



3Q22 Financial Results Conference Call & Webcast

At the Forefront of Therapies
for Rare Diseases

November 7, 2022



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, expenses, cash position, and future profitability 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 in particular the potential goals, progress, timing, and results of preclinical studies and clinical trials, 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, and PMDA, may not grant or may delay approval for our product candidates; the potential that we may not be successful in commercializing Galafold in Europe, Japan, the US and other geographies or our other product candidates 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, manufacturing and launch preparations. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. With respect to statements regarding projections of the Company's revenue, expenses, cash position, and future profitability, 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, 2021 and Form 10-Q for the quarter ended September 30, 2022, that was 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



Gene Therapy PLATFORM

Leveraging Experience in Protein Engineering & Glycobiology

World-class CLINICAL DEVELOPMENT Capabilities



Non-GAAP PROFITABILITY expected in 2023

EMPLOYEES in 20 Countries

GLOBAL COMMERCIAL ORGANIZATION

AT-GAA

a Two-component Therapy Under Global Regulatory Reviews for Pompe Disease

15% - 20%

FY22 Galafold Revenue Growth at CER

GALAFOLD & AT-GAA

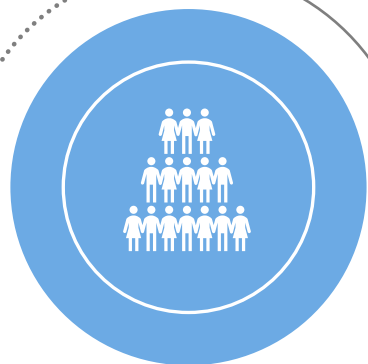
Cumulative \$2B Peak Potential

\$354.7M

Cash as of 9/30/22

Positioned for Significant Value Growth

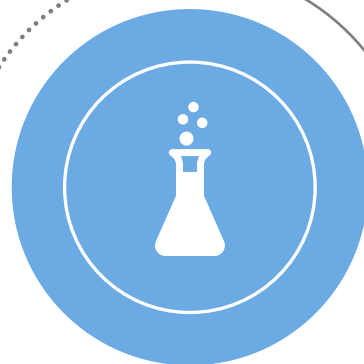
Focused on Execution and Driving Sustainable Double-digit Revenue Growth on Path to Profitability



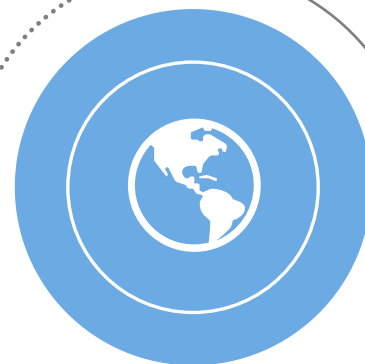
Continue to bring Galafold[®] to as many patients as possible, sustain double-digit revenue growth



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 2023¹

2022 Strategic Priorities to Drive Value

- 1** > **Double-digit Galafold growth (15-20%) with revenue of \$350M to \$365M at CER¹**
- 2** > **Secure FDA approval and positive CHMP opinion for AT-GAA**
- 3** > **Initiate successful, rapid launch in U.S. for AT-GAA**
- 4** > **Advance best-in-class, next-generation genetic medicines and capabilities**
- 5** > **Maintain strong financial position on path to profitability**



Galafold[®] (migalastat) Continued Growth...

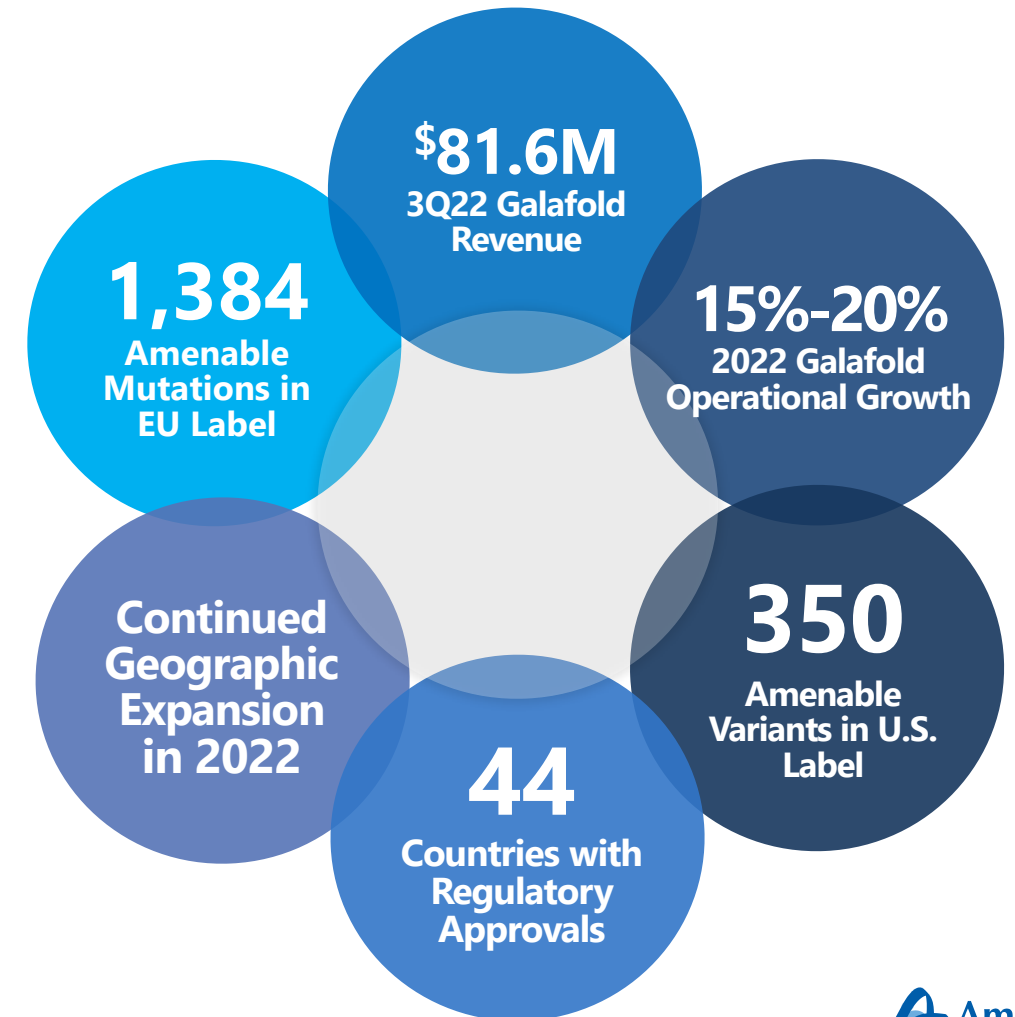
... building a leadership position in the
treatment of Fabry disease



