AT THE FOREFRONT OF
THERAPIES FOR RARE DISEASES

Corporate Overview

December 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 approval of our products, commercialization plans, manufacturing and supply plans, financing plans, the collaboration with Dimerix, 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, 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 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 the Dimerix license agreement for of DMX-200 may not be successful, including without limitation expectations of the timing of the Phase 3 clinical trial evaluating DMX-200; the likelihood of success of such clinical trial; the prospects for FDA approval of DMX-200 for FSGS or other indications; the estimated prevalence of FSGS; the achievement of any milestone and timing of any payments associated with milestones and the success of any efforts to commercialize DMX-200, including any projections of future financial performance or payments; the potential that we may not be able to manufacture or supply sufficient commercial products; and the potential that we will need additional funding to complete 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, GAAP and 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, 2024 and our Quarterly Report on Form 10-Q 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.

Amicus Therapeutics

Our Passion is for Patients

Our Mission:

To develop and deliver transformative medicines for people living with rare diseases.

Our Vision:

To become one of the leading rare disease focused biotech companies.

Definition:

\əˈmēkəs (noun) *Latin* Friend

Amicus Therapeutics

A Rare Company

A unique story in biotech with significant revenue growth and profitability

First Oral Precision Medicine for Fabry Disease

Galafold® (migalastat)

First Two-Component Therapy for Pompe Disease

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

10-15%

FY 2025 Galafold Revenue Growth[1]

50-65%

FY 2025 Pombiliti + Opfolda Revenue Growth[1]

Expanded Portfolio with U.S. Licensing of DMX-200 Phase 3 Program

Leverageable Global Commercial Organization

$169M

Q3 2025 Total Revenue (+17% Growth)[1]

$1B+

Total Revenue Expected in FY 2028

[1] At CER: Constant Exchange Rates

2025 Strategic Priorities

1. Deliver total revenue growth of 15-22% at CER[1]
2. Double-digit Galafold® revenue growth of 10-15% at CER[1]
3. Pombiliti® + Opfolda® revenue growth of 50-65% at CER[1]
4. Advance ongoing studies in Fabry, Pompe and FSGS
5. Deliver positive GAAP net income during H2 2025

[1] CER: Constant Exchange Rates

Amicus Therapeutics

Galafold® *(migalastat)* Continued Growth

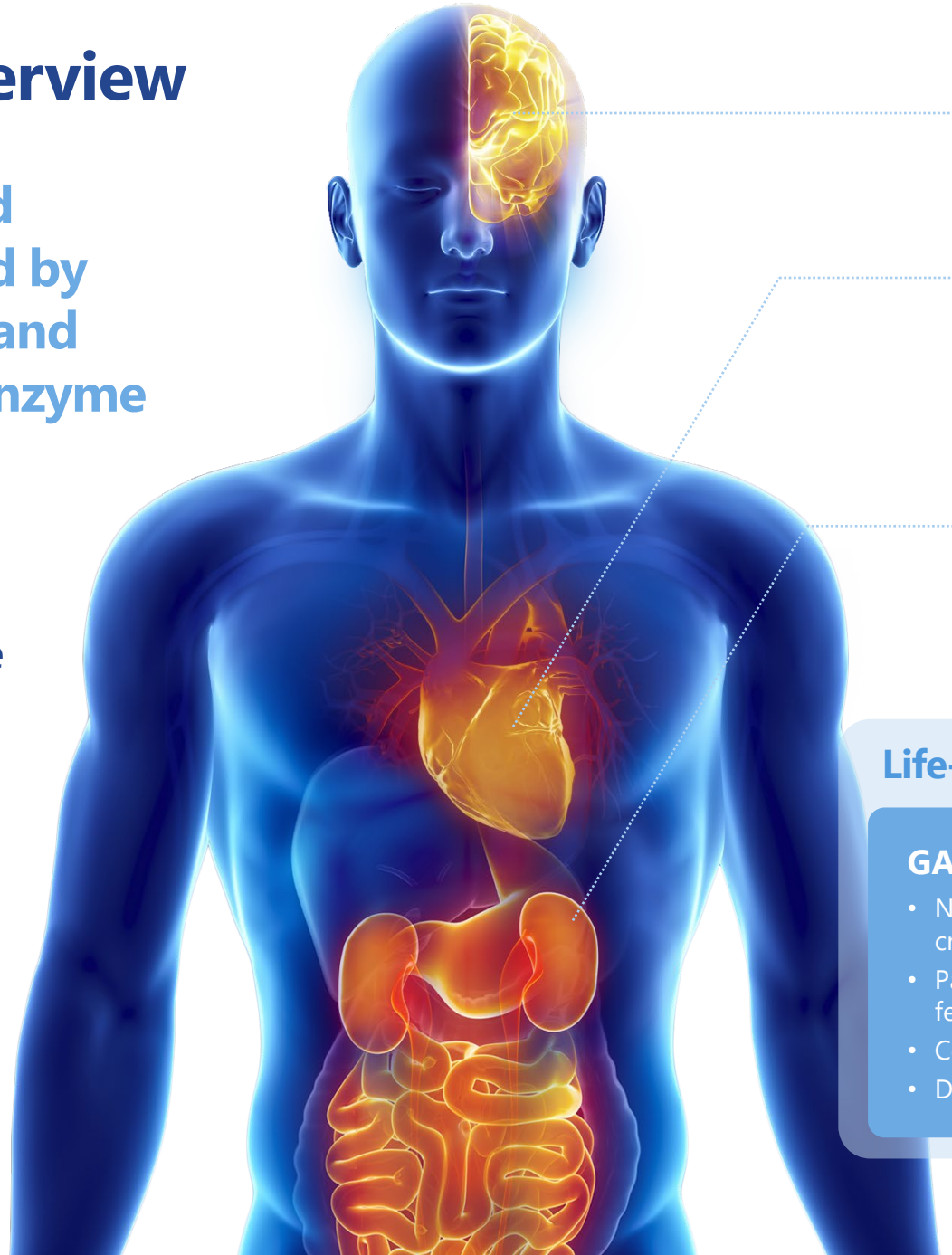
Building a leadership position
in the treatment of Fabry disease

Amicus Therapeutics

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]

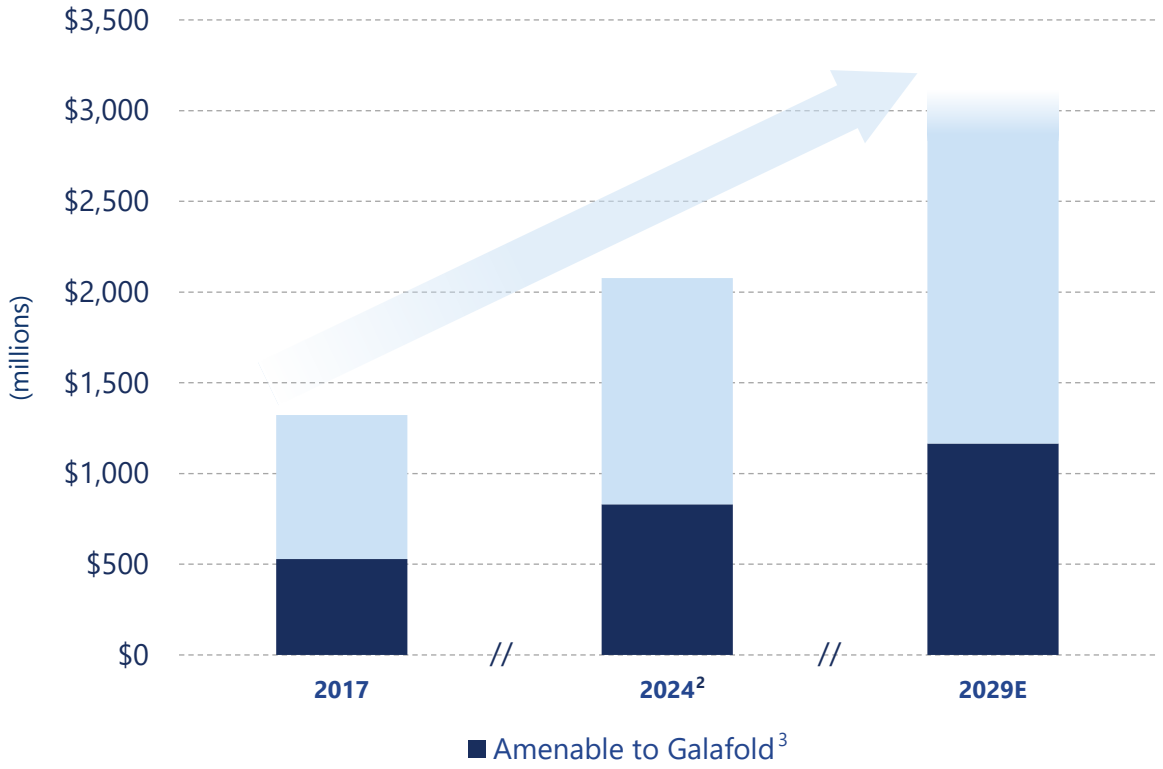
[1] Desnick R, *et al.* Ann Intern Med. 2003
[2] Yousef Z, *et al.* Eur Heart J. 2013
[3] Germain D. Orphanet J Rare Dis. 2010

