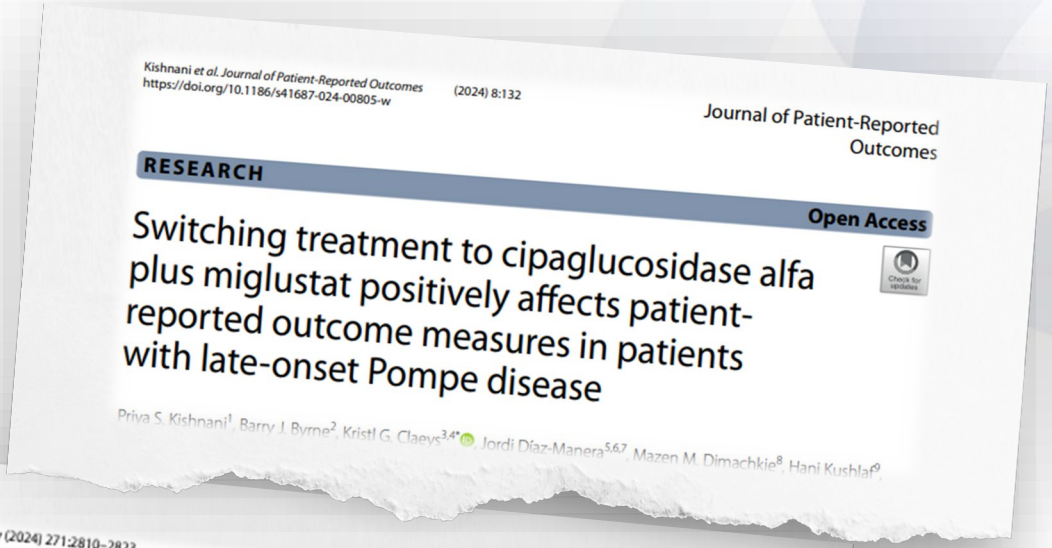
Cipaglucosidase alfa plus miglustat: linking mechanism of action to clinical outcomes in late-onset Pompe disease

Barry J. Byrne[1]*, Giancarlo Parenti[2,3], Benedikt Schoser[4],


Kishnani et al. Journal of Patient-Reported Outcomes
https://doi.org/10.1186/s41687-024-00805-w (2024) 8:132

