

AT THE FOREFRONT OF  
THERAPIES FOR RARE DISEASES

# FY22 Financial Results Conference Call & Webcast

March 1, 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 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, 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 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 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 press release 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 Global  
Regulatory Reviews for  
Pompe Disease

**12-17%**

FY23 Galafold  
Revenue Growth  
at CER



**GALAFOLD  
&  
AT-GAA**

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

**\$294M**

Cash  
as of 12/31/22



# 2023 Strategic Priorities

- 1 Sustain double-digit Galafold revenue growth of 12-17% at CER<sup>1</sup>**
- 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**



# Galafold<sup>®</sup> (migalastat) Continued Growth

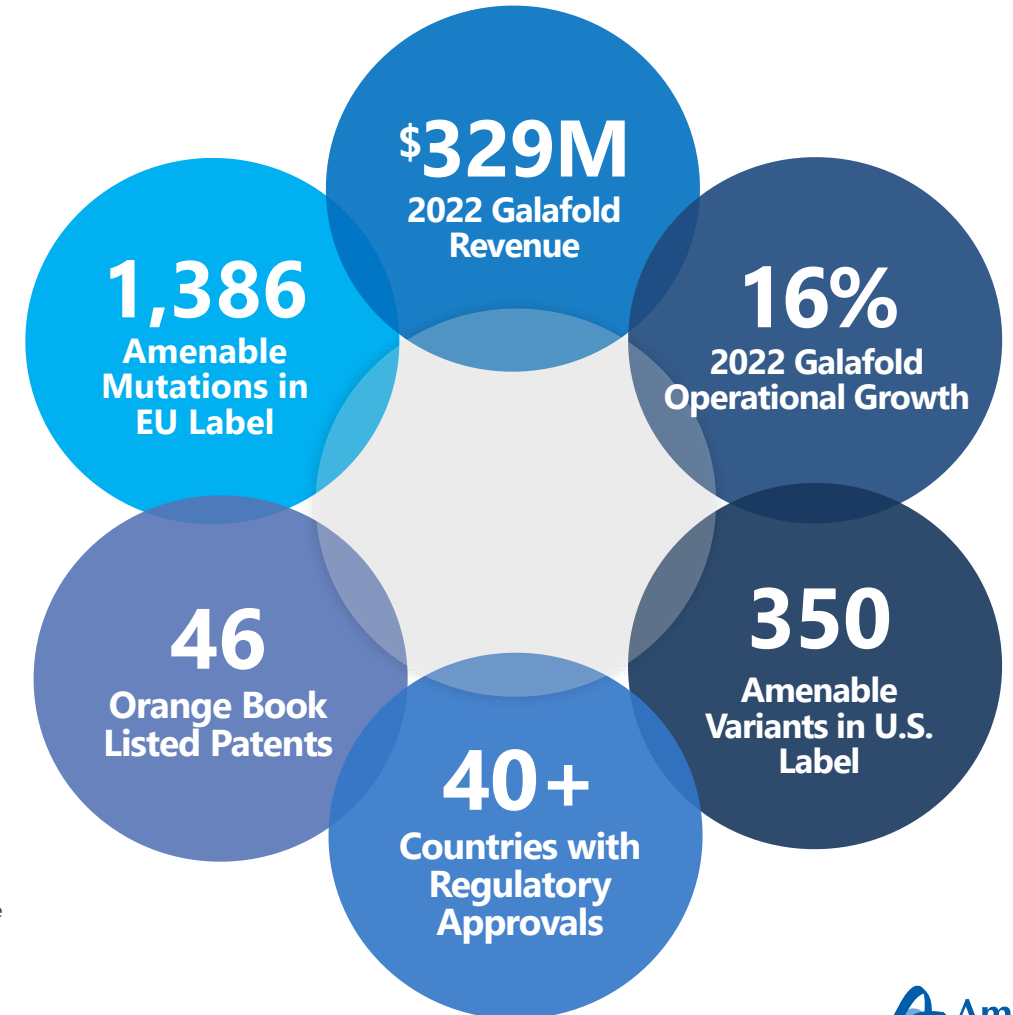
Building a leadership position in the  
treatment of Fabry disease