Amicus Therapeutics

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]



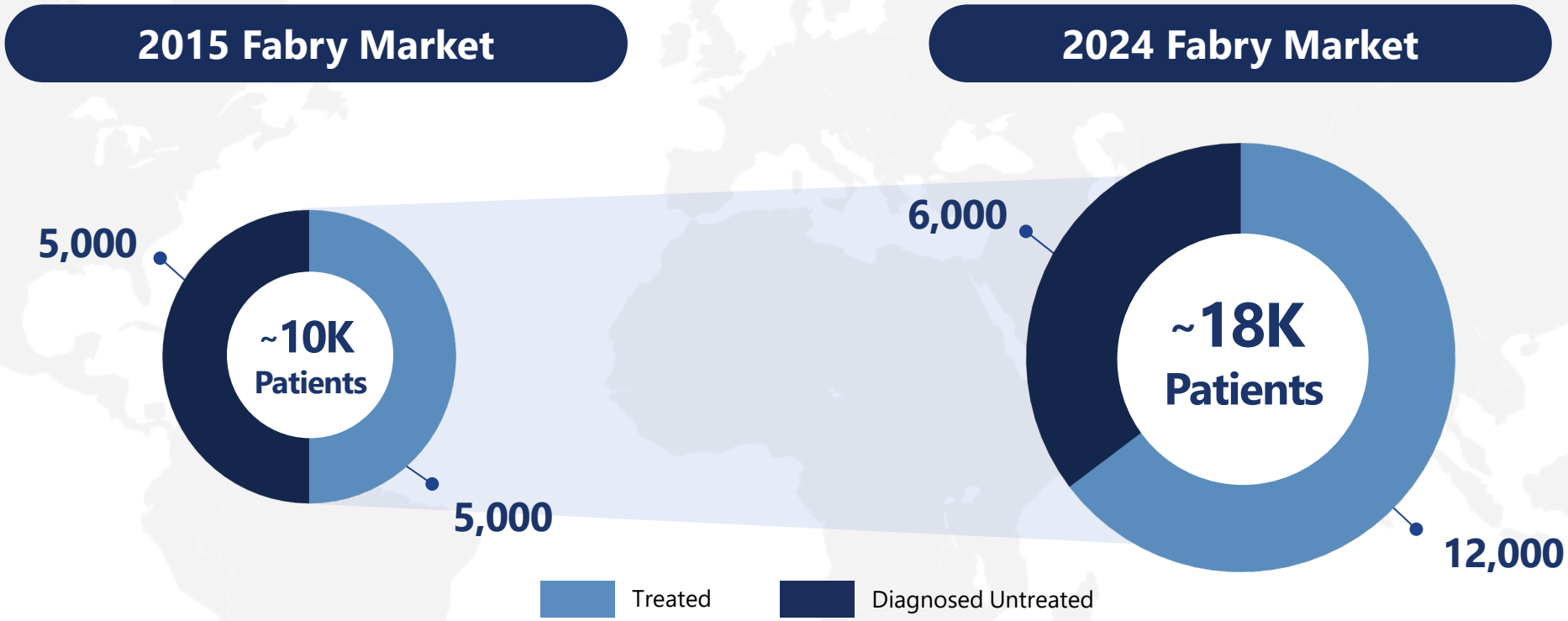
- Significantly underdiagnosed
  - Newborn screening studies suggest Fabry is one of the more prevalent rare genetic diseases (~1:1,500 to ~1:4,000 incidence)[4]
- Continued market growth driven by increased diagnosis
- Anticipate market size for amenable patients to surpass $1B in 2029
- Galafold continues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

Amicus Therapeutics

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

Amicus Therapeutics

2025 Galafold Success (as of September 30, 2025)

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

$138.3M
Q3 25 Galafold Revenue

69%
Share of Treated Amenable Patients

+15%
Q3 25 Reported Sales Growth

~2,730
Individuals Treated[1]

35-50%
Fabry Patients Amenable to Galafold

40+
Countries with Regulatory Approvals

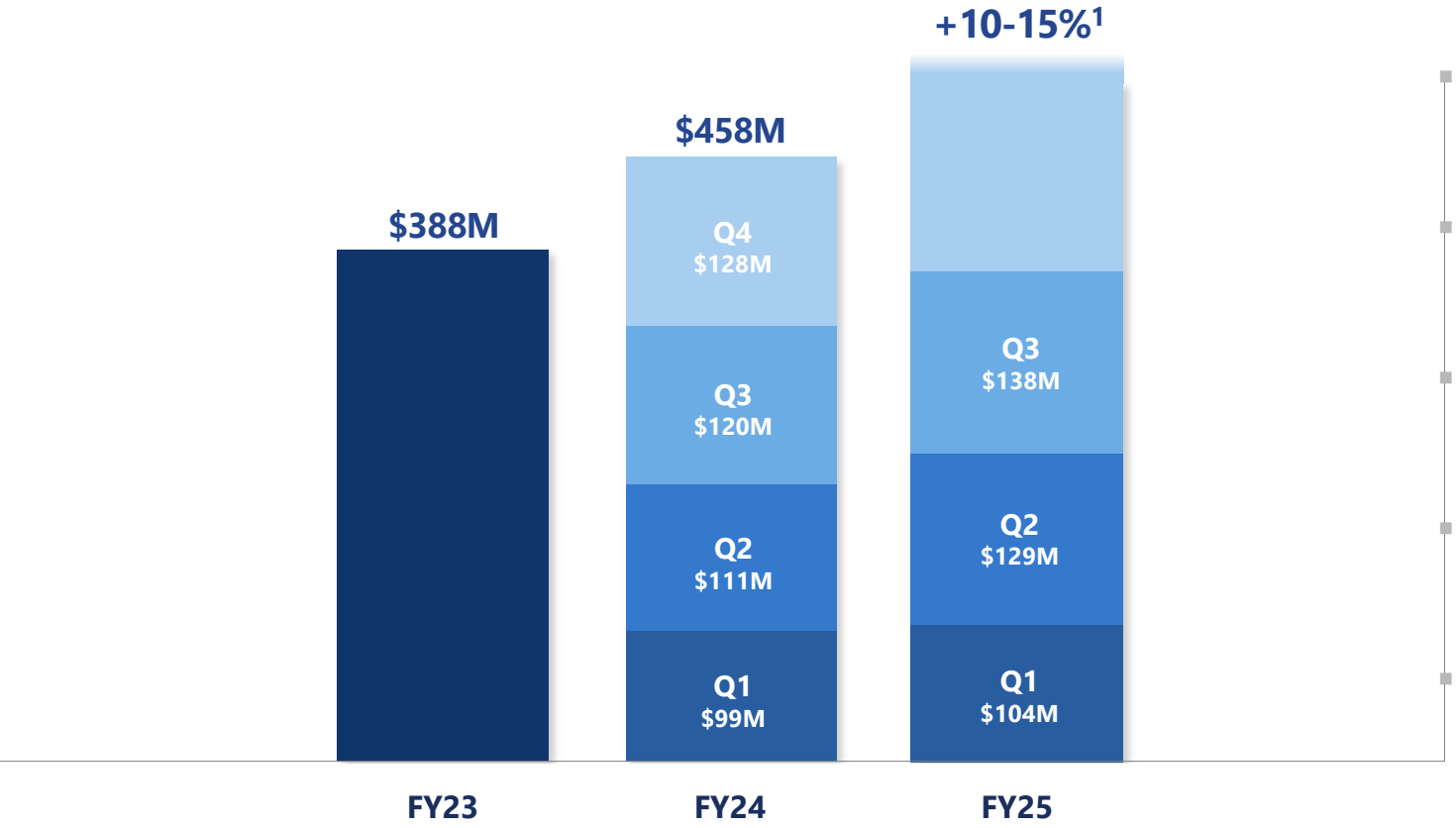
Galafold is indicated for adults with a confirmed diagnosis of Fabry disease and an amenable variant. The most common adverse reactions reported with Galafold (≥10%) were headache, nasopharyngitis, urinary tract infection, nausea, and pyrexia. For additional information about Galafold, including the 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] As of YE 2024