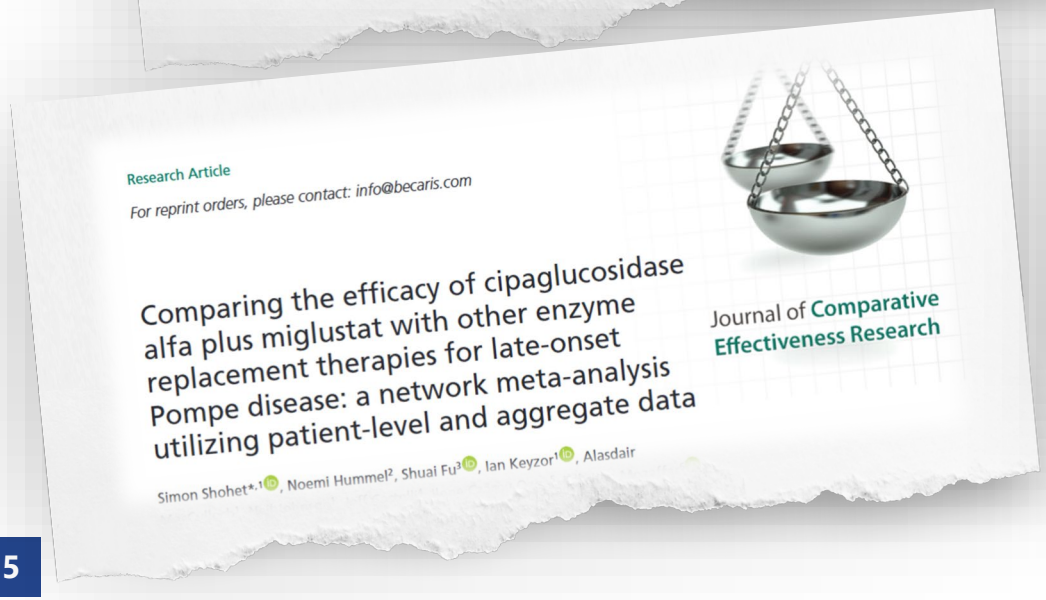
Journal of Patient-Reported Outcomes

RESEARCH

Open Access

Switching treatment to cipaglucosidase alfa plus miglustat positively affects patient-reported outcome measures in patients with late-onset Pompe disease

Priya S. Kishnani[1], Barry J. Byrne[2], Kristl G. Claeys[3,4*], Jordi Díaz-Manera[5,6,7], Mazen M. Dimachkie[8], Hani Kushlaf[9],

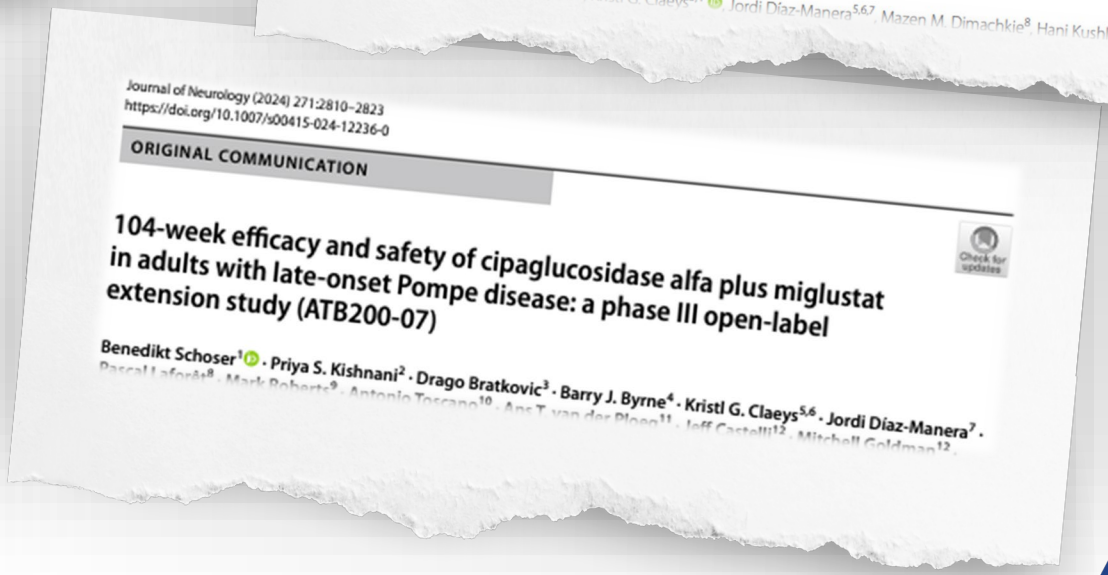

Research Article

For reprint orders, please contact: info@becaris.com

Comparing the efficacy of cipaglucosidase alfa plus miglustat with other enzyme replacement therapies for late-onset Pompe disease: a network meta-analysis utilizing patient-level and aggregate data

Journal of Comparative Effectiveness Research

Simon Shohet*[,1], Noemi Hummel[2], Shuai Fu[3], Ian Keyzor[1], Alasdair


Journal of Neurology (2024) 271:2810–2823
https://doi.org/10.1007/s00415-024-12236-0

ORIGINAL COMMUNICATION

104-week efficacy and safety of cipaglucosidase alfa plus miglustat in adults with late-onset Pompe disease: a phase III open-label extension study (ATB200-07)

Benedikt Schoser[1] · Priya S. Kishnani[2] · Drago Bratkovic[3] · Barry J. Byrne[4] · Kristl G. Claeys[5,6] · Jordi Díaz-Manera[7] ·