# 2022 Galafold Success (as of December 31, 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

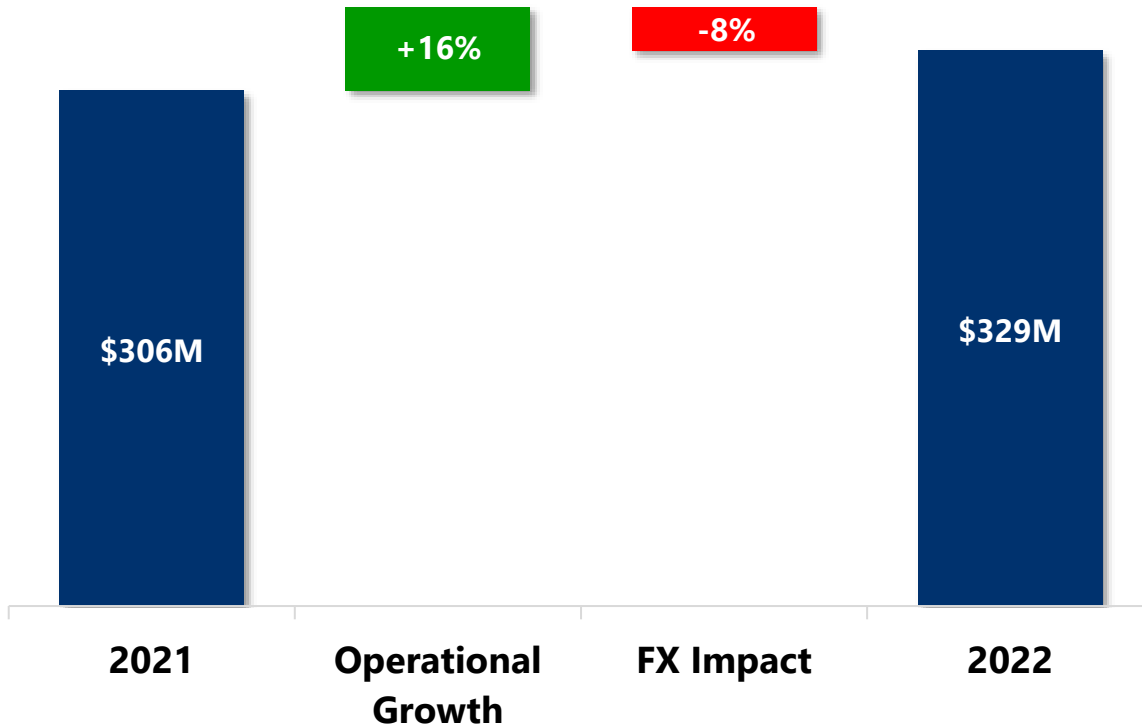


Galafold is indicated for adults with a confirmed diagnosis of Fabry disease and an amenable variant. The most common adverse reactions reported with Galafold ( $\geq 10\%$ ) were headache, nasopharyngitis, urinary tract infection, nausea, and pyrexia. For additional information about Galafold, including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

# Galafold Performance

2022 reported revenue growth of +8% to \$329M – Strong operational growth of +16% at CER

## Year-over-Year Sales Growth



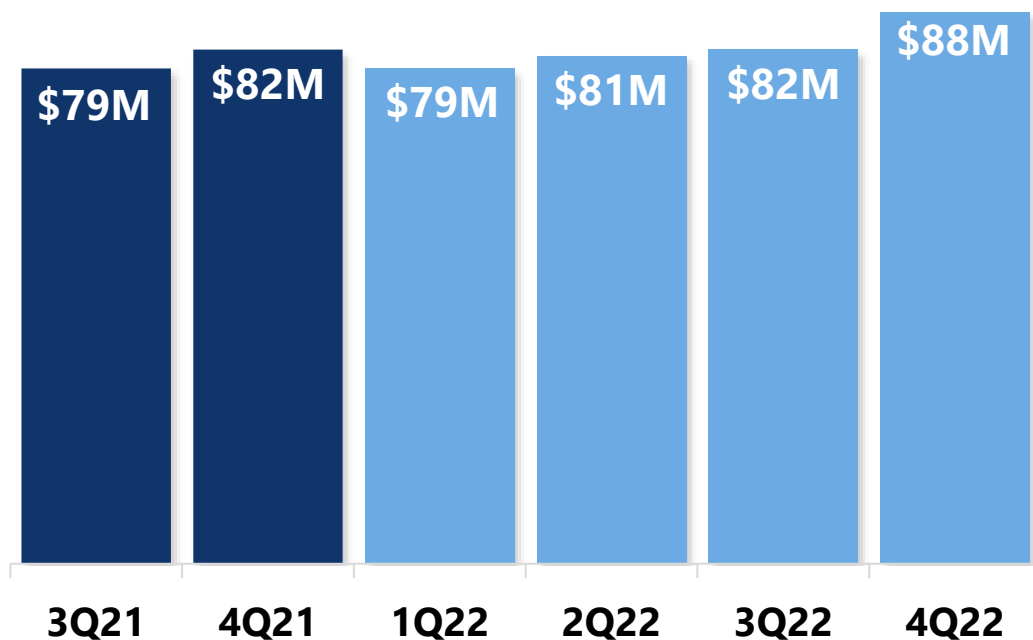
## FY22 Strength Reflects Increasing Demand with >2,000 Individuals Treated

- Global 3-month net new patients trend highest in 2 years
- ~50% share of treated amenable patients
- Interactions with HCPs increasing from same period last year
- Healthy global mix of switch (~55%) and previously untreated patients (~45%)<sup>1</sup>
- Compliance and adherence over 90%+

# Galafold Quarterly Trends

Growth remains strong with Q4 revenue of \$88M and FY22 revenue of \$329M

### Quarterly Galafold Sales



- Expect non-linear quarterly growth to continue due to uneven ordering patterns and FX fluctuations

