

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 8-K

CURRENT REPORT PURSUANT TO
SECTION 13 OR 15(d) OF THE
SECURITIES EXCHANGE ACT OF 1934

Date of Report (Date of earliest event reported): September 27, 2023

AMICUS THERAPEUTICS, INC.

(Exact Name of Registrant as Specified in Its Charter)

Delaware
(State or Other Jurisdiction
of Incorporation)

001-33497
(Commission
File Number)

71-0869350
(I.R.S. Employer
Identification No.)

3675 Market Street, Philadelphia, PA 19104
(Address of Principal Executive Offices, and Zip Code)

215-921-7600
Registrant's Telephone Number, Including Area Code

(Former Name or Former Address, if Changed Since Last Report.)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock Par Value \$0.01	FOLD	NASDAQ

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (17 CFR §230.405) or Rule 12b-2 of the Securities Exchange Act of 1934 (17 CFR §240.12b-2).
Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

Item 7.01 – Regulation FD Disclosure.

On September 27, 2023, Amicus Therapeutics, Inc. (the “Company”) released an updated corporate overview presentation that it plans to use in meetings with investors and analysts. A copy of this presentation is attached hereto as Exhibit 99.1 and incorporated herein by reference.

The information in this Item 7.01, including Exhibit 99.1, is being furnished and shall not be deemed “filed” for purposes of Section 18 of the Act, or otherwise subject to the liabilities of that Section. The information in this Item 7.01, including Exhibit 99.1, shall not be incorporated by reference into any registration statement or other document pursuant to the Act.

Item 9.01 Financial Statements and Exhibits

(d) Exhibits:

Exhibit No.	Description
99.1	September 2023 Corporate Overview Presentation
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

Signature Page

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

AMICUS THERAPEUTICS, INC.