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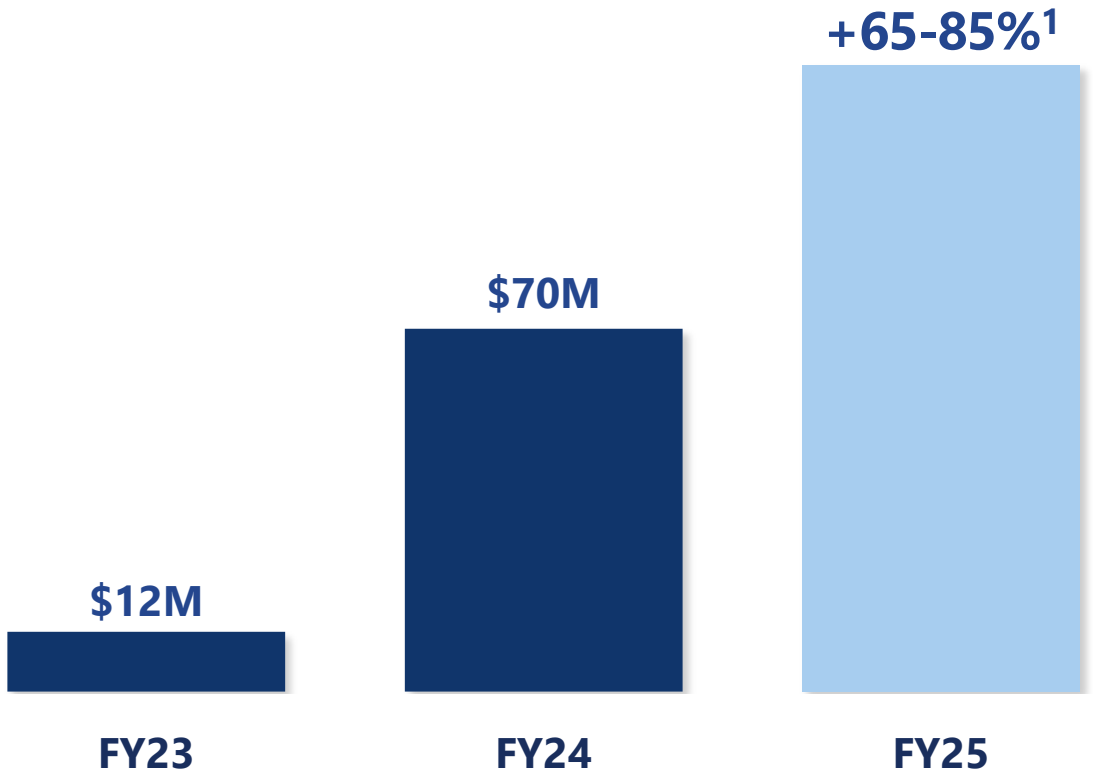
Pombiliti + Opfolda Growth Drivers

Amicus focused on key drivers of growth in 2025

Pombiliti + Opfolda Revenue



- Increasing number of net new patients
- Increasing depth and breadth of prescribers
- Expect to launch in up to 10 new countries throughout 2025
- Continuing to drive differentiation through evidence generation and real-world evidence
- Anticipate 90%+ compliance and adherence