### Distribution of Galafold Revenue by Quarter in Past 5 years:

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



# Galafold Global Launch Momentum (as of December 31, 2022)

**Strong patient demand with 2,000+ individuals treated with Galafold and performance against key metrics lay the foundation for continued double-digit growth in 2023**

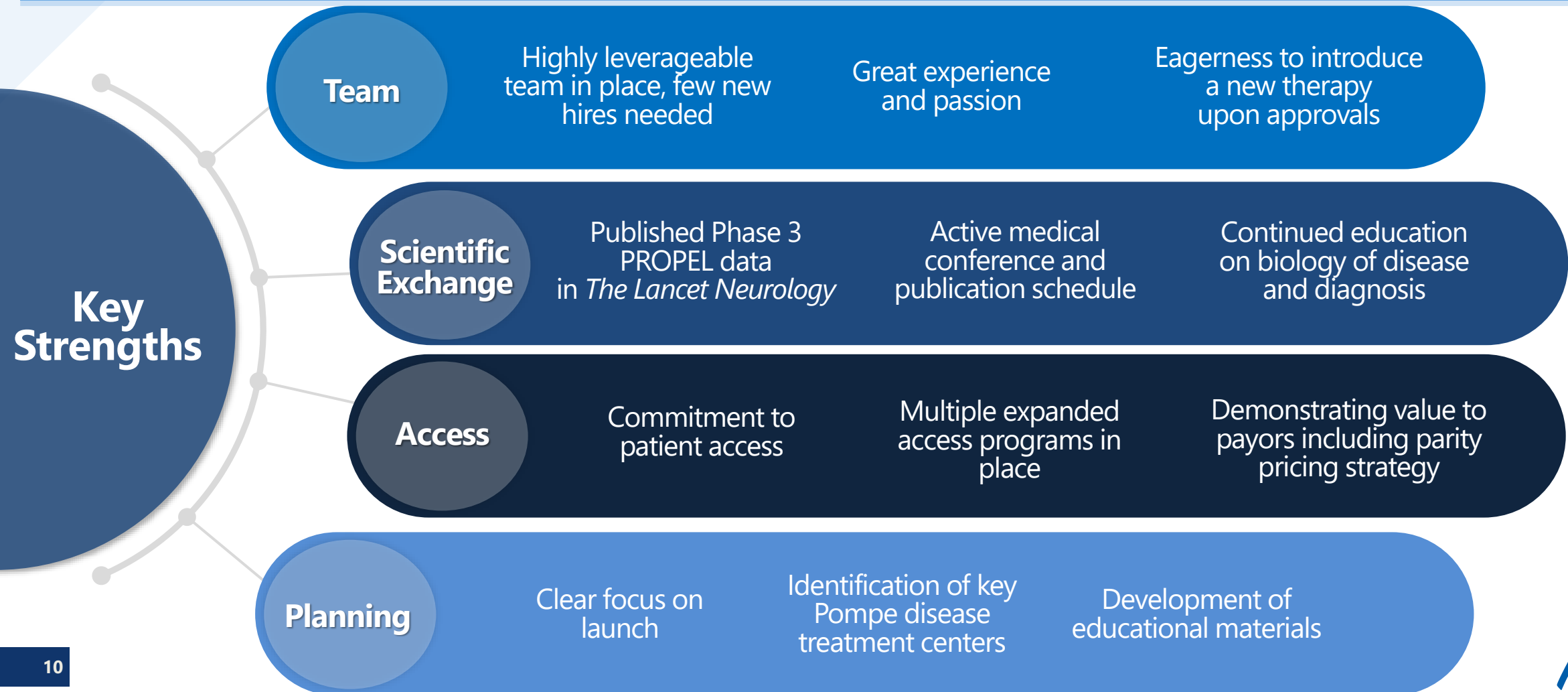
## **Sustained Growth in 2023 Driven by:**

- **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**



# 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;  
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 sales<sup>1</sup>



# AT-GAA: Global Regulatory Status

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



- **Pombiliti™ (cipaglucosidase alfa) European Commission (EC) decision expected in 1Q 2023**
- **Miglustat CHMP opinion expected in 2Q 2023 with EC decision anticipated in 3Q 2023**



- **Pre-approval inspection now scheduled**
- **Anticipating 3Q 2023 FDA approval**



- **U.K. MAA submitted via recognition procedure based on CHMP opinion**
- **Anticipating 3Q 2023 MHRA approval**