Date: September 27, 2023

By: /s/ Ellen S. Rosenberg

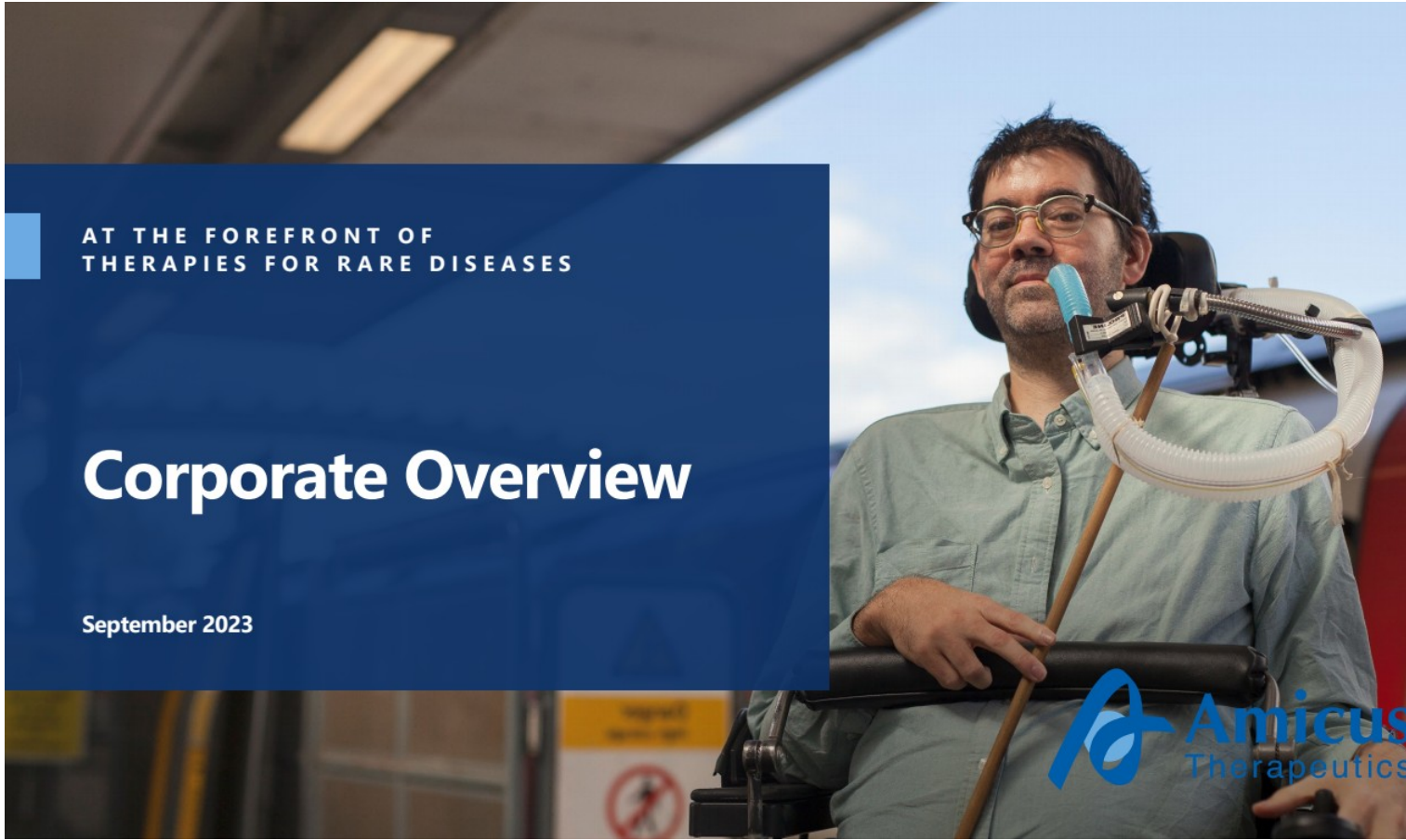
Name: Ellen S. Rosenberg

Title: Chief Legal Officer and Corporate Secretary

AT THE FOREFRONT OF
THERAPIES FOR RARE DISEASES

Corporate Overview

September 2023



Forward-Looking Statements

This presentation contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to preclinical and clinical development of our product candidates, the timing and reporting of results from preclinical studies and clinical trials, the prospects and timing of the potential regulatory approval of our product candidates, commercialization plans, manufacturing and supply plans, financing plans, and the projected revenues and cash position for the Company. The inclusion of forward-looking statements should not be regarded as a representation by us that any of our plans will be achieved. Any or all of the forward-looking statements in this presentation may turn out to be wrong and can be affected by inaccurate assumptions we might make or by known or unknown risks and uncertainties. For example, with respect to statements regarding the goals, progress, timing, and outcomes of discussions with regulatory authorities, including as they are impacted by COVID-19 related disruption, are based on current information. The potential impact on operations from the COVID-19 pandemic is inherently unknown and cannot be predicted with confidence and may cause actual results and performance to differ materially from the statements in this release, including without limitation, because of the impact on general political and economic conditions, including as a result of efforts by governmental authorities to mitigate COVID-19, such as travel bans, shelter in place orders and third-party business closures and resource allocations, manufacturing and supply chain disruptions and limitations on patient access to commercial or clinical product. In addition to the impact of the COVID-19 pandemic, actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in our business, including, without limitation: the potential that results of clinical or preclinical studies indicate that the product candidates are unsafe or ineffective; the potential that it may be difficult to enroll patients in our clinical trials; the potential that regulatory authorities, including the FDA, EMA, MHRA, and PMDA, may not grant or may delay approval for our product candidates; the potential that required regulatory inspections may be delayed or not be successful and delay or prevent product approval; the potential that we may not be successful in commercializing Galafold in Europe, Japan, the US and other geographies or AT-GAA if and when approved; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; the potential that we may not be able to manufacture or supply sufficient clinical or commercial products; and the potential that we will need additional funding to complete all of our studies and manufacturing. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. Statements regarding corporate financial guidance and financial goals and the attainment of such goals. With respect to statements regarding projections of the Company's revenue and cash position, actual results may differ based on market factors and the Company's ability to execute its operational and budget plans. In addition, all forward-looking statements are subject to other risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2022, and on Form 10-Q for the quarter ended June 30, 2023. 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 release to reflect events or circumstances after the date hereof.

Non-GAAP Financial Measures

In addition to financial information prepared in accordance with U.S. GAAP, this presentation also contains adjusted financial measures that we believe provide investors and management with supplemental information relating to operating performance and trends that facilitate comparisons between periods and with respect to projected information. These adjusted financial measures are non-GAAP measures and should be considered in addition to, but not as a substitute for, the information prepared in accordance with U.S. GAAP. We typically exclude certain GAAP items that management does not believe affect our basic operations and that do not meet the GAAP definition of unusual or non-recurring items. Other companies may define these measures in different ways. When we provide our expectation for non-GAAP operating expenses on a forward-looking basis, a reconciliation of the differences between the non-GAAP expectation and the corresponding GAAP measure generally is not available without unreasonable effort due to potentially high variability, complexity and low visibility as to the items that would be excluded from the GAAP measure in the relevant future period, such as unusual gains or losses. The variability of the excluded items may have a significant, and potentially unpredictable, impact on our future GAAP results.