Galafold Success (as of September 30, 2022)

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

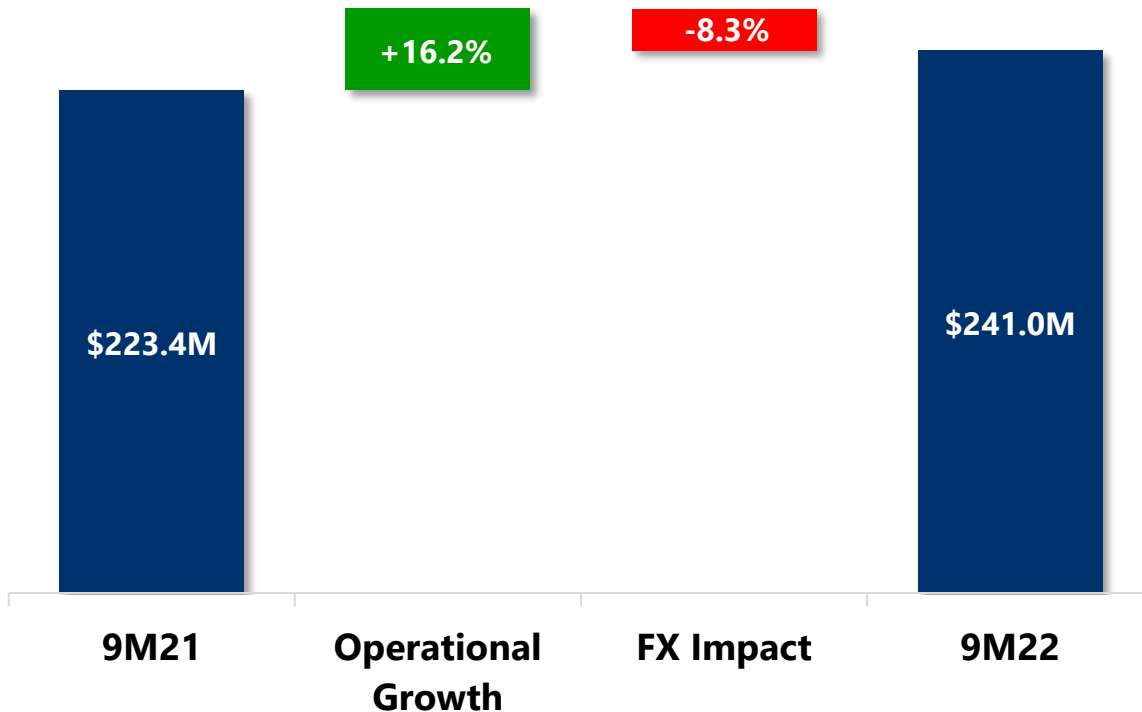


Galafold is indicated for adults with a confirmed diagnosis of Fabry Disease and an amenable variant. The most common adverse reactions reported with Galafold ($\geq 10\%$) were headache, nasopharyngitis, urinary tract infection, nausea and pyrexia. For additional information about Galafold, including 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.

Galafold Performance

YTD Reported Revenue Growth of +7.9% to \$241.0M – Strong Operational Growth of +16.2% at CER

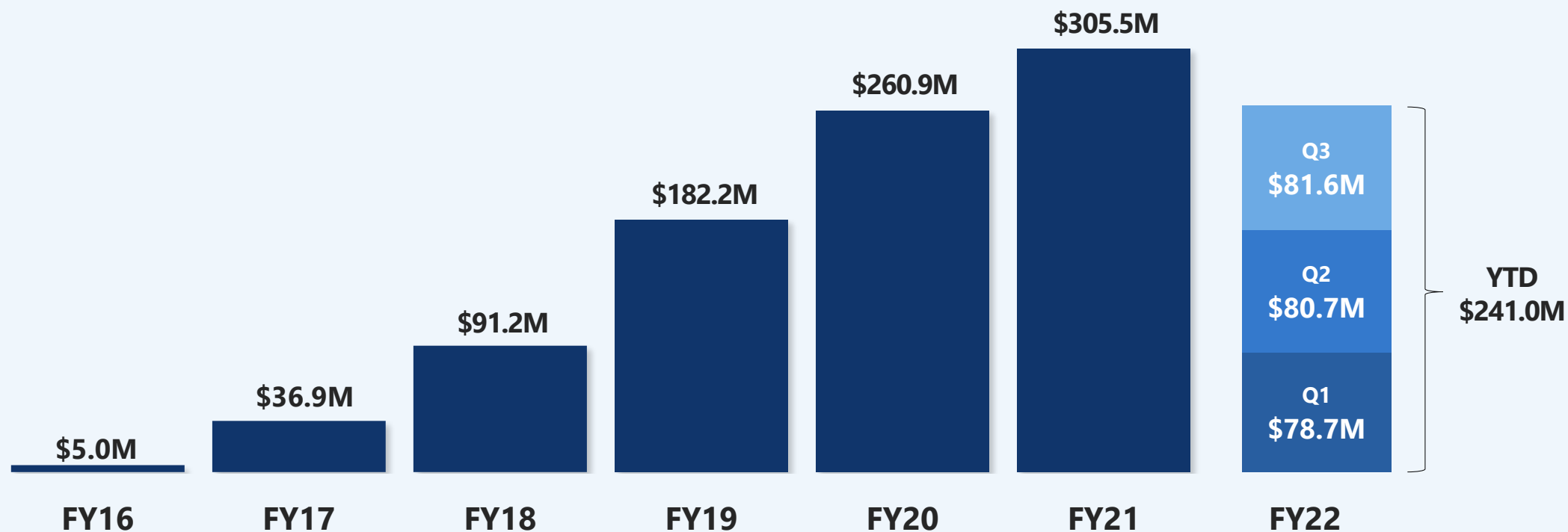
Year-over-Year Sales Growth



- Global demand remains strong: 3-month net new patients trend best in 2 years
- Call volume increasing from same period last year
- Global mix of switch (~55%) and previously untreated patients (~45%)
- Compliance and adherence over 90%+
- Expect non-linear quarterly growth to continue due to uneven ordering patterns and FX fluctuations