# AT-GAA: Ongoing Clinical Studies and Expanded Access Mechanisms

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

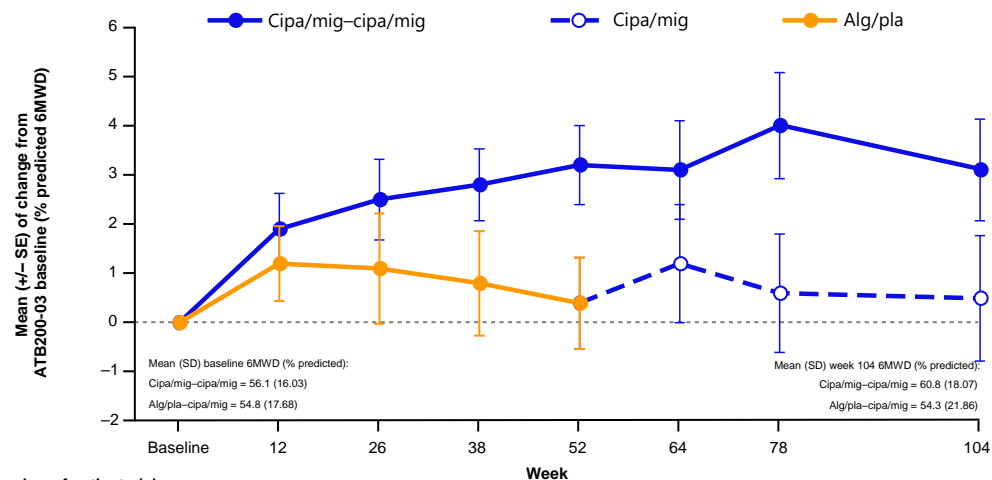
- Ongoing clinical studies in children and adolescents<sup>1</sup> with LOPD as well as in Infantile-Onset Pompe Disease (IOPD)
- Multiple expanded access mechanisms in place, including in the U.S., U.K., Germany, France, Japan, and others
- ~200 people living with Pompe disease are now on AT-GAA across extension studies and expanded access programs
- ~75 centers worldwide currently participating in clinical trials and access programs



# WORLDsymposium Update

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

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



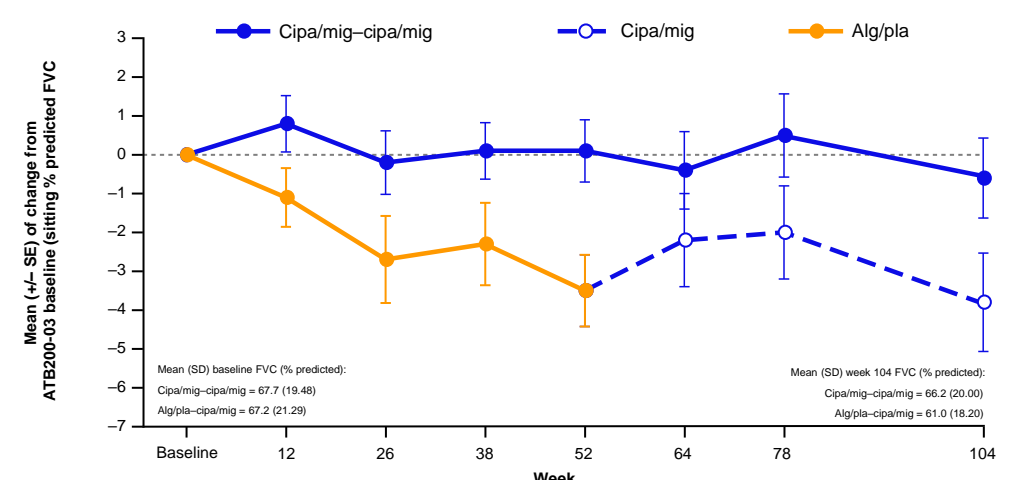
### Number of patients (n)

Cipa/mig-cipa/mig  
Alg/pla-cipa/mig

Week	Cipa/mig-cipa/mig	Alg/pla-cipa/mig
Baseline	62	29
12	61	29
26	54	27
38	58	28
52	61	29
64	55	27
78	56	26
104	56	26

- 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



### Number of patients (n)

Cipa/mig-cipa/mig  
Alg/pla-cipa/mig

Week	Cipa/mig-cipa/mig	Alg/pla-cipa/mig
Baseline	62	29
12	61	29
26	53	26
38	54	27
52	55	26
64	50	25
78	51	26
104	53	24

- 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





# Corporate Outlook

Delivering on our mission for patients and shareholders





# 2022 Select Financial Results

2022 revenue of \$329M and growth rate of 16% at CER from global Galafold sales

*(in thousands, except per share data)*

	Dec. 31, 2022	Dec. 31, 2021
Product Revenue	\$329,233	\$305,514
Cost of Goods Sold	38,599	34,466
R&D Expense	276,677	272,049
SG&A Expense	213,041	192,710
Changes in Fair Value of Contingent Consideration	1,078	6,514
Loss on Impairment of Assets	6,616	—
Depreciation and Amortization	5,342	6,209
Loss from Operations	(212,120)	(206,434)
Income Tax Benefit (Expense)	5,471	(8,906)
Net Loss	(236,568)	(250,460)
Net Loss Per Share	(0.82)	(0.92)

# Financial Outlook and Path to Profitability

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



## Sustain Galafold Revenue Growth

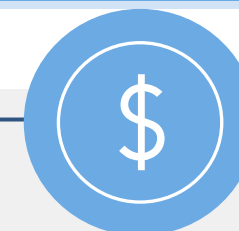
**\$329M** FY22 revenue,  
+16% YoY  
operational growth