Amicus Therapeutics

Galafold Performance

Q3 2025 Galafold reported revenue of $138.3M (+12% at CER)



- Quarterly patient starts remain strong: +13% growth YoY in Q3 2025
- Q3 2025 one of the highest demand quarters and best number of starts YTD since launch
- Global mix of naïve (~65%) and switch (~35%) patients[2]
- Expanding market through uptake in naïve population as well as label and geographic expansion
- Maintaining >90% adherence and compliance through HCP and patient education and support

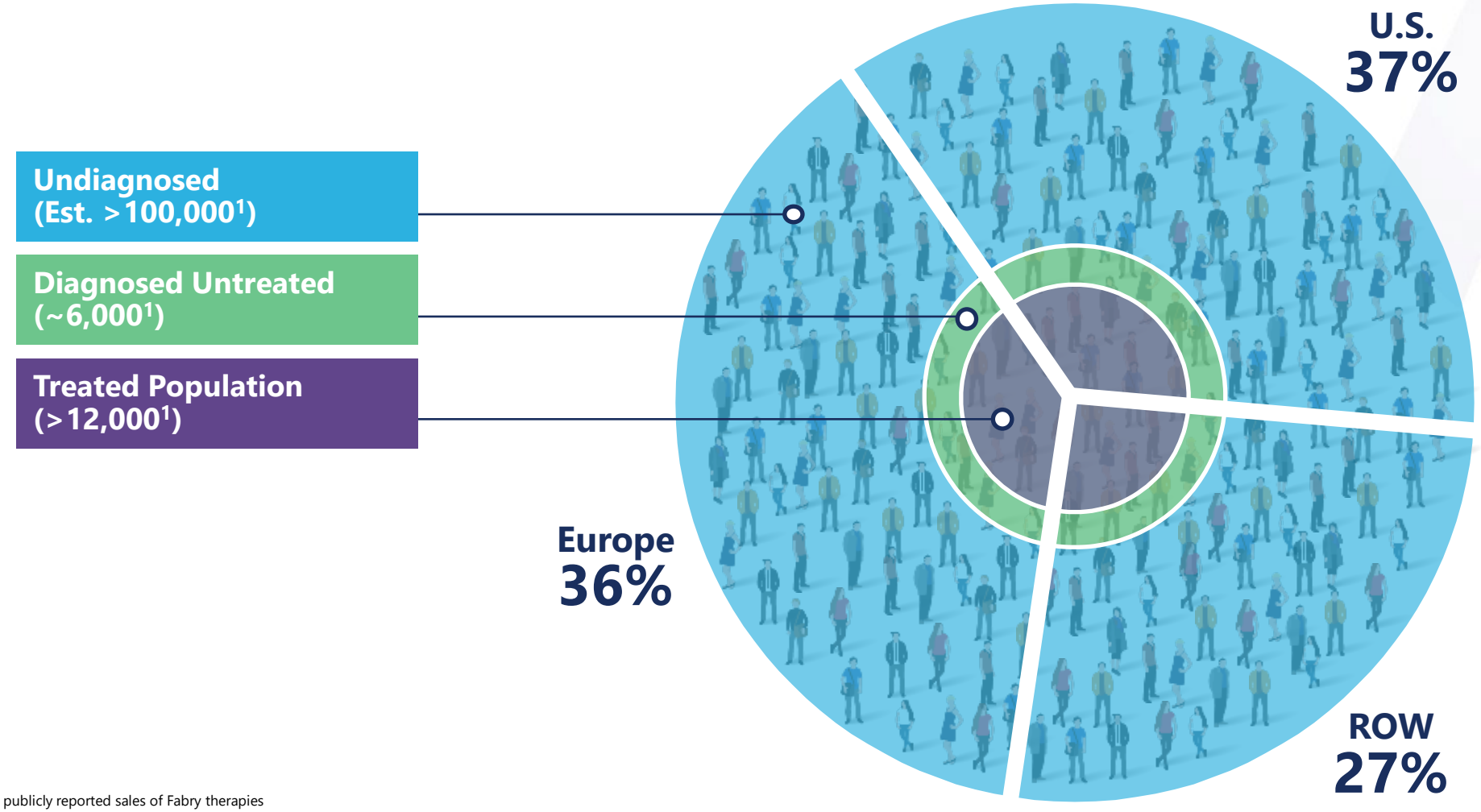
Reiterating FY 2025 Galafold growth guidance of 10-15% at CER

[1] CER: Constant Exchange Rates
[2] Data on file

Amicus Therapeutics

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

[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

Amicus Therapeutics

Improving Diagnosis of Fabry Disease

Multiple initiatives leveraging AI and family screening to improve diagnosis of Fabry disease

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

Amicus Therapeutics

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[1]

Patients

$458M

Revenue

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

[1] As of YE 2024

Amicus Therapeutics

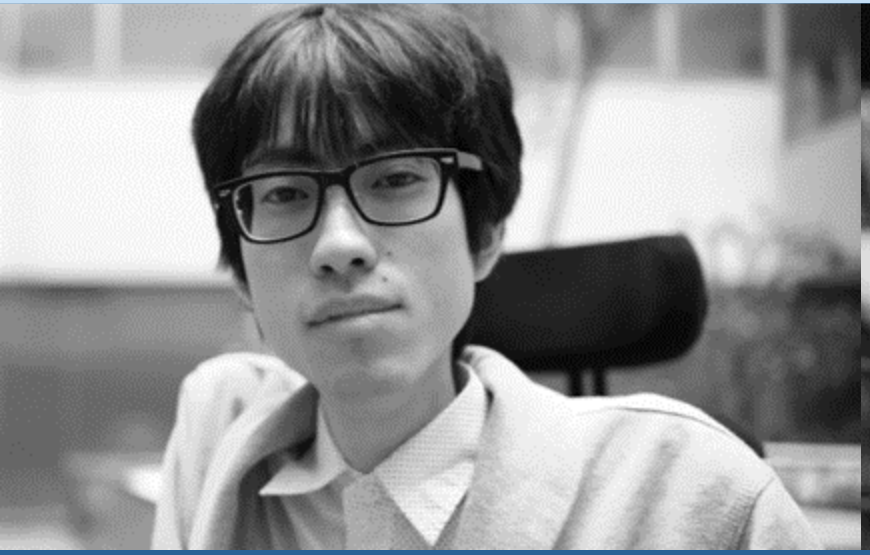
Pombiliti® *(cipaglucosidase alfa-atga)*
+
Opfolda® *(miglustat)*