A Rare Company

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



First Oral Precision Medicine for Fabry Disease

GLOBAL COMMERCIAL ORGANIZATION

World-class Clinical Development Capabilities



Gene Therapy Platform

Leveraging Experience in Protein Engineering & Glycobiology

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

EMPLOYEES in 20 Countries



AT-GAA
Under U.S. Regulatory Review for Pompe Disease

14-18%
FY23 Galafold Revenue Growth at CER

GALAFOLD & POMBILITI + OPFOLDA

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

\$266M
Cash as of 6/30/23

2023 Strategic Priorities

1

Sustain double-digit Galafold revenue growth of 14-18% at CER

2

Secure FDA, EMA, and MHRA approvals for AT-GAA

3

Initiate successful global launches of AT-GAA

4

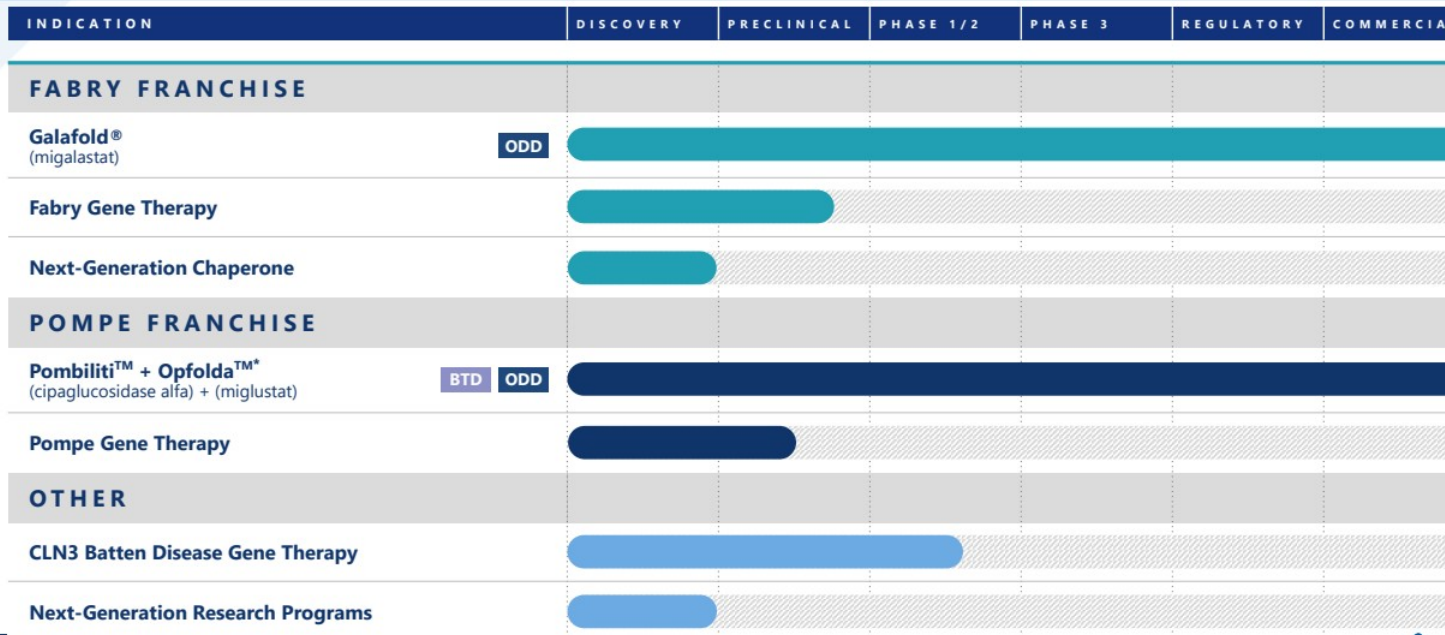
Advance best-in-class, next-generation Fabry and Pompe pipeline programs and capabilities

5

Maintain strong financial position on path to profitability

Amicus Pipeline

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





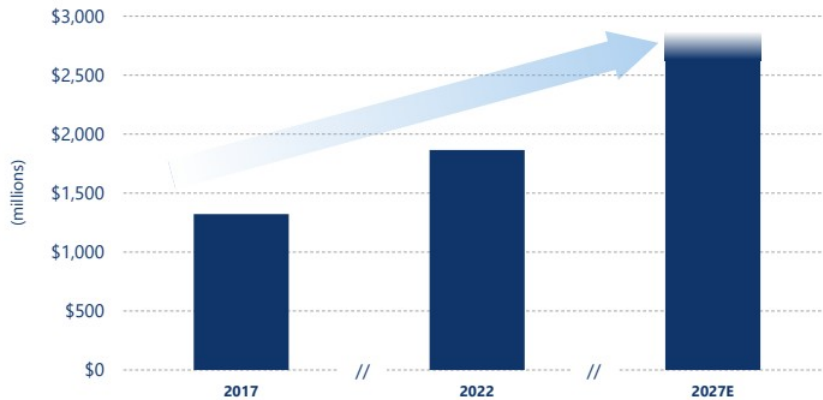
Galafold® (*migalastat*) Continued Growth

Building a leadership position in the
treatment of Fabry disease

Global Fabry Market

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

Global Fabry Market of ~\$1.9B in 2022 and Tracking toward ~\$2.6B+ by 2027¹

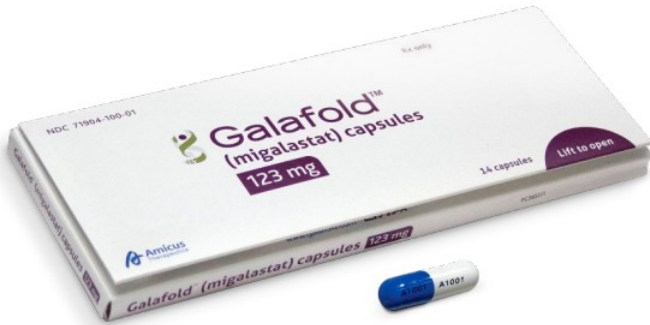


- Believed to be significantly underdiagnose
 - Newborn screening studies suggest Fabry is of the more prevalent genetic diseases (~1:1,000 to ~1:4,000 incidence)
- In 2021 and 2022, Galafold was the fastest growing Fabry treatment and the greatest contributor to market growth
 - Galafold has led to market expansion with >1,000+ naive patients treated

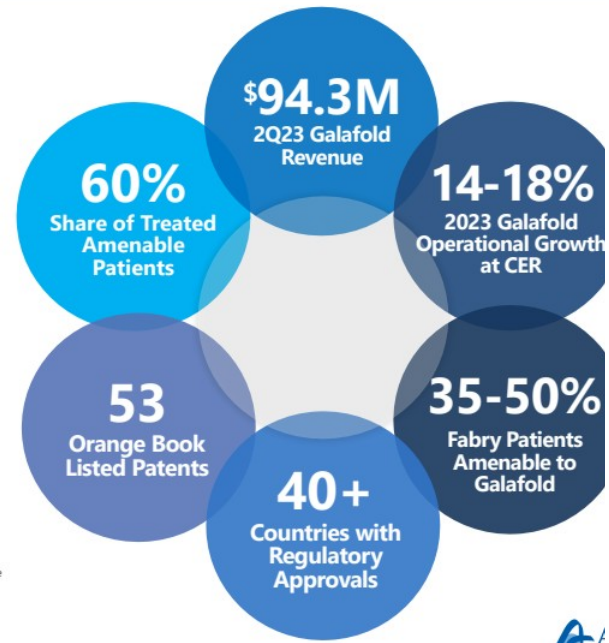
2023 Galafold Success (as of June 30, 2023)