[1] At CER: Constant Exchange Rates

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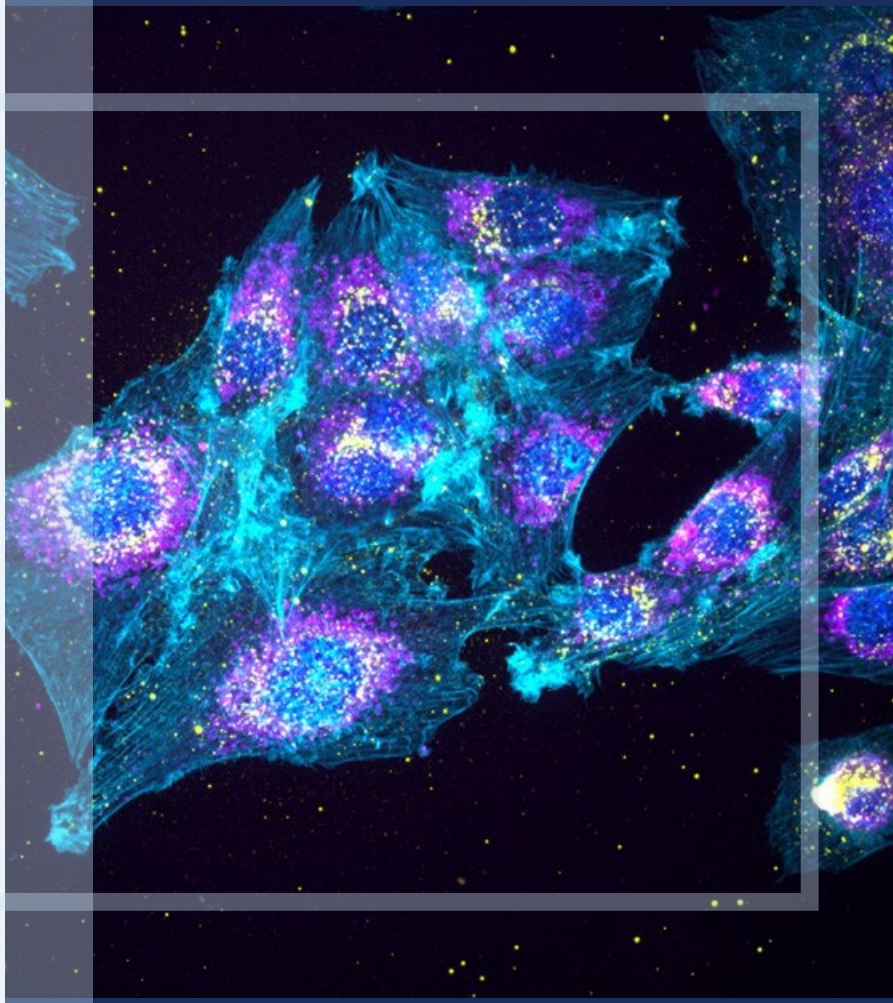
Corporate Outlook