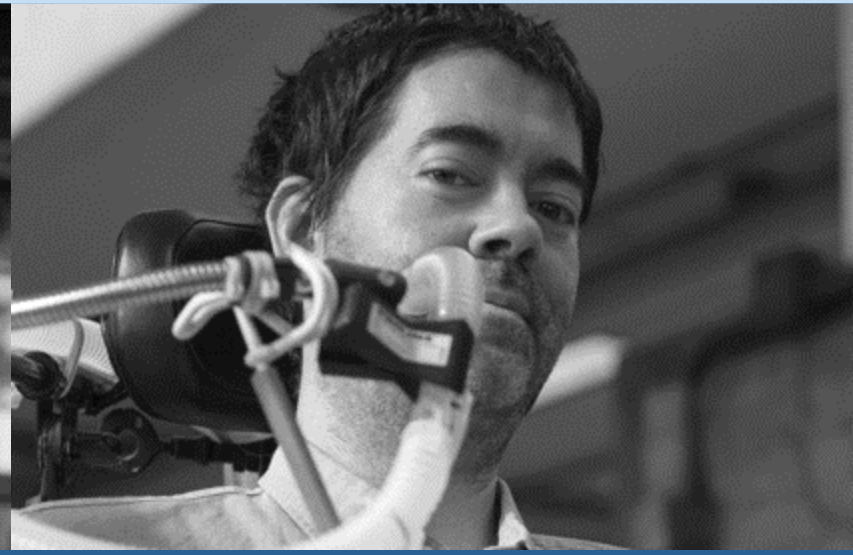
Potential to establish a new standard of care
for people living with late-onset Pompe disease

Amicus Therapeutics

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





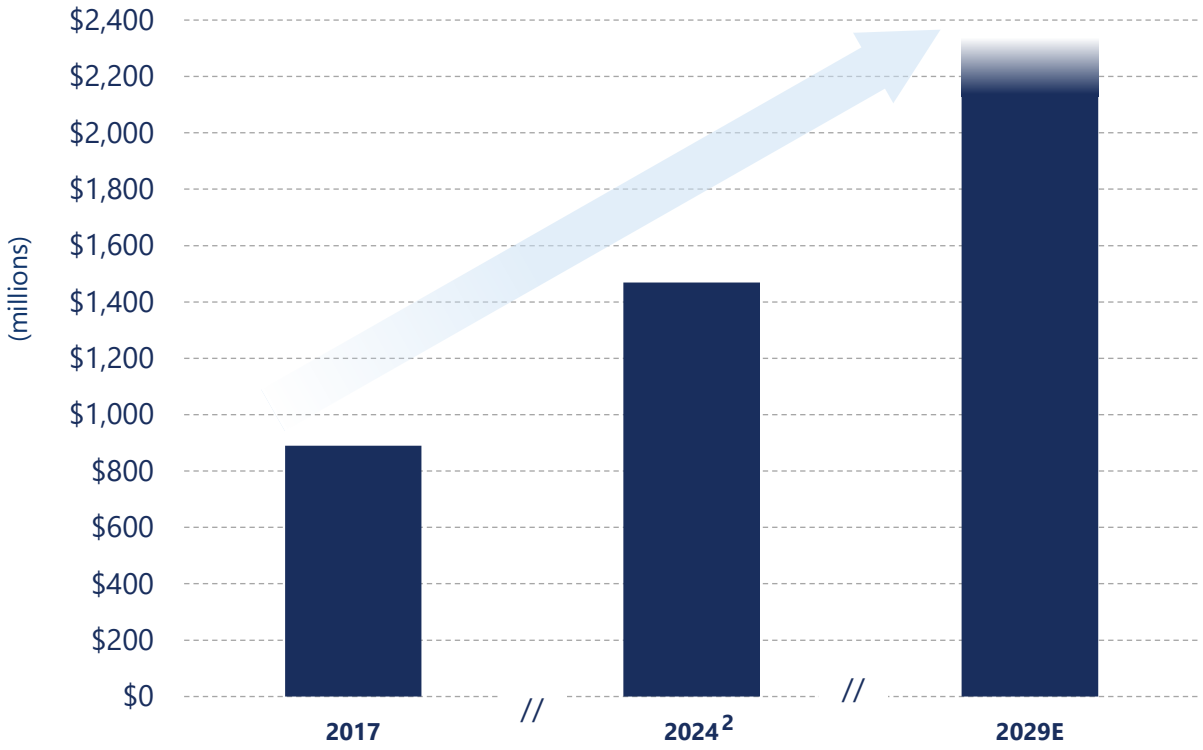
- ~5,000-10,000 people diagnosed globally
- Deficiency of GAA leading to lysosomal glycogen accumulation and cellular dysfunction
- Significantly underdiagnosed
- Respiratory failure is major cause of mortality
- Symptoms include systemic muscle weakness that worsens over time
- ~$1.5B+ global Pompe ERT sales[1]

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

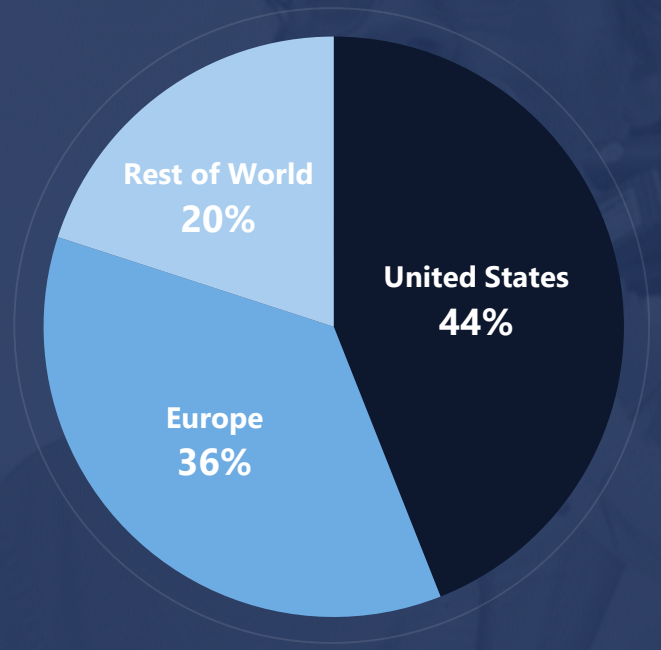
Global Pompe Market

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

Amicus Therapeutics

Pombiliti + Opfolda Profile

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

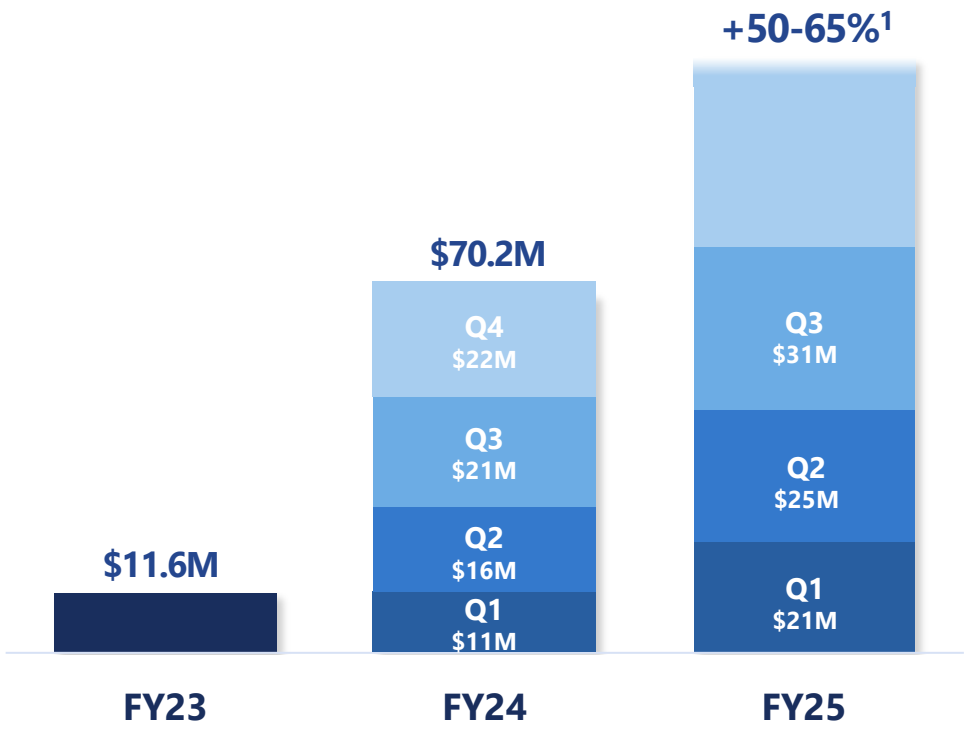
- Differentiated **mechanism of action** combining cipaglucosidase alfa-atga, an ERT, with miglustat, an orally administered enzyme stabilizer
- Only Pompe therapy with **Phase 3** study that included **ERT-experienced** patients
- Phase 3 PROPEL study demonstrated mean improvement in **6MWD** and stabilization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