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

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

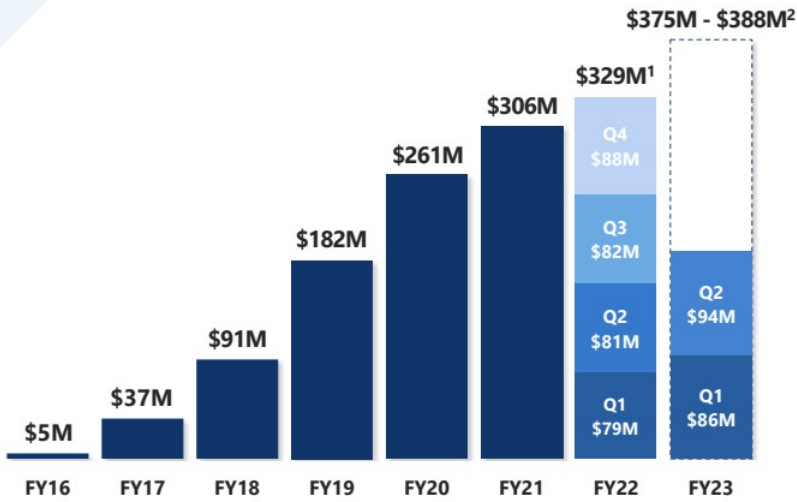


Galafold is indicated for adults with a confirmed diagnosis of Fabry disease and an amenable variant. The most common adverse reactions reported with Galafold (≥10%) were headache, nasopharyngitis, urinary tract infection, nausea, and pyrexia. For additional information about Galafold, including the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

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



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

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

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

Sustained Growth in 2023 Driven by:

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



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

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



Late-Onset Pompe Disease (LOPD) Overview

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



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

Diagnosed at different stages of life, from childhood to adulthood

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

Respiratory failure is a major cause of mortality

Deficiency of GAA leading to lysosomal glycogen accumulation and cellular dysfunction

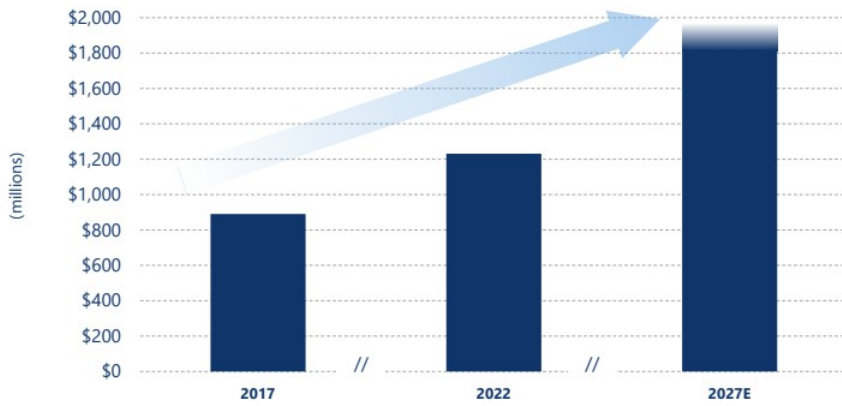
Symptoms include progressive muscle weakness, particularly skeletal and respiratory muscles, that worsens over time

~\$1.2B+ global Pompe ERT sales¹

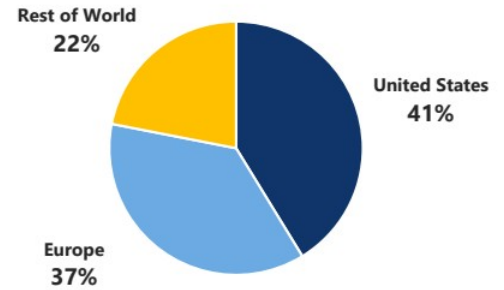
Global Pompe Market

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

Global Pompe Market of ~\$1.2B in 2022 and Tracking toward \$1.8B+ by 2027¹



Global Pompe Market Sales Split FY2022²

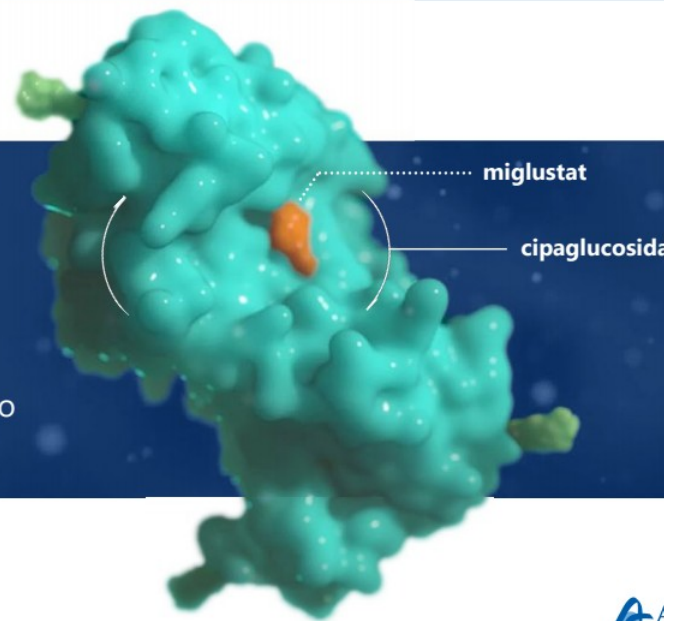


- An estimated 3,500-4,000 Pompe patients globally are being treated by ERT³

AT-GAA: An Innovative Approach to Pompe Disease

Our scientists created a uniquely glycosylated and highly phosphorylated ERT (cipaglucoaldase alfa) that significantly enhances targeting to key affected muscles