Galafold Success and FY22 Revenue Guidance

Galafold Momentum on Track to Achieve Full-year 2022 Revenue Guidance at CER



Reiterating FY22 Revenue Growth Guidance of 15% and 20% growth at CER

Galafold Growth Opportunity

\$1B Annual Sales Opportunity at Peak

Sustained double-digit revenue growth:

3Q operational revenue growth of +13.4%

Near-term growth to \$500M driven by:

Continued penetration into existing markets

Expansion into new geographies

Broadening of labels

Long-term growth towards peak sales potential driven by:

Penetration of the diagnosed untreated population

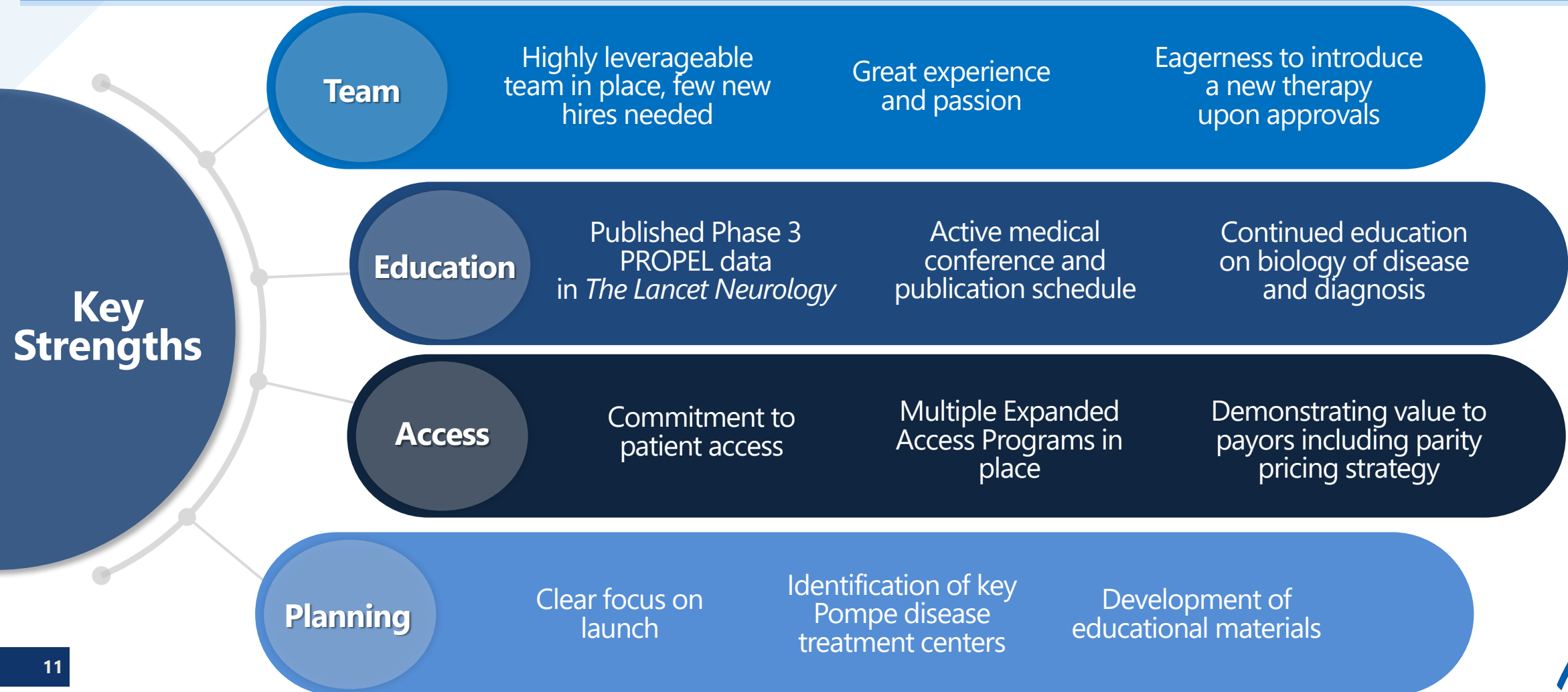
Increase in newborn screening and diagnostic initiatives

Strong IP rights, including COM protection through 2038



AT-GAA Launch Preparations

Experienced and Passionate Rare Disease Medical and Commercial Organization
Poised for Second Successful Launch



AT-GAA (cipaglucosidase alfa + miglustat)

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



Pompe Disease Overview

Pompe is a Severe and Fatal Neuromuscular Disease Caused by the Deficiency of Lysosomal Enzyme GAA



Estimated incidence of ~1:28,000; newborn screening suggests significant underdiagnosis

Age of onset ranges from infancy to adulthood

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

Respiratory and cardiac failure are leading causes of morbidity and mortality

Deficiency of GAA leading to lysosomal glycogen accumulation and cellular dysfunction