ERT: Enzyme Replacement Therapy

Amicus Therapeutics

Pombiliti + Opfolda Performance

Q3 2025 Pombiliti + Opfolda reported revenue of $30.7M (+42% at CER)



- Strong Q3 sales growth YoY
  - Increasing depth and breadth of prescribers
  - Doubled number of naïve starts (ex-U.S) in first 9 months of 2025 vs FY 2024
  - Doubled number of avalglucosidase alfa switches in first 9 months of 2025 vs FY 2024
- H2 2025 growth driven by:
  - Benefit of new patient starts in existing and newly launched markets
  - Growing body of RWE[2] supporting switch from both alternative therapies

Reiterating FY 2025 Pombiliti + Opfolda growth guidance of 50-65% at CER[1]

[1] CER: Constant Exchange Rates
[2] Real-World Evidence

Amicus Therapeutics

Pombiliti + Opfolda Expansion

Significant progress made towards expanding access to Pombiliti + Opfolda in 2025

Regulatory

- Approved in Australia and Canada in Q1 2025
- Approved in Japan in June 2025

Regulatory approvals in 2025:


AUSTRALIA


CANADA


JAPAN

Reimbursement

- Now reimbursed in 15 countries with 10 countries added in 2025 providing access to ~2,400 treated patients
- First commercial patients in 8 new countries YTD
- Pombiliti + Opfolda selected as preferred treatment for adults with LOPD in the Netherlands

Reimbursement agreements completed in 2025:


SWEDEN


SWITZERLAND


ITALY


JAPAN


IRELAND


NETHERLANDS


PORTGUAL


CZECH REPUBLIC


BELGIUM


LUXEMBOURG

Amicus Therapeutics

Pombiliti + Opfolda Body of Evidence

Growing number of abstracts, manuscripts, and case studies supporting Pombiliti + Opfolda differentiation

Clinical Trials & Long-Term Data

- Long-term Phase 1/2 open-label safety and efficacy study (ATB200-02)
- 104-week Phase 3 open-label extension study of efficacy and safety (ATB200-07)

Mechanistic & Translational Insights

- Miglustat: a first-in-class enzyme stabilizer for LOPD
- Linking mechanism of action to clinical outcomes in LOPD

Comparative & Real-World Data

- Network meta-analysis comparing the efficacy of cipaglucosidase alfa + miglustat with other ERTs
- U.K. EAMS[1] registry post-baseline outcomes
- CDMU–UCLH[2] cohort analysis comparing cipaglucosidase alfa + miglustat vs. avalglucosidase alfa

Case Studies & Real-World Reports

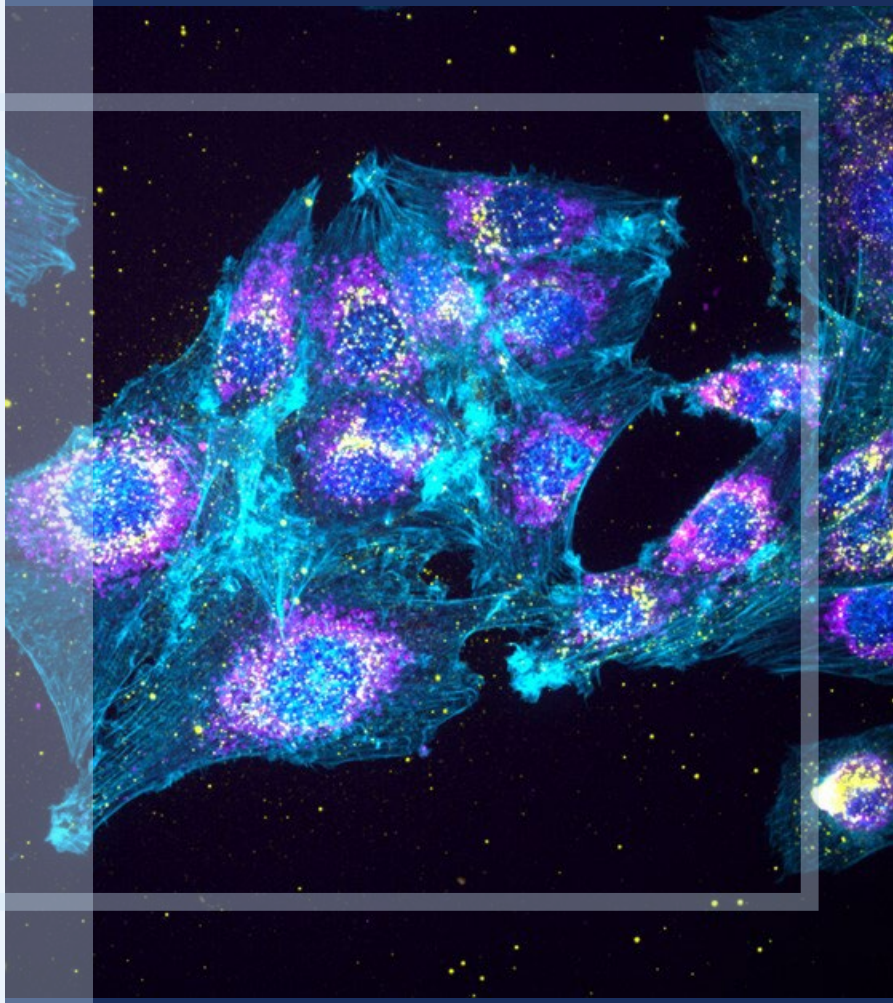
- Case studies supporting the switch from both alternative therapies
- Case studies of patients switching from high dose, high frequency alglucosidase alfa

[1] EAMS: Early Access to Medicines Scheme
[2] Dr. Robin Lachmann - Charles Dent Metabolic Unit, University College London Hospitals NHS Foundation Trust, London, UK

Amicus Therapeutics

DMX-200

Potential first-in-class investigational small molecule for the treatment of FSGS in the U.S.