2023 Galafold revenue  
growth guidance of  
**+12-17% 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

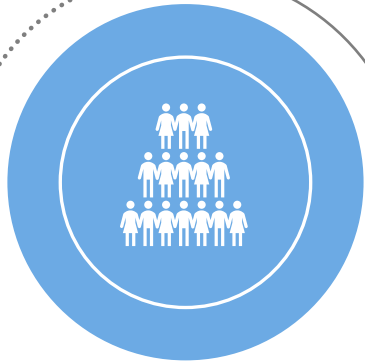
Focused on prudent  
expense management

2023 non-GAAP operating  
expense guidance of  
**\$340M-\$360M**

Achieve profitability<sup>1</sup>  
in 2H 2023

# Positioned for Significant Value Growth

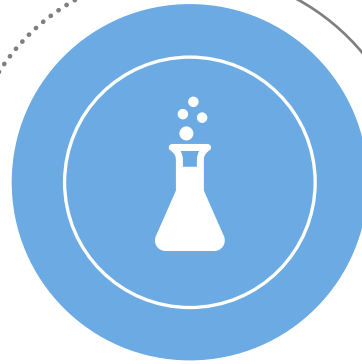
Focused on execution and driving sustainable double-digit revenue growth on path to profitability



Continue to bring Galafold<sup>®</sup> 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



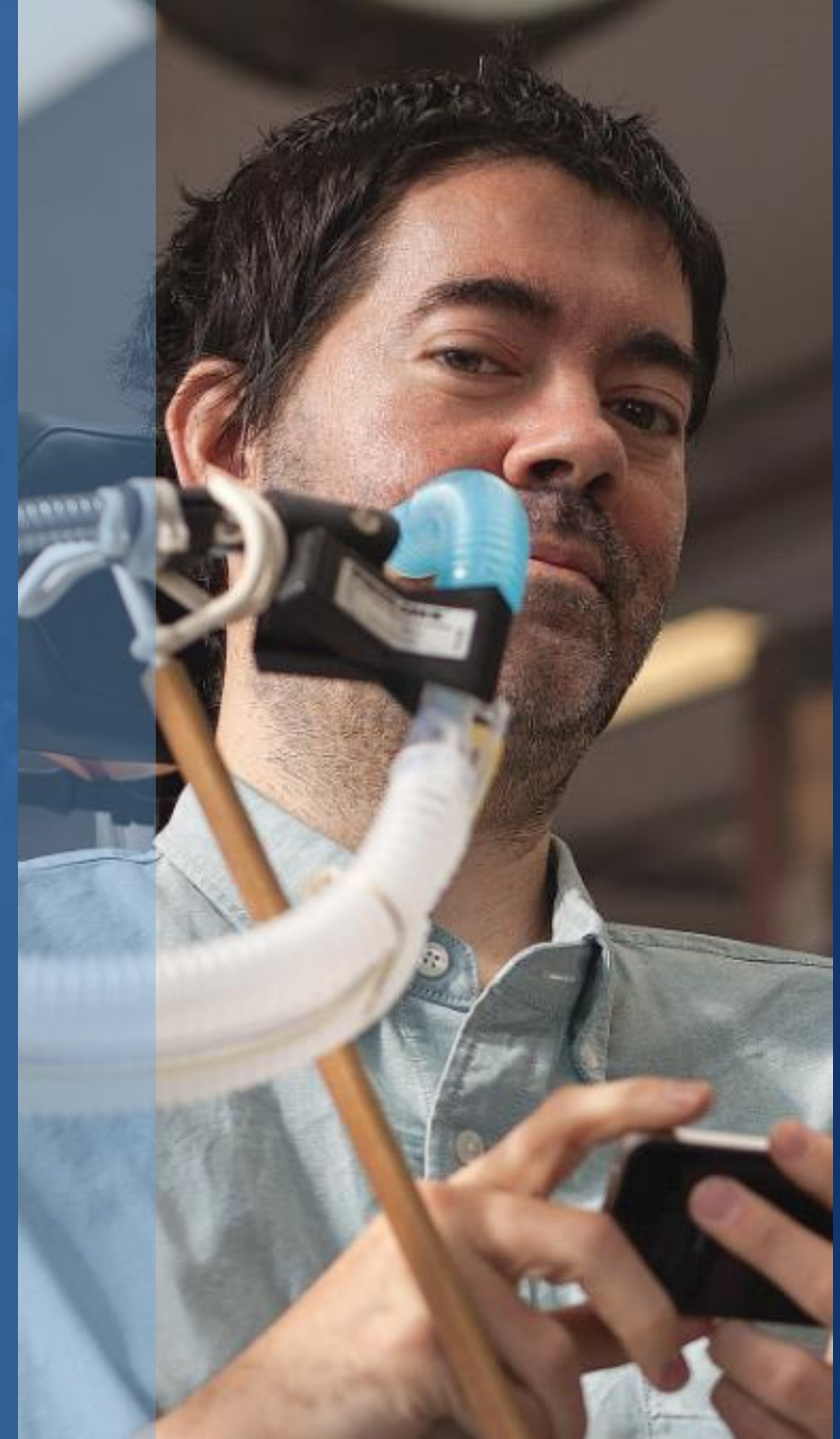
Fully leverage global capabilities and infrastructure as a leader in rare diseases