Symptoms include muscle weakness, respiratory failure, and cardiomyopathy

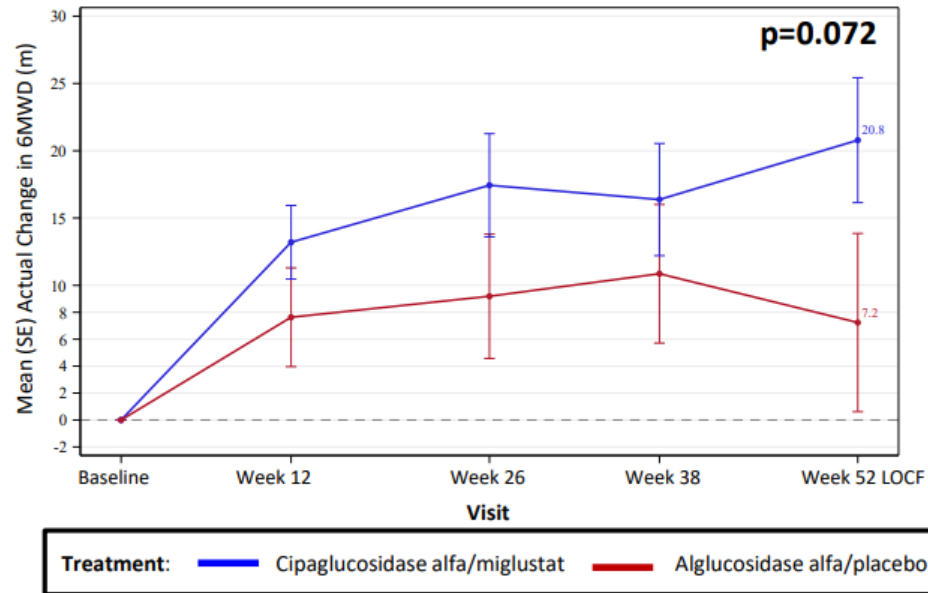
~\$1.2B+ global Pompe ERT sales¹

Phase 3 PROPEL Study Results

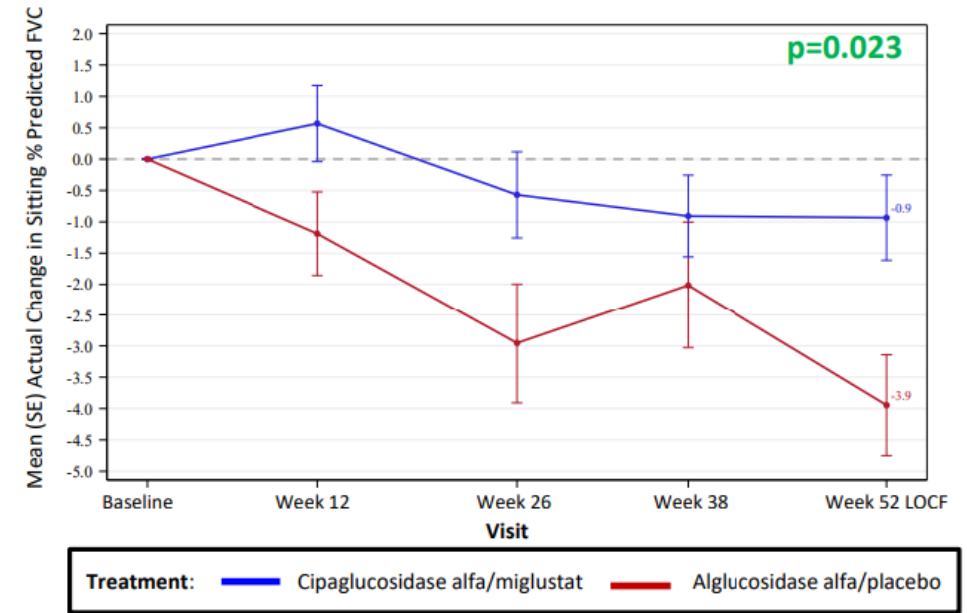
Overall Population (n=122*)

Primary and First Key Secondary Endpoint Showed Greater Improvement with AT-GAA vs. alglucosidase alfa in the Overall Population of ERT-Naïve and ERT-Experienced Patients

6MWD (m): Change from Baseline (n=85, n=37)



FVC (% predicted): Change from Baseline (n=85, n=37)

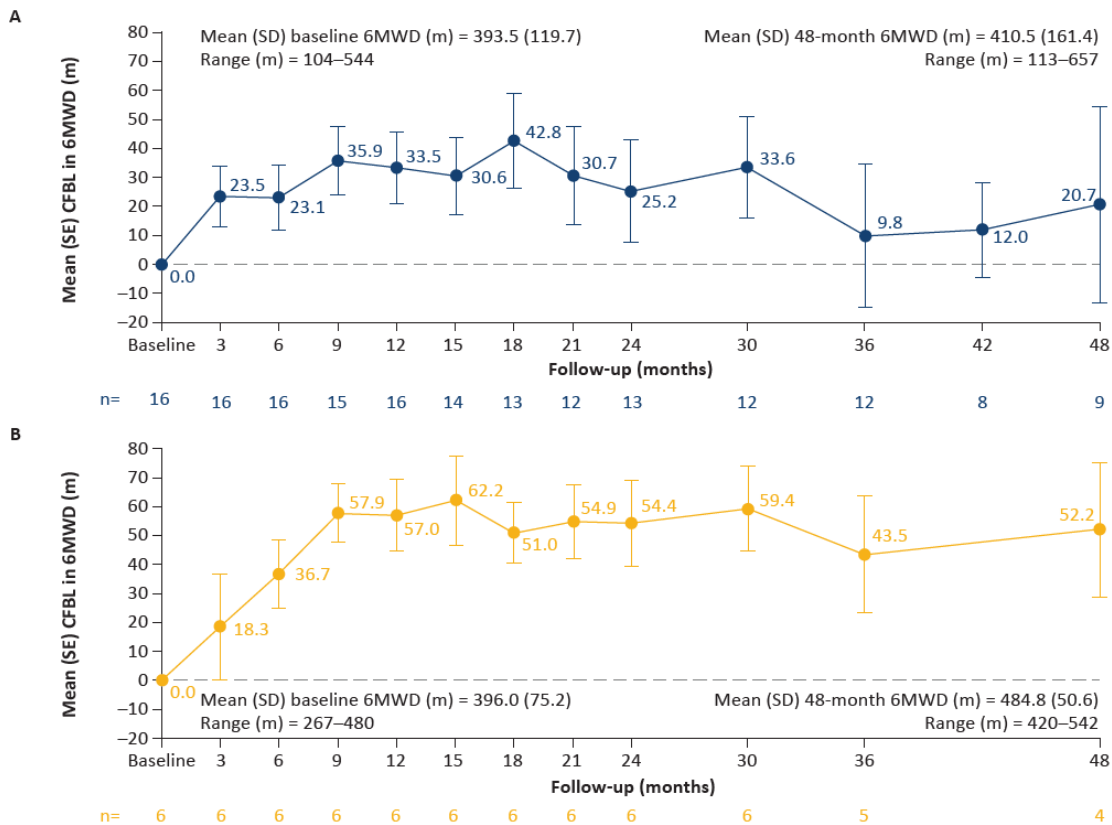


Results in ERT-Experienced Patients (n=92) Showed Meaningful Improvement for Both 6MWD (P=0.046) and FVC (P=0.006)