Delivering on our mission for patients and shareholders



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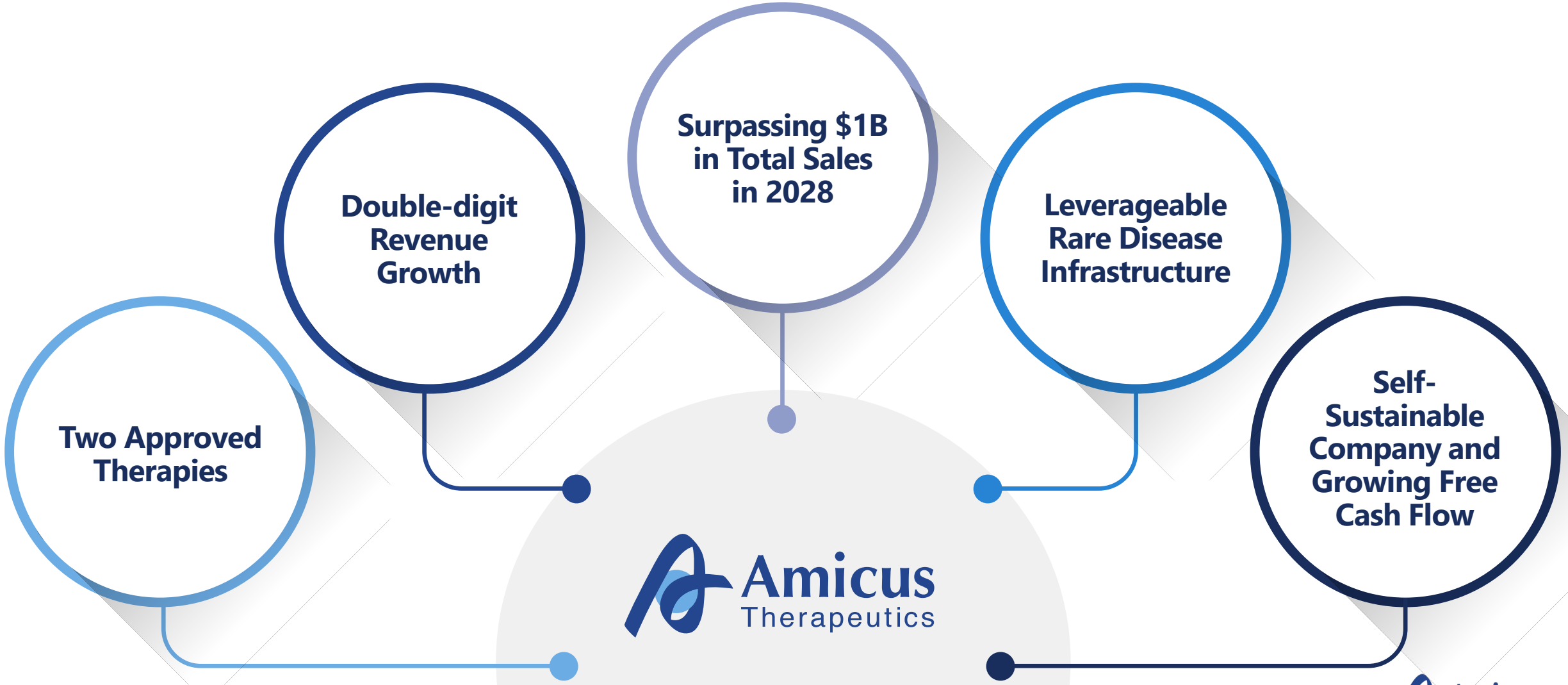
A Rare Company

A unique story in biotech with significant revenue growth and profitability



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

>350 Patients*

YE17

>3,000 Patients*

YE24

Many Thousands of Patients*

2025+

*Clinical, early access, and commercial, all figures approximate

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Thank You

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Appendix

Full-Year 2025 Revenue Guidance and Sensitivity

FY 2025 Revenue Guidance[1]	2025
Total Revenue Growth[1]	17% to 24%
Galafold Revenue Growth[1]	10% to 15%
Pombiliti + Opfolda Revenue Growth[1]	65% to 85%

[1] Full-Year 2025 guidance is provided at CER (Constant Exchange Rates) using Full-Year 2024 Average Exchange Rates

FY 2025 Revenue Sensitivity

Given the proportion of Amicus revenue ex-US (**~60%** in 2024), a change in USD exchange rates of **+/- 1%** compared to year-end 2024 rates could lead to a **~$3.6M** move in Total Reported Revenues in 2025

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Exchange Rates

Q4 2024 Currency Average Rates

FX Rates	Q4 2023	Q4 2024	Variance
USD/EUR	1.076	1.067	(0.8%)
USD/GBP	1.241	1.282	3.3%
USD/JPY	0.007	0.007	(3.0%)

Year-End 2024 vs. Full-Year 2024 Currency Average Rates[1]

FX Rates	Year-End 2024	Full-Year 2024	Variance
USD/EUR	1.041	1.082	(3.8%)
USD/GBP	1.255	1.278	(1.8%)
USD/JPY	0.006	0.007	(3.5%)

[1] The variance between **Year-End 2024** and **Full-Year 2024** USD exchange rates of **~4%** would translate into a negative impact of **~$15M** on Total Revenue in 2025 if rates were to remain at Year-End 2024 level

Distribution of Quarterly Sales

Distribution of Galafold® Revenue by Quarter over Past 5 Years

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

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

(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 December 31, 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

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