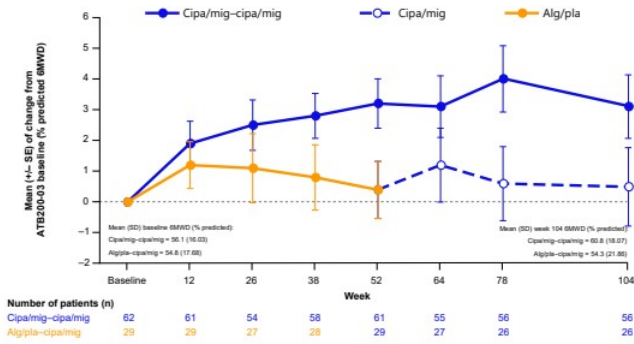
- AT-GAA is a two-component therapy combining cipaglucoaldase alfa, an ERT, with miglustat, an orally administered enzyme stabilizer
- Consists of a unique cell line producing a naturally glycosylated enzyme that can be properly processed within the lysosome to its mature form which is required to optimally break down glycogen¹



WORLDsymposium Update – Phase 3 OLE of AT-GAA in LOPD

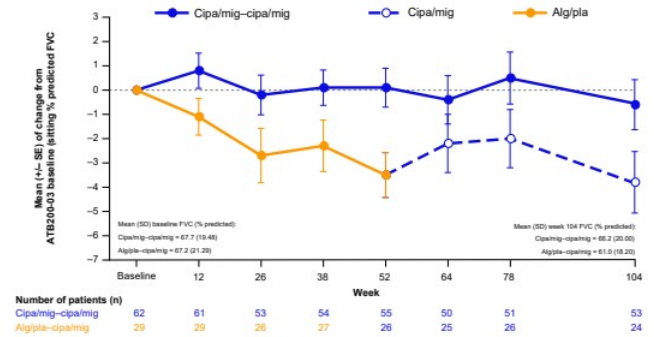
Phase 3 open-label extension study data demonstrate that treatment with AT-GAA up to 2 year was associated with a durable effect, supporting the long-term benefits

ERT-Experienced 6MWD (%): Change from baseline



- ERT-experienced and -naïve patients treated with AT-GAA throughout PROPEL showed durable improvements in % predicted 6MWD that were maintained throughout to week 104
- ERT-experienced and -naïve patients who received alglucosidase alfa/placebo in PROPEL and switched to AT-GAA in the OLE showed stability in % predicted 6MWD throughout the OLE study

ERT-Experienced FVC (%): Change from baseline

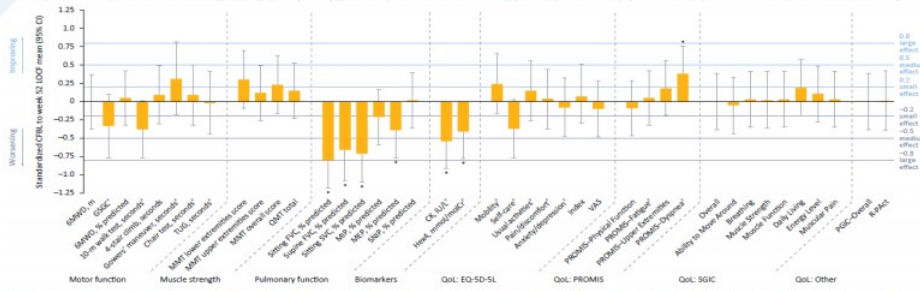


- ERT-experienced patients treated with AT-GAA throughout PROPEL remained stable while patients who received alglucosidase alfa/placebo experienced a decline in sitting % predicted FVC that stabilized after switching to AT-GAA in the OLE study

SSIEM Update – PROPEL Effect Size Analysis in ERT-Experienced Adult

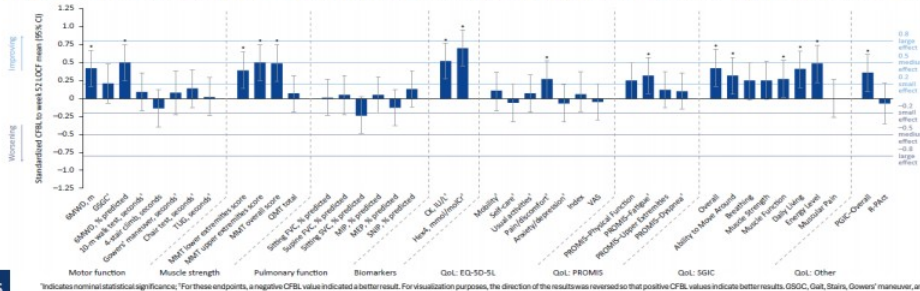
Post hoc analysis shows majority of study parameters demonstrated stabilization or improvement after switching from SoC to Pombiliti + Opfolda

ERT-experienced patients remaining on alg+pbo (n=30) generally showed worsening ($d < -0.2$) or stability ($-0.2 \leq d < 0.2$) across most outcomes, with significant worsening for various lung function assessments and biomarkers



- Significant improvement was only observed for (unadjusted mean CFBL) QoL: PROMIS-Dyspnea (-1.53).
- Significant worsening was observed for (unadjusted mean CFBL) sitting forced vital capacity (FVC, -4.02%); supine FVC (-2.63%); sitting slow vital capacity (SVC, -6.52%); maximum expiratory pressure (MEP, -3.85%); creatine kinase (CK, 79.6 IU/L); and hexc tetrasaccharide (Hex4, 1.89 mmol/molCr).