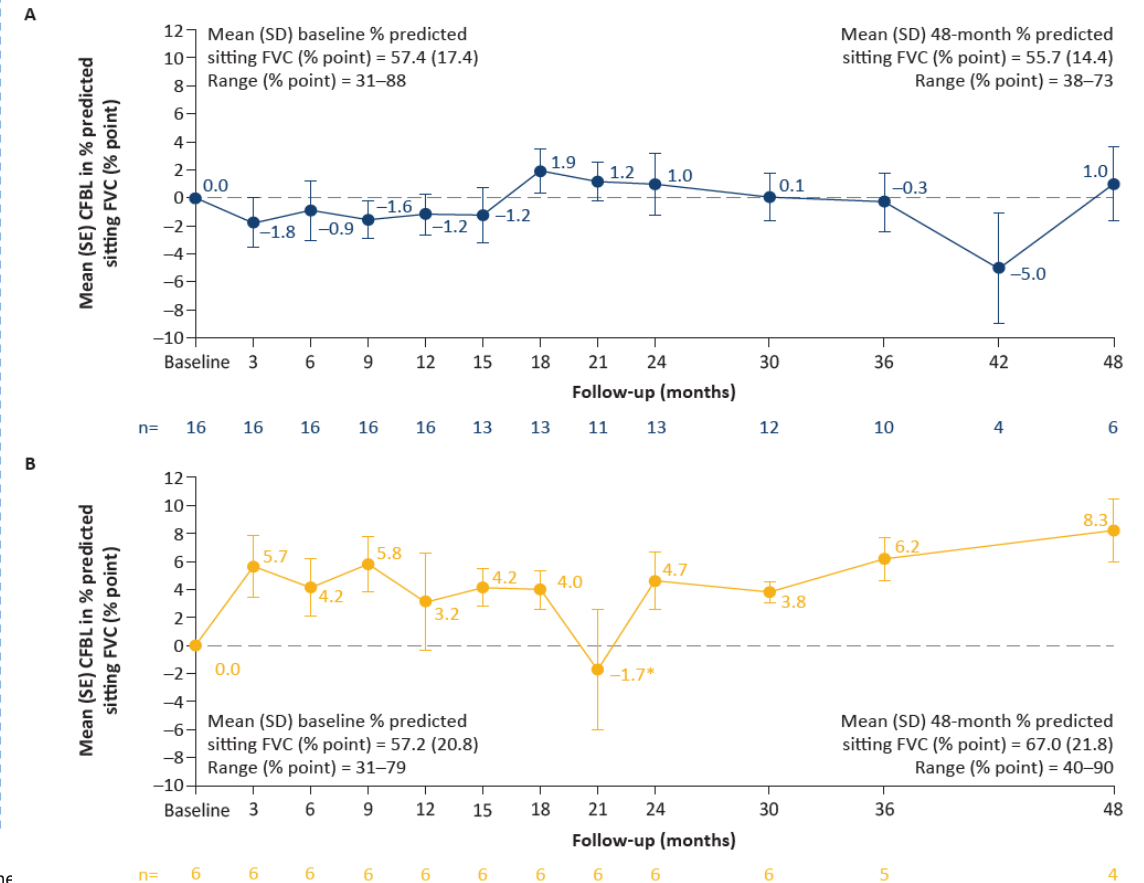
Long-Term Data from Phase 1/2 Clinical Study (ATB200-02)

Persistent and Durable Improvements in Motor and Respiratory Function and Reductions in Biomarkers of Muscle Damage and Disease Substrate Observed in Patients out to 48 Months

CFBL in 6MWD in (A) ERT-Experienced and (B) ERT-Naïve Patients



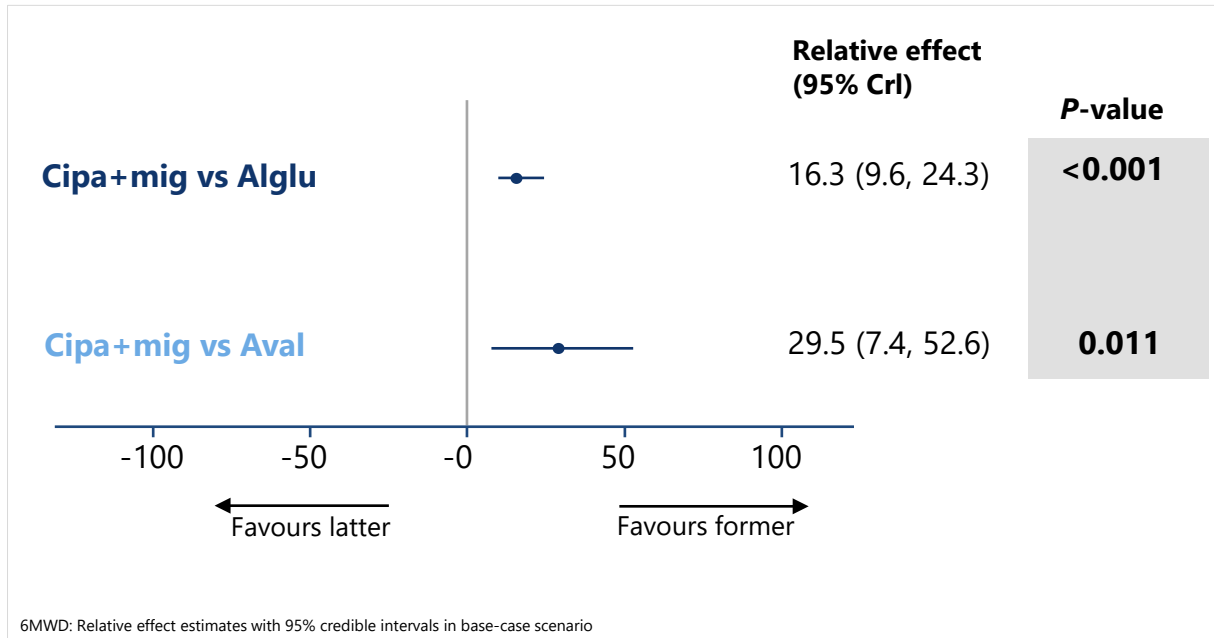
CFBL in FVC in (A) ERT-Experienced and (B) ERT-Naïve Patients