Achieve non-GAAP profitability in 2H 2023<sup>1</sup>



# Appendix





# Appendix

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

	December 31		
	2022	2021	2020
<b>Total operating expenses - as reported GAAP</b>	<b>\$502,754</b>	<b>\$ 477,482</b>	<b>\$ 476,840</b>
<b>Research and development:</b>			
Share-based compensation	25,089	17,340	20,817
<b>Selling, general and administrative:</b>			
Share-based compensation	51,423	40,498	28,334
<b>Loss on impairment of assets</b>	6,616	—	—
<b>Changes in fair value of contingent consideration payable</b>	1,078	6,514	3,144
<b>Depreciation and amortization</b>	5,342	6,209	8,846
<b>Total operating expense adjustments to reported GAAP</b>	<b>89,548</b>	<b>70,561</b>	<b>61,141</b>
<b>Total operating expenses - as adjusted</b>	<b>\$ 413,206</b>	<b>\$ 406,921</b>	<b>\$ 415,699</b>

# Environmental, Social, & Governance (ESG) Snapshot

## Whom 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 12/31/21)

Contributions allocated:  
**\$1,677,000** U.S.

**\$832,976** Intl.

Expanded Access through Jan 2023:

**74** patients / **20** countries

Amicus supported community programs:

**20+**

Volunteer hours (U.S.):

**770**

## Diversity, Equity, & Inclusion (DEI)

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

### 2023 and Beyond:

- Maintain strength in global gender diversity
- Increase US diversity through intentional and ongoing action
- Continuously evaluate compensation practices to ensure pay parity

Global Employees % female employees

**496** **58%**

% Hiring Slate Diversity **82%**

## Board of Directors

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

### Director Diversity

**3** Female  
**2** Veteran Status  
**1** African American

**80%** Board Independence

**60%** Overall Board Diversity

## Environmental Management

Eco-friendly decision-making has unearthed economic efficiencies while continuing to bolster our standing as a good corporate citizen.

Green building design

Energy & water conservation

Hazardous waste management

## Employee Recruitment, Engagement, & Retention

Leverage employee capabilities and expertise to provide a culture that drives performance and ultimately attracts, energizes, and retains critical talent.

*Pulse surveys reveal employees feel **high personal satisfaction** in their job, are **proud of their work** and what they contribute to the community*

## Career Development

Reimagined performance management process to measure the what and the how, rewarding those who role-model our **Mission-Focused Behaviors.**

# FX Sensitivity and Galafold Distribution of Quarterly Sales

## Impact from Foreign Currency Q4 2022

Currency Variances: USD/	Q4 2021	Q4 2022	Variance
EUR	1.144	1.021	(10.7%)
GBP	1.348	1.174	(12.9%)
JPY	0.009	0.007	(19.5%)

## Full Year 2023 Revenue Sensitivity

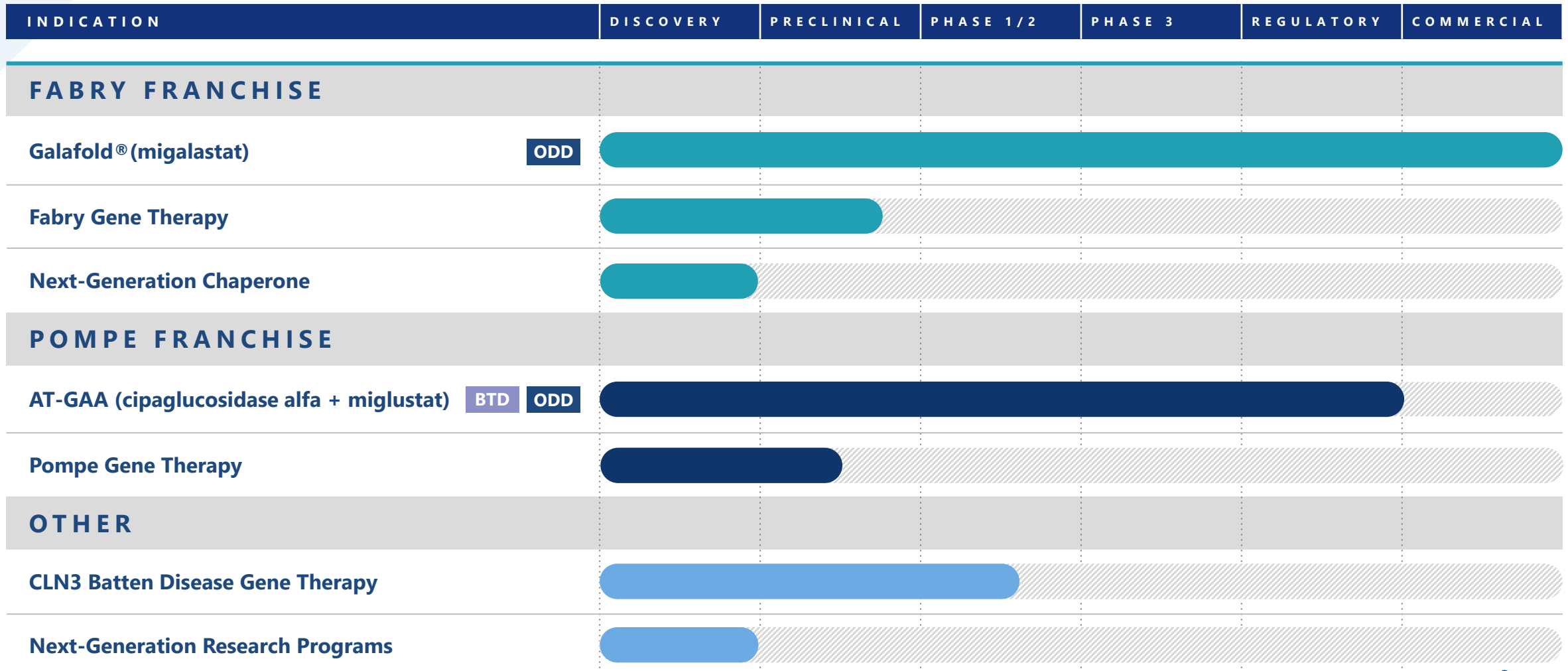
Given the high proportion of Amicus revenue Ex-US, a change in exchange rates of +/- 5% compared to year end 2022 rates could lead to a \$11M-\$12M change in global reported revenues in 2023.