ERT-experienced patients switching to cipa+mig mostly showed improvement or stability, with significant improvements for various assessments of motor function, muscle strength, biomarker, and global impression of change scales



- Significant improvement was observed for (unadjusted mean CFBL): 6MWD (16.89 m); 6MWD % predicted (3.20); manual muscle test (MMT) lower extremities (1.63); MMT upper extremities (1.7); MMT overall score (3.38); CK (-118.0 IU/L); Hex4 (-1.69 mmol/molCr); QoL: EuroQol Dimensions-5 Levels Instrument (EQ-5D-5L)-Pain/Discomfort (-0.19); QoL: PROMIS-Fatigue (-1.87); C Subject's Global Impression of Change (SGIC)-Overall (0.34); QoL: SGIC-Ability to Move Around (0.21); QoL: SGIC-Muscle Function (0.20); QoL: SGIC-Daily Living (0.28); QoL: SGIC-Energy Level (0.27); and QoL: other-Physician's Global Impression of Change (PGIC) Overall (0.27).
- Significant worsening was not observed for any assessments.

*Indicates nominal statistical significance. For these endpoints, a negative CFBL value indicated a better result. For visualization purposes, the direction of the results was reversed so that positive CFBL values indicate better results. OSOC, Gait, Stair, Gowen's maneuver, and Chair; LOCF, last observation carried forward; MP, maximum inspiratory pressure; QM1, quantitative muscle test; SNIP, sniff nasal inspiratory pressure; TUG, timed up and go; VAS, visual analogue scale.



Global Regulatory Status

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



- **Pombiliti® + Opfolda® now approved in the EU**



- **Pombiliti® + Opfolda® now approved in the U.K.**



- **Based on recent engagements with the U.S. FDA, approval expected in the coming days**



Ongoing Clinical Studies and Expanded Access Mechanisms

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

- Ongoing clinical studies in children and adolescents¹ with LOPD and infantile-onset Pompe disease (IOPD)
- Multiple expanded access mechanisms in place, including in the U.S., U.K., Germany, France, Japan, and others
- At time of first regulatory approval, ~200 people living with Pompe disease on AT-GAA across extension studies and expanded access programs
- ~75 centers worldwide currently participating in clinical trials and access programs



Pombiliti + Opfolda EU Opportunity

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

- Strong indication statement:
 - *Pombiliti® (cipaglucosidase alfa) is a long-term enzyme replacement therapy used in combination with the enzyme stabiliser Opfolda® (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 Europe¹
 - ~60 Patients throughout EU currently on Pombiliti + Opfolda, including ~20 in Germany and Austria
- Launch underway in Germany
 - 6 month “free pricing” period and AMNOG reimbursement process
 - First patients dosed and additional patients scheduled to start infusions



Launch of Pombiliti + Opfolda Underway in the EU

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

 **Pombiliti™** +  **Opfolda™**
(cipaglucosidase alfa) (miglustat) capsules



Performance

Patient Demand

Initial focus on clinical trial and expanded access patients

First patients dosed; Multiple scheduled for infusion

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



KOL and Patient Outreach

Promotion and Education Efforts

Existing relationships with HCPs at key treatment centers

Engaging top prescribers within first 30 days

Ongoing disease education



Access and Reimbursement

Positive Interactions with Payors

Focus on broad patient access

Country-by-country reimbursement process

Active discussions to demonstrate value

Corporate Outlook

Delivering on our mission for patients and shareholders

Financial Outlook and Path to Profitability

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



Sustain Revenue Growth

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

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



Secure Approvals of AT-GAA

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



Deliver on Financial Goals

Focused on prudent
expense management

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

Achieve profitability¹
in 2H 2023

Positioned for Significant Value Growth

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



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



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



Advance next-generation gene therapies in Fabry and Pompe diseases



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



Achieve non-GAAP profitability in 2H 2023¹

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

>350 Patients*

YE17

>2,000 Patients*

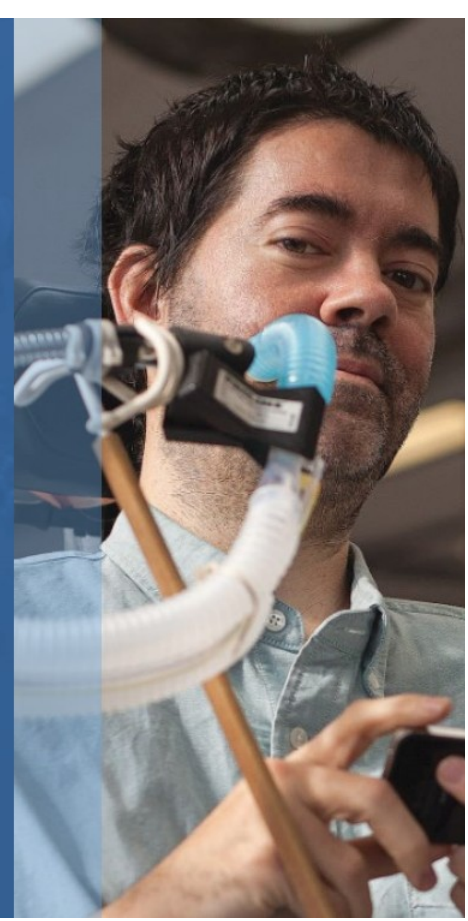
YE22

Thousands of Patients*

2023+



Appendix



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

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

2023 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

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*

Diversity, Equity, & Inclusion (DEI)

Pledge to support a more inclusive culture to improve our employees, our communities, and society.