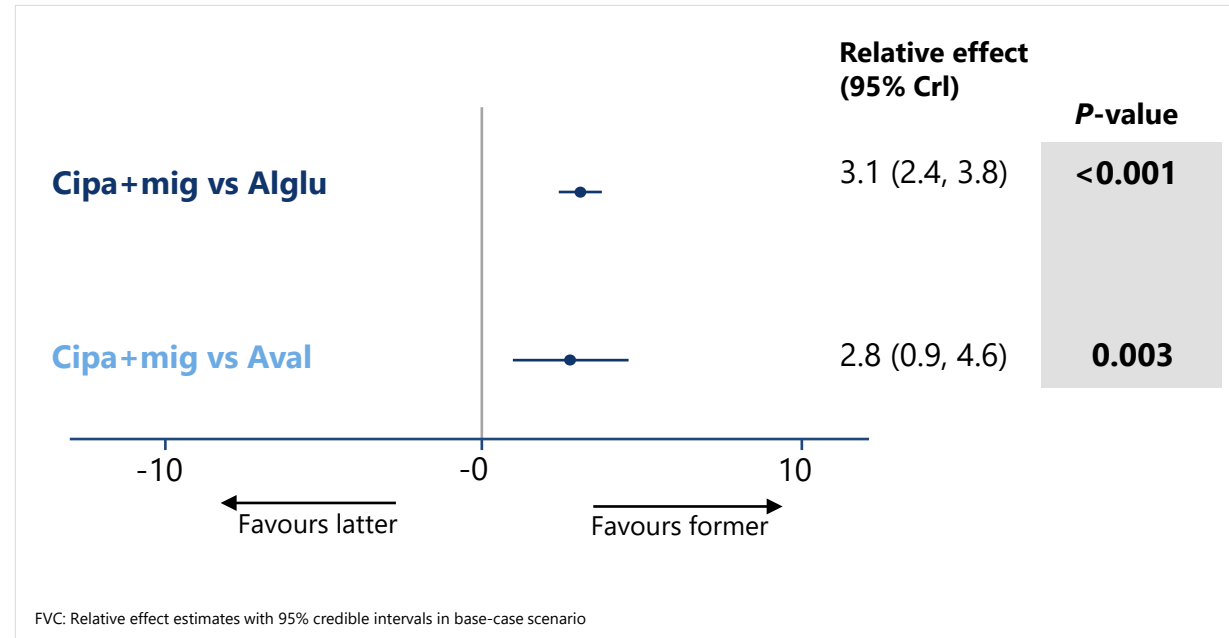
AT-GAA: Ongoing Evidence Generation

Indirect Treatment Comparison across Pompe ERT Studies Recently Presented at World Muscle Society 2022 Congress Highlights Potential Clinical Differentiation of AT-GAA

Relative Effect (6MWD Change from Baseline at Week 52)



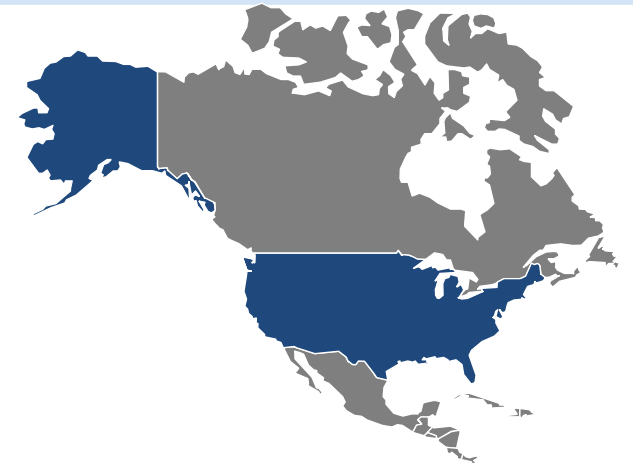
Relative Effect (FVC Change from Baseline at Week 52)



AT-GAA: Key Takeaways

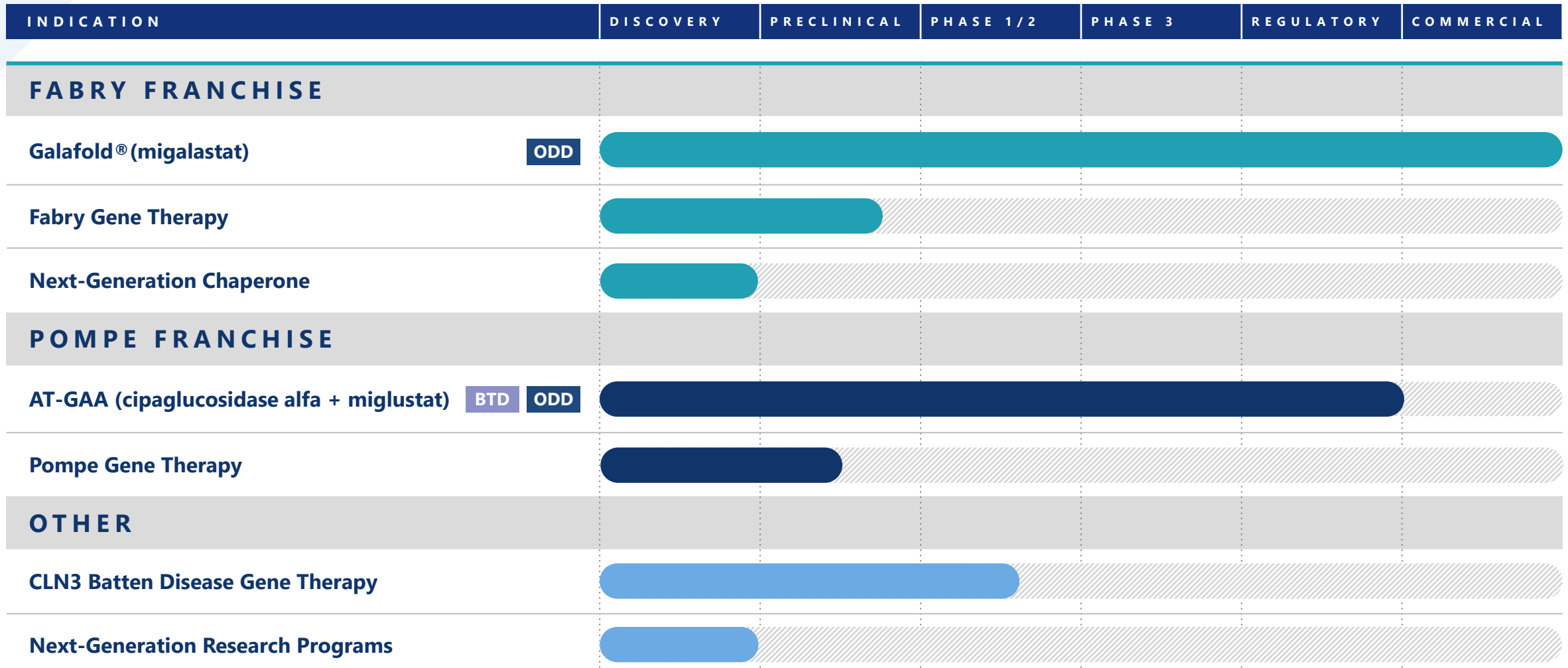
Focused on Advancing AT-GAA to as Many Patients as Possible through Global Regulatory Pathways and Expanded Access Mechanisms