Amicus Therapeutics

Exclusive U.S. License Agreement with Dimerix

Deal adds significant value to Amicus today and aligns with Amicus strategy to leverage our rare disease commercial infrastructure with promising late-stage program

1. **Focal Segmental Glomerulosclerosis** (FSGS) is a rare and fatal kidney disease[1] that affects **>40,000 people in the U.S.**, with no approved treatments
2. **DMX-200** is a **Phase 3** program, with **Orphan Drug** designation, in development for a disease with **blockbuster market potential** in the U.S.
3. **ACTION3 study** is well underway with **positive interim analysis**[2] and agreement with FDA on **proteinuria** as primary endpoint
4. **Structured in-licensing deal** with modest upfront and downstream success-based milestones and tiered royalties
5. Opportunity to pursue **additional indications**

Amicus Therapeutics®

Dimerix

[1] Guruswamy Sangameswaran KD, Baradhi KM. Focal Segmental Glomerulosclerosis (July 2021), online: https://www.ncbi.nlm.nih.gov/books/NBK532272/;
[2] Predictive power statistical model, using industry standard as set by the independent renal biostatistician consultant for Dimerix

Focal Segmental Glomerulosclerosis (FSGS) is a rare disease leading to irreversible kidney damage

- Irreversible scarring leads to permanent **kidney damage** and eventual **end-stage renal failure**[1]
- Symptoms include **proteinuria**, edema, high cholesterol and blood pressure, low albumin levels
- Average time from diagnosis to onset of complete kidney failure is typically **five to ten years**[2]
- FSGS kidney damage can lead to **dialysis, kidney transplants**, or **death**

Pathogenic Feedback Loop in FSGS

[1] Guruswamy Sangameswaran KD, Baradhi KM. Focal Segmental Glomerulosclerosis (July 2021), online: https://www.ncbi.nlm.nih.gov/books/NBK532272/;
[2] Kiffel et. Al. Adv Chronic Kidney Dis. (September 2011), online: https://pmc.ncbi.nlm.nih.gov/articles/PMC3709971/pdf/nihms286597.pdf

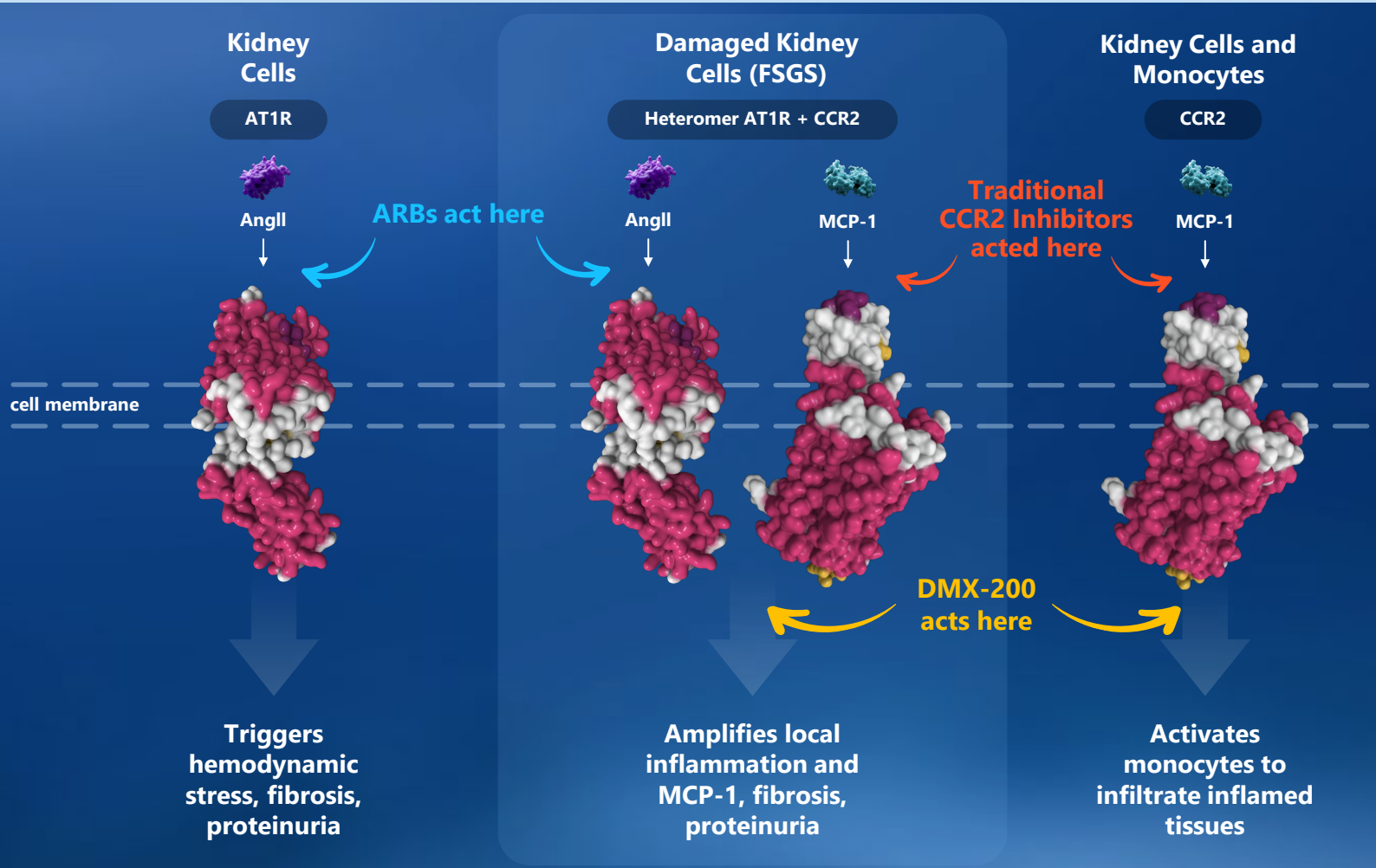
Amicus Therapeutics®

DMX-200 Mechanism of Action

DMX-200 works synergistically with ARBs to act on innate inflammatory signaling pathway in the kidney

DMX-200 + ARB

- Blocks MCP-1–mediated signaling through AT1R–CCR2 receptor complex expressed on damaged kidney cells – a pathway not disrupted by corticosteroids or ARBs alone
- Delivers kidney-specific, non-immunosuppressive reduction in monocyte-driven inflammation
- Directly targets key unaddressed drivers of disease in patients with persistent proteinuria and active inflammation



Summers RJ et al. Nephrology. 2020;25(5):364–72. • Gilbert RE et al. Kidney Int Rep. 2022;7(7):1729–38. • Bascands JL, Schanstra JP. J Am Soc Nephrol. 2005;16(4):1050–60. • Segerer S et al. Kidney Int. 2000;57(2):766–73.

Amicus Therapeutics

DMX-200 Clinical and Regulatory Progress

Dimerix has built a strong body of evidence and made significant clinical and regulatory progress

MOA	Precision therapy to disrupt the pathogenic monocyte-driven inflammatory feedback loop in the kidney of patients with FSGS
Phase 2	Positive efficacy signals and well-tolerated across studies (n=80), including impacts on proteinuria and inflammation in FSGS study
ACTION3 Phase 3	Enrollment well underway (259 of 286 pts to date); Interim analysis (n=72 at 36 wks) showed DMX-200 performing better than placebo in reducing proteinuria[1]
FDA and Project PARASOL	Alignment on proteinuria as a primary endpoint for approval
ACTION3 Part 2 Interim Analysis	Expected after planned follow-up meeting with FDA
ACTION3 Part 3 Final Analysis	2-year proteinuria (primary) and eGFR (secondary) data serves as basis for Full Approval (n=286)

[1] Predictive power statistical model, using industry standard as set by the independent renal biostatistician consultant for Dimerix