Goal of maintaining gender diversity while increasing overall diversity throughout our global workforce

0% Amicus Owned Direct Manufacturing and Related GHG Emissions

Global Employees **484** % Female Employees **57%**
% Hiring Slate Diversity **97%**



Employee Recruitment, Engagement, & Retention

Leverage employee capabilities and expertise to promote 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*

Board of Directors

Committed to ongoing Board refreshment and diversity of background, gender, skills, and experience:

Director Diversity **80%** Board Independence
3 Female
2 Veteran Status
1 African American
60% Overall Board Diversity

Career Development

Reimagined performance management process to measure the what and the how, rewarding the best and role-model our **Mission-Focused Behavior**



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

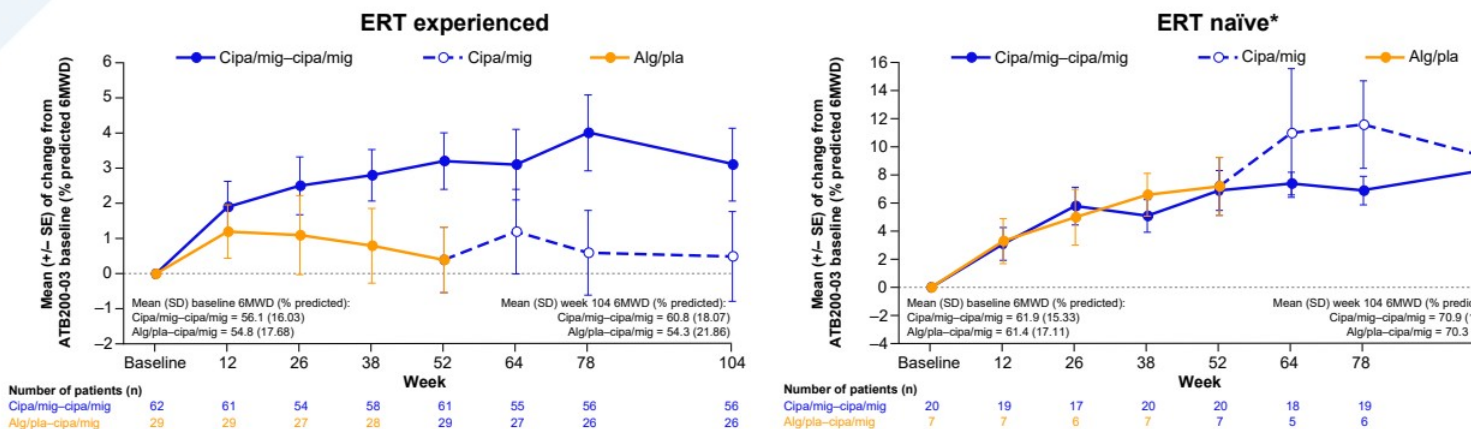
Contributions allocated:
\$2,288,998 U.S.
\$954,349 Intl.

Expanded Access through Feb 2023:
79 patients / **19** countries

Amicus supported community programs: **22**
Volunteer hours (U.S.): **580**

WORLDsymposium Update – Phase 3 OLE of AT-GAA in LOPD

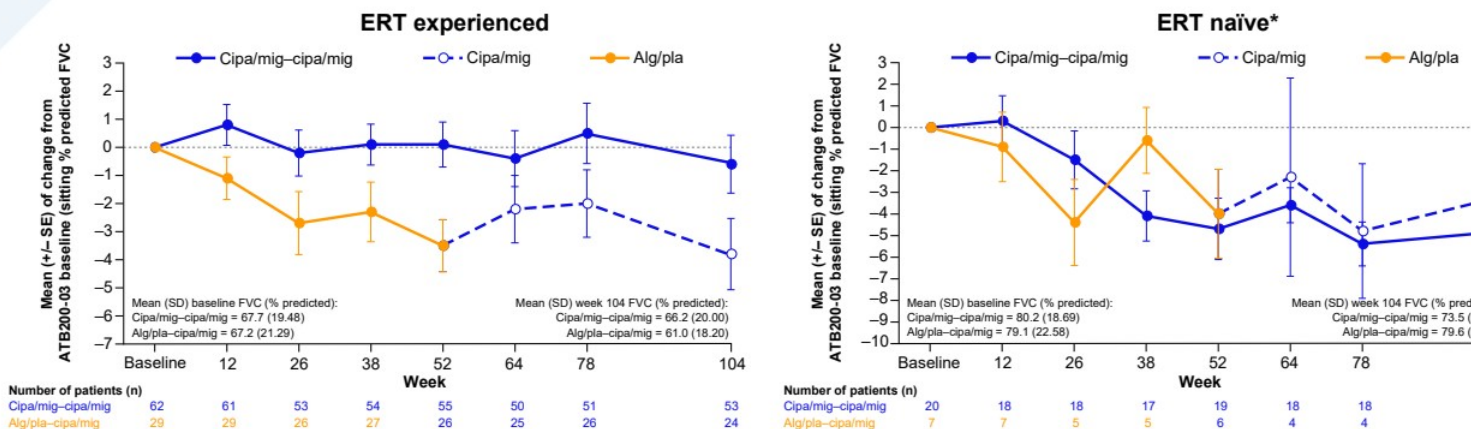
Improvement from the PROPEL baseline in % predicted 6MWD for the cipa/mig group was maintained throughout the OLE for ERT-experienced and ERT-naïve patients



- ERT-experienced and -naïve patients treated with cipa/mig throughout showed durable improvements in % predicted 6MWD in PROPEL that were maintained throughout the OLE to week 104
- ERT-experienced and -naïve patients who received alg/pla in PROPEL and switched to cipa/mig in the OLE showed stability in % predicted 6MWD throughout the OLE