- U.S. Regulatory status update:
 - PDUFA action date deferred due to Agency's inability to conduct manufacturing inspection in China¹
 - At the Agency's direction, the Company has requested a Type A meeting to develop plans and logistics for a pre-approval inspection
- International Regulatory status update:
 - CHMP opinion expected as early as December 2022
 - On track for additional regulatory submissions
- Multiple expanded access mechanisms in place, including in the U.S., U.K., Germany, France, Japan, and others
- ~190 people living with Pompe disease are now on AT-GAA across our clinical extension studies and expanded access programs
- Ongoing supportive studies:
 - LOPD in children and adolescents aged 0 to <18; Infantile-Onset Pompe Disease (IOPD)



Amicus Pipeline

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





Financial & Operational Strategy

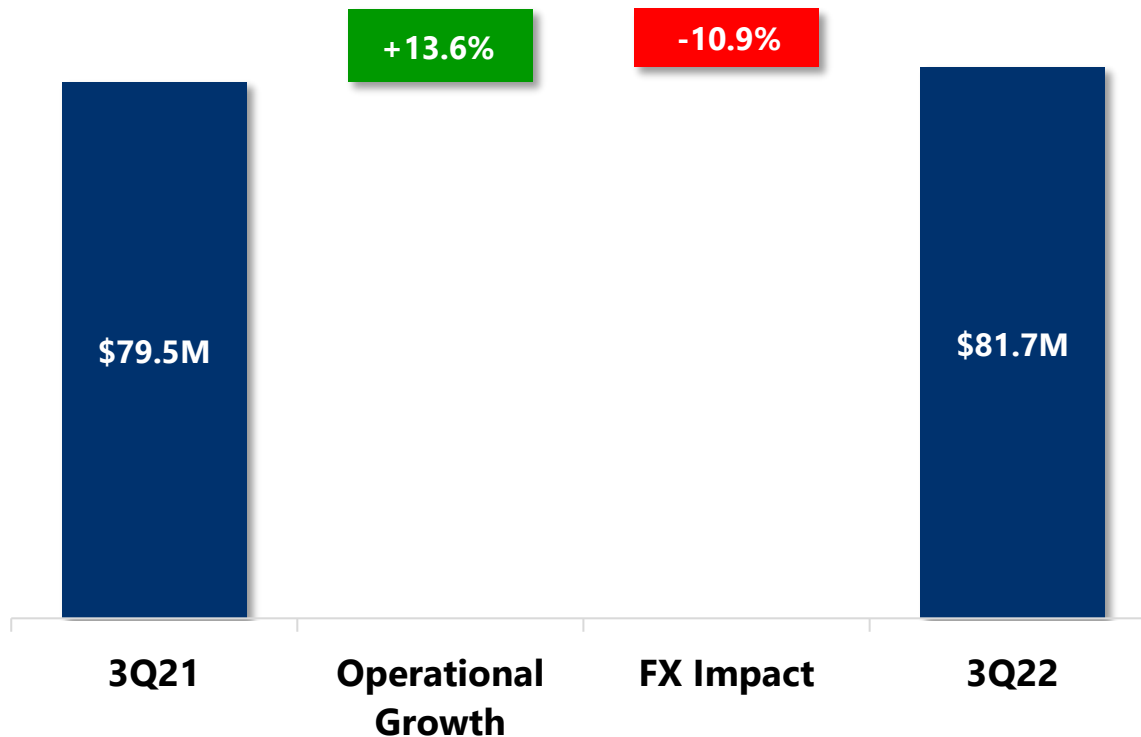
... maintaining a strong financial outlook



Q3 2022 Revenue Performance

Q3 2022 Reported Revenue Growth of +2.7% to \$81.7M resulting from Strong Operational Growth of +13.6% at CER Offset by Negative FX impact of -10.9%

Year-over-Year Sales Growth



- Significant currency exposure as 63% of Galafold revenue generated outside the U.S.
- Applying average October 2022 exchange rates, the negative FX impact on full-year 2022 reported sales would be approximately -9%, or ~\$28.5 million.