Amicus Therapeutics

DMX-200 Phase 3 Clinical Trial in FSGS

ACTION3
FSGS CLINICAL STUDY

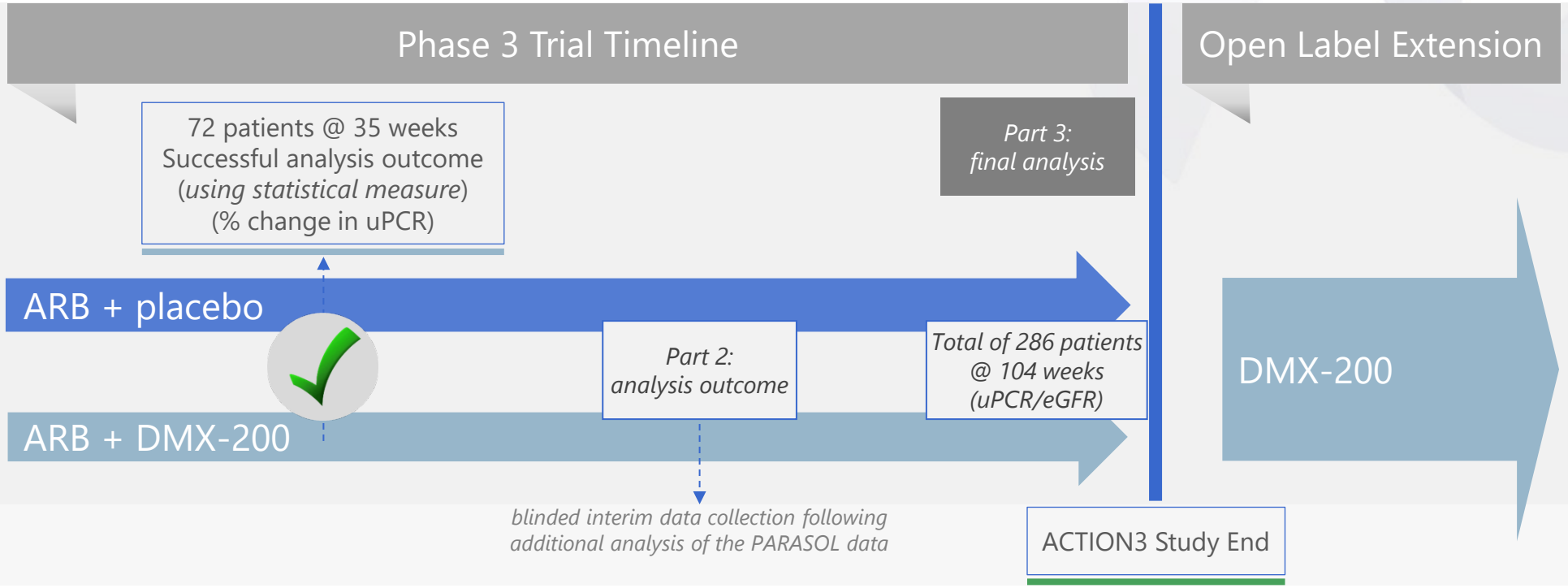
FDA aligned on proteinuria endpoint for approval; 259 patients already randomized/dosed to date

A randomized, double-blind, multi-center, placebo-controlled study of renal outcomes of DMX-200 in patients with FSGS receiving an ARB

Background

- Patients recruited, then screened and stabilized on background medications
- Patients randomized to receive drug or placebo
- DXB remains blinded at all times during study

Phase 3 Trial Timeline



Open Label Extension

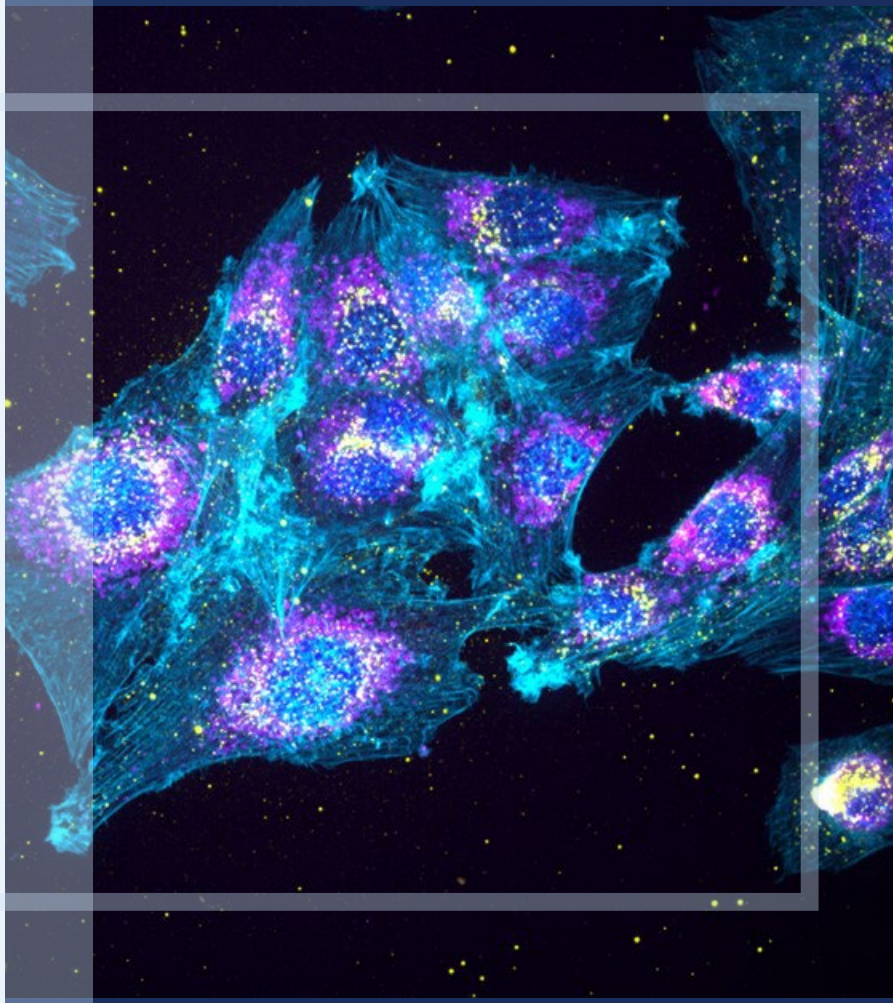
DMX-200

Dimerix will continue to fund and execute the ACTION3 study

Amicus Therapeutics

Corporate Outlook

Delivering on our mission for patients and shareholders



Amicus Therapeutics

FY 2025 Financial Guidance Reiterated

FY 2025 Financial Guidance[1]

Total Revenue Growth[1]	15% to 22%
Galafold Revenue Growth[1]	10% to 15%
Pombiliti + Opfolda Revenue Growth[1]	50% to 65%
Gross Margin	Mid 80%
Non-GAAP Operating Expense	$380M to $400M[2]
GAAP Net Income	Positive during H2 2025