## Distribution of Galafold Revenue by Quarter in Past 5 years:

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

# Amicus Pipeline

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





# AT-GAA Phase 3 PROPEL Study Results

Clinically meaningful outcomes from Phase 3 PROPEL study provide the basis for global regulatory submissions of AT-GAA

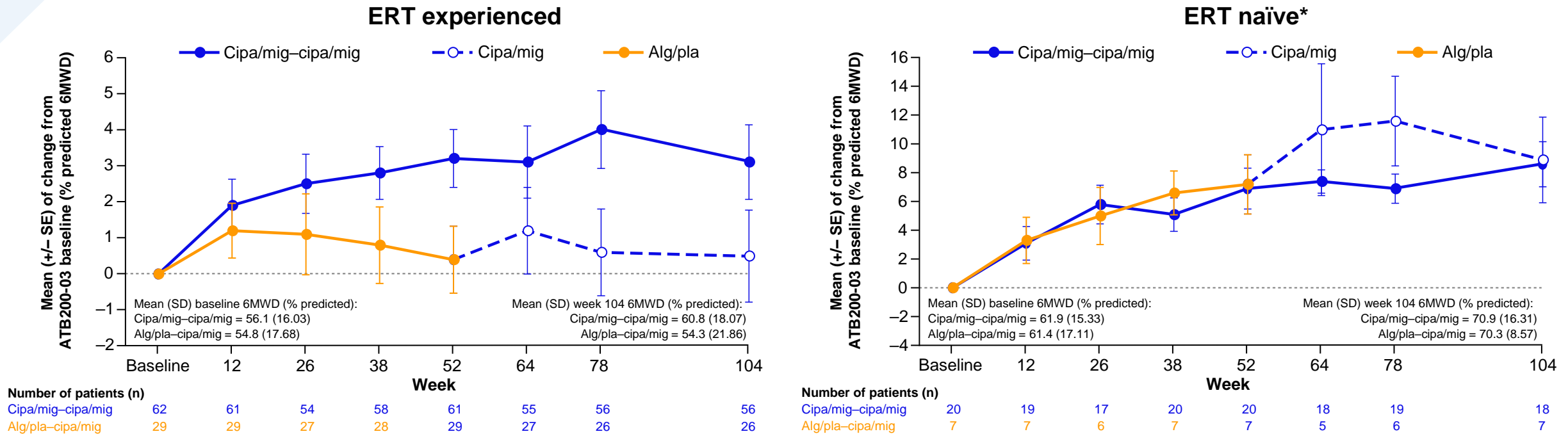
	Endpoints	Overall population				ERT-experienced			
		Cipaglucosidase alfa/miglustat n=85		Alglucosidase alfa/placebo n=37		Cipaglucosidase alfa/miglustat n=65		Alglucosidase alfa/placebo n=30	
		Baseline, mean	CFBL at week 52, mean (SE)	Baseline, mean	CFBL at week 52, mean (SE)	Baseline, mean	CFBL at week 52, mean (SE)	Baseline, mean	CFBL at week 52, mean (SE)
Motor function	6MWD, m	357.9	20.8 (4.6)	351.0	7.2 (6.6)	346.9	16.9 (5.0)	334.6	0.0 (7.2)
	GSGC total score	14.5	-0.5 (0.3)	14.5	0.8 (0.3)	15.6	-0.5 (0.3)	15.5	0.6 (0.4)
	10-meter walk, s	9.7	-0.5 (0.6)	9.6	1.9 (1.0)	10.4	-0.6 (0.9)	10.2	2.5 (1.2)
	4-stair climb, s	14.1	-8.5 (7.9)	8.2	0.3 (1.0)	17.3	-11.1 (10.5)	9.3	0.6 (1.2)
	Gower's maneuver, s	10.8	-0.3 (0.7)	19.8	-2.2 (1.4)	11.5	-0.4 (0.8)	23.9	-2.6 (1.9)
	Rising from chair, s	13.6	-10.2 (9.7)	4.5	-0.5 (0.7)	17.6	-13.7 (13.0)	5.2	-0.4 (0.9)
Pulmonary function	FVC, % predicted	70.7	-0.9 (0.7)	69.7	-4.0 (0.8)	67.9	0.1 (0.7)	67.5	-4.0 (0.9)
	MIP, % predicted	61.8	2.1 (2.1)	59.9	-2.7 (2.8)	61.3	1.0 (2.5)	55.0	-1.7 (1.5)
	MEP, % predicted	70.7	0.6 (2.4)	65.1	-1.6 (2.1)	70.7	-2.7 (2.7)	62.2	-3.9 (1.8)
Muscle strength	Lower MMT score	28.0	1.6 (0.4)	27.7	0.9 (0.4)	26.4	1.6 (0.5)	26.1	0.9 (0.5)
	Upper MMT score	34.3	1.5 (0.4)	34.7	0.7 (0.6)	33.7	1.8 (0.4)	34.2	0.4 (0.7)
	Total MMT score	62.3	3.1 (0.7)	62.4	1.4 (0.8)	60.1	3.4 (0.9)	60.3	1.1 (0.9)
PROs	PROMIS®-Physical Function	66.9	1.9 (0.8)	68.0	0.2 (1.8)	64.4	1.8 (0.9)	66.9	-1.0 (2.0)
	PROMIS®-Fatigue	22.3	-2.0 (0.6)	21.1	-1.7 (1.1)	22.0	-1.9 (0.7)	20.4	-0.3 (1.0)
Biomarkers	Urine Hex4, mmol/mol	4.6	-1.9 (0.3)	6.9	1.2 (0.7)	4.6	-1.7 (0.3)	7.2	1.9 (0.8)
	Serum CK, U/L	447.0	-130.5 (25.1)	527.8	60.2 (26.2)	441.8	-118.0 (28.4)	492.3	79.6 (26.9)