WORLDsymposium Update – Phase 3 OLE of AT-GAA in LOPD

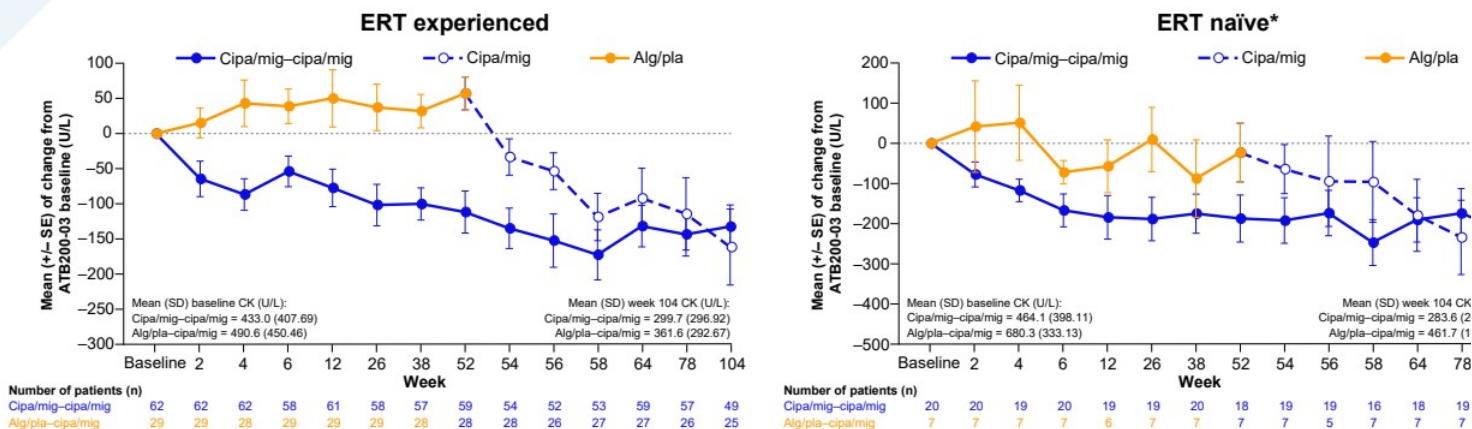
Sitting % predicted FVC remained stable in ERT-experienced and ERT-naïve patients throughout the OLE for both PROPEL treatment groups



- ERT-experienced patients treated with cipa/mig throughout remained stable, while patients who received alg/pla in PROPEL experienced a decline in sitting % predicted FVC that stabilized after switching to cipa/mig in the OLE
- ERT-naïve patients in both treatment groups experienced some decline in PROPEL that stabilized in the OLE with no further decline in FVC to week 104

WORLDsymposium Update – Phase 3 OLE of AT-GAA in LOPD

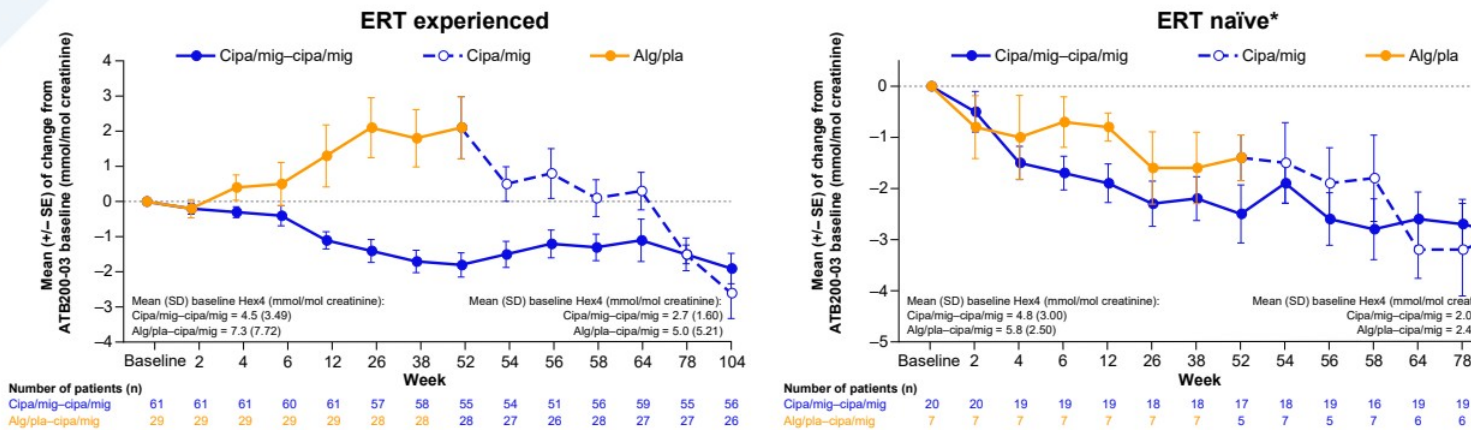
Cipa/mig treatment was associated with a durable reduction in serum CK during PROPEL and the OLE in both ERT-experienced and ERT-naïve patients



- ERT-experienced and -naïve patients treated with cipa/mig throughout showed a decline in serum CK levels during PROPEL that was maintained throughout the OLE
- ERT-experienced and -naïve patients who received alg/pla in PROPEL showed a slight increase or stability in serum CK levels to week 52, and a marked decline after switching to cipa/mig in the OLE

WORLDsymposium Update – Phase 3 OLE of AT-GAA in LOPD

Cipa/mig treatment was associated with a durable reduction in urine Hex4 during PROPEL and the OLE in both ERT-experienced and ERT-naïve patients



- ERT-experienced patients treated with cipa/mig throughout experienced a decline in urine Hex4 levels in PROPEL that stabilized during the OLE. ERT-experienced patients who received alg/pla in PROPEL experienced an increase in Hex4 and a marked decline after switching to cipa/mig in the OLE
- ERT-naïve patients experienced a decline in Hex4 levels during PROPEL in both treatment groups that stabilized or declined further during the OLE to week 104



Thank you