FY 2025 Revenue Sensitivity

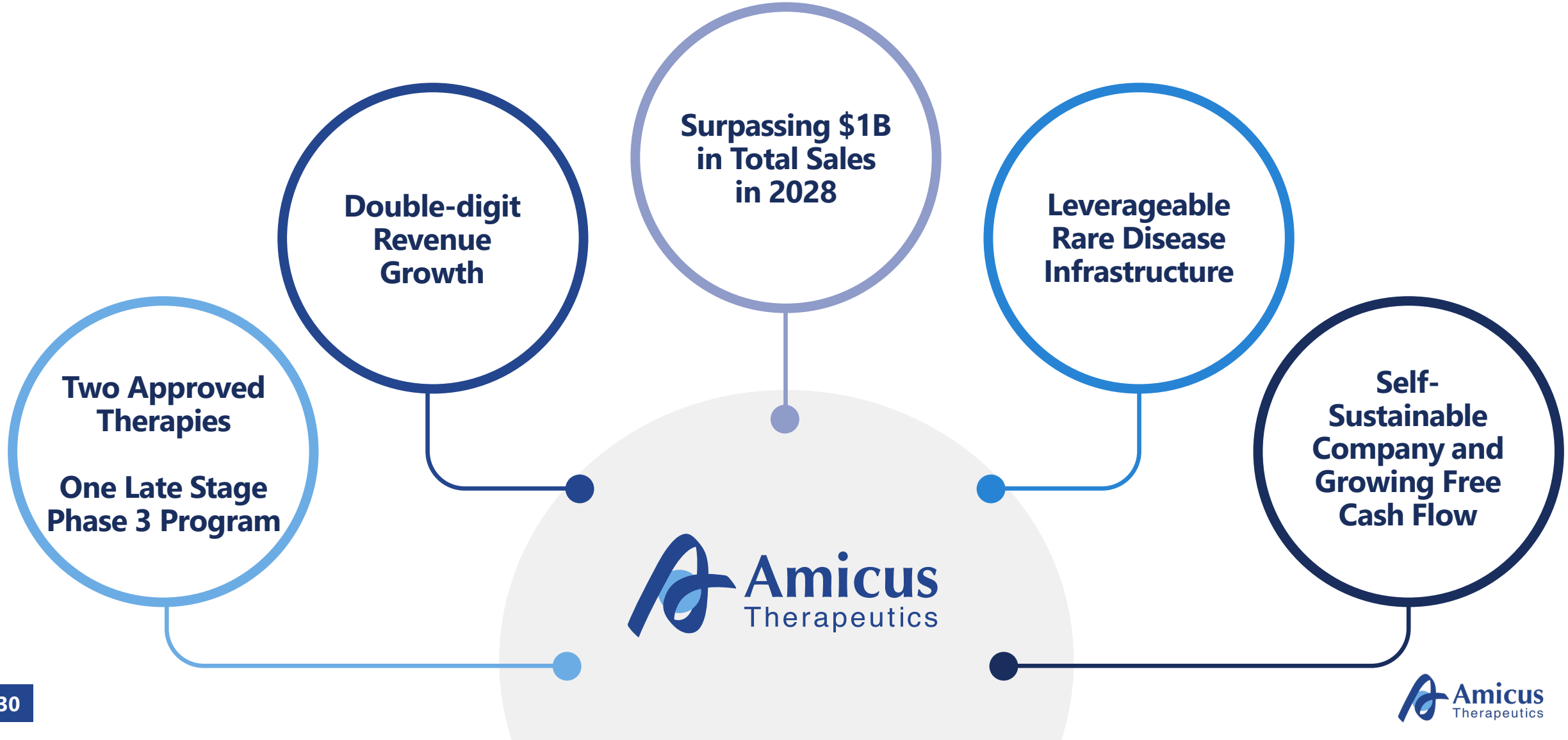
Given the proportion of Amicus revenue ex-US (**~60%** in 2024), a change in USD exchange rates of **+/- 1%** compared to 2024 rates could lead to a **~$4M** move in Total Reported Revenues in 2025

[1] Full-Year 2025 guidance is provided at CER (Constant Exchange Rates) using Full-Year 2024 Average Exchange Rates
[2] Inclusive of $30M upfront payment for the U.S. licensing agreement of DMX-200

Amicus Therapeutics

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

Amicus Therapeutics

Amicus Therapeutics

Thank You

Amicus Therapeutics

Appendix

Reconciliation of Non-GAAP Financial Measures

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Total operating expenses - as reported GAAP	**$ 115,325**	**$ 106,579**	**$ 385,773**	**$ 331,577**
Research and development:				
Share-based compensation	2,368	4,397	8,765	12,329
Selling, general and administrative:				
Share-based compensation	15,721	14,291	52,055	53,359
Loss on impairment of assets	—	—	1,702	—
Restructuring Charges	—	3,143	—	9,188
Depreciation and amortization	1,874	2,170	5,563	6,506
Total operating expense adjustments to reported GAAP	19,963	24,002	68,085	81,382
Total operating expenses - as adjusted	$ 95,362	$ 82,577	$ 317,688	$ 250,195

Amicus Therapeutics

Reconciliation of Non-GAAP Financial Measures *(Cont'd)*

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
GAAP net income (loss)	**$ 17,306**	**$ (6,729)**	**$ (28,800)**	**$ (70,845)**
Share-based compensation	18,089	18,688	60,820	65,688
Depreciation and amortization	1,874	2,170	5,563	6,506
Loss on impairment of assets	—	—	1,702	—
Restructuring charges	—	3,143	—	9,188
Income tax expense	16,968	13,514	25,848	34,155
Non-GAAP net income	$ 54,237	$ 30,786	$ 65,133	$ 44,692
Non-GAAP net income attributable to common stockholders per common share — basic	$ 0.18	$ 0.10	$ 0.21	$ 0.15
Non-GAAP net income attributable to common stockholders per common share — diluted	$ 0.17	$ 0.10	$ 0.21	$ 0.15
Weighted-average common shares outstanding — basic	308,468,423	304,690,596	308,139,134	303,792,479
Weighted-average common shares outstanding — diluted	310,433,494	304,690,596	308,139,134	303,792,479

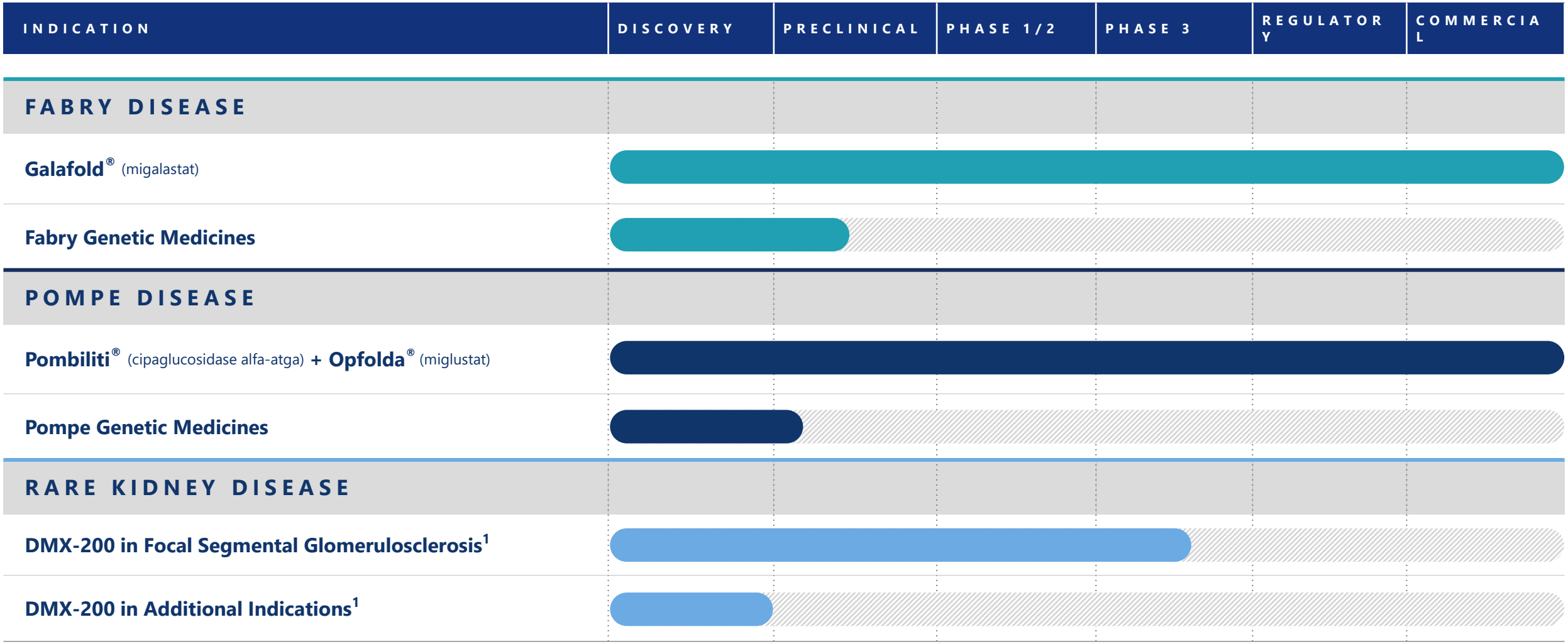
Amicus Therapeutics

Exchange Rates

Currency Average Rates

FX Rates	Q3 2024	Q3 2025	Variance
USD/EUR	1.099	1.169	6.4%
USD/GBP	1.301	1.349	3.7%
USD/JPY	0.007	0.007	0.9%

Rare Disease Pipeline



[1] Exclusive rights to commercialize in the United States