Based on LOCF means

■ Treatment group favored ■ Nominal statistical significance ( $P < 0.05$ )

# WORLDSymposium Update – Phase 3 OLE of AT-GAA in LOPD

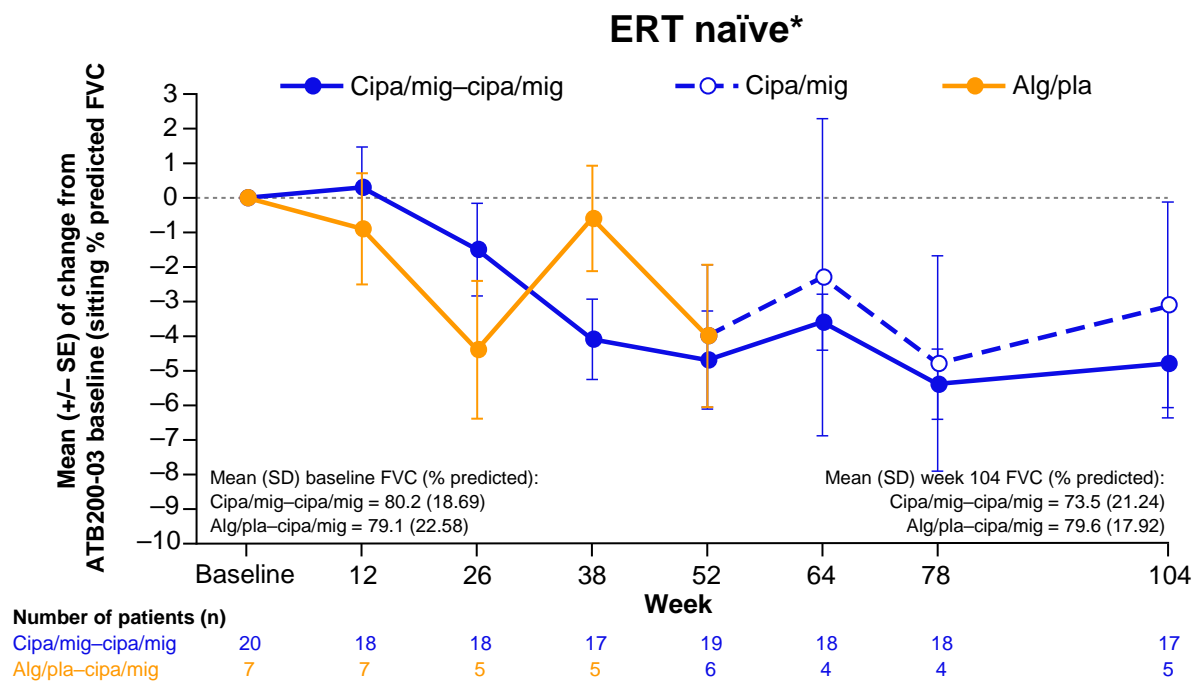