Q3 2022 Select Financial Results

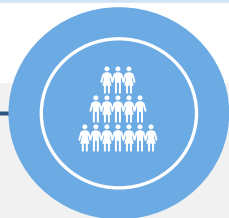
Q3 2022 OpEx Decrease Related to the Reprioritization of the Gene Therapy Portfolio

(in thousands, except per share data)

| | Sep. 30, 2022 | Sep. 30, 2021 |
|--|---------------|---------------|
| Product Revenue | \$81,691 | \$79,545 |
| Cost of Goods Sold | 13,436 | 11,696 |
| R&D Expense | 52,970 | 59,333 |
| SG&A Expense | 47,272 | 46,107 |
| Changes in Fair Value of Contingent Consideration | 567 | 3,288 |
| Depreciation and Amortization | 1,286 | 1,520 |
| Loss from Operations | (33,840) | (42,399) |
| Income Tax (Expense) Benefit | (4,023) | 182 |
| Net Loss | (33,286) | (50,294) |
| Net Loss Per Share | (0.12) | (0.19) |

Financial Outlook and Path to Profitability

Clear Strategy to Build Our Business, Advance Our Portfolio, and Achieve Profitability



Sustain Galafold Revenue Growth

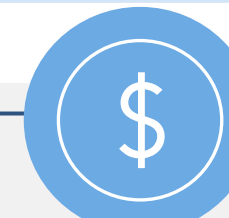
\$241M YTD revenue,
+16.2% YoY
Operational Growth

2022 Galafold revenue
growth guidance of
+15-20% 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

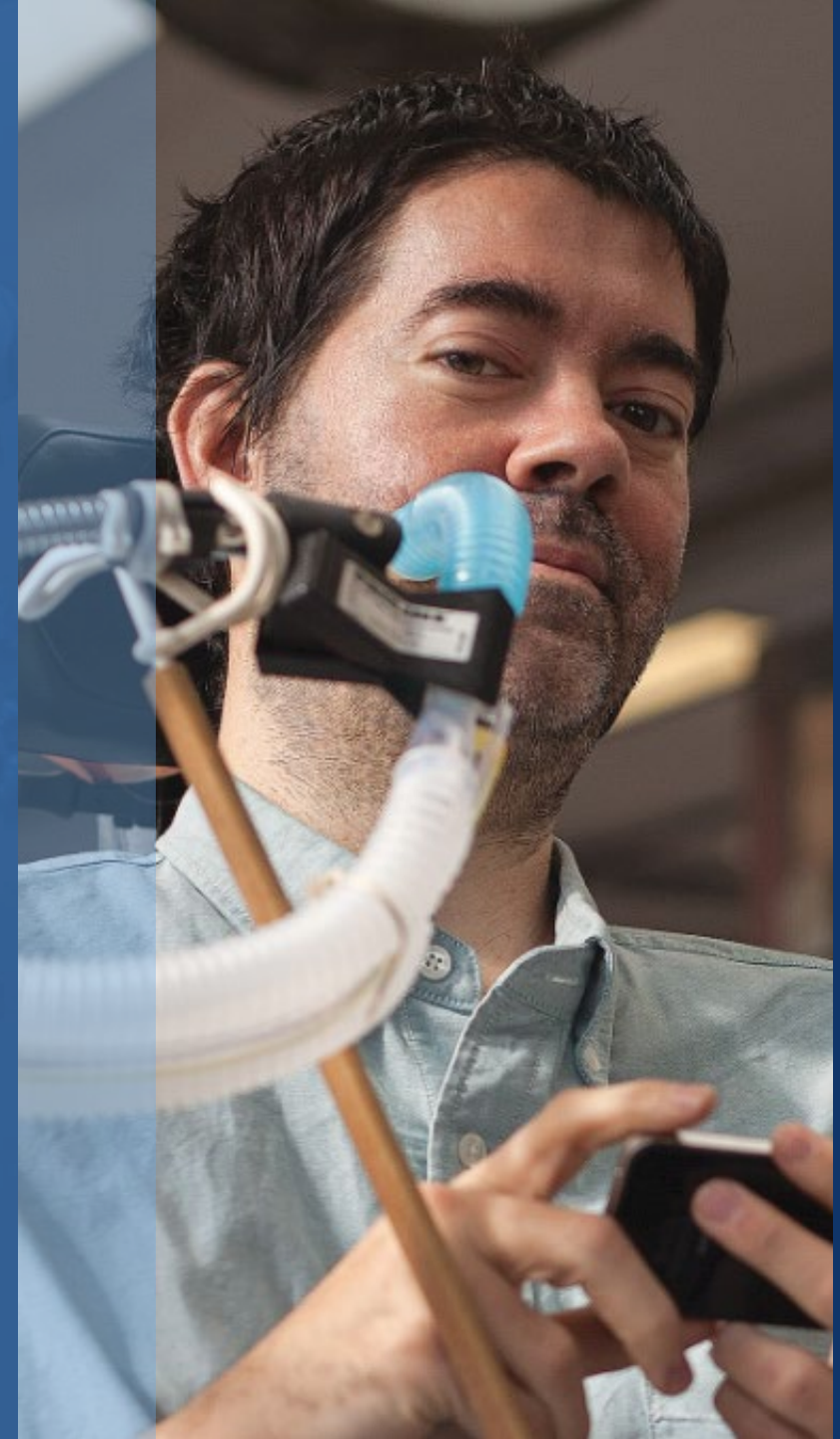
Focused on prudent
expense management

2022 non-GAAP operating
expense guidance of
\$430M-\$440M

Achieve profitability¹
in 2023



Appendix



Appendix

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

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|--|----------------------------------|-------------------|---------------------------------|-------------------|
| | 2022 | 2021 | 2022 | 2021 |
| Total operating expenses - as reported GAAP | \$ 102,095 | \$ 110,248 | \$ 381,714 | \$ 331,033 |
| Research and development: | | | | |
| Share-based compensation | 5,428 | 3,775 | 19,172 | 13,232 |
| Selling, general and administrative: | | | | |
| Share-based compensation | 9,344 | 8,066 | 38,714 | 30,699 |
| Loss on impairment of assets | — | — | 6,616 | — |
| Changes in fair value of contingent consideration payable | 567 | 3,288 | (506) | 4,780 |
| Depreciation and amortization | 1,286 | 1,520 | 4,031 | 4,691 |
| Total operating expense adjustments to reported GAAP | 16,625 | 16,649 | 68,027 | 53,402 |
| Total operating expenses - as adjusted | \$ 85,470 | \$ 93,599 | \$ 313,687 | \$ 277,631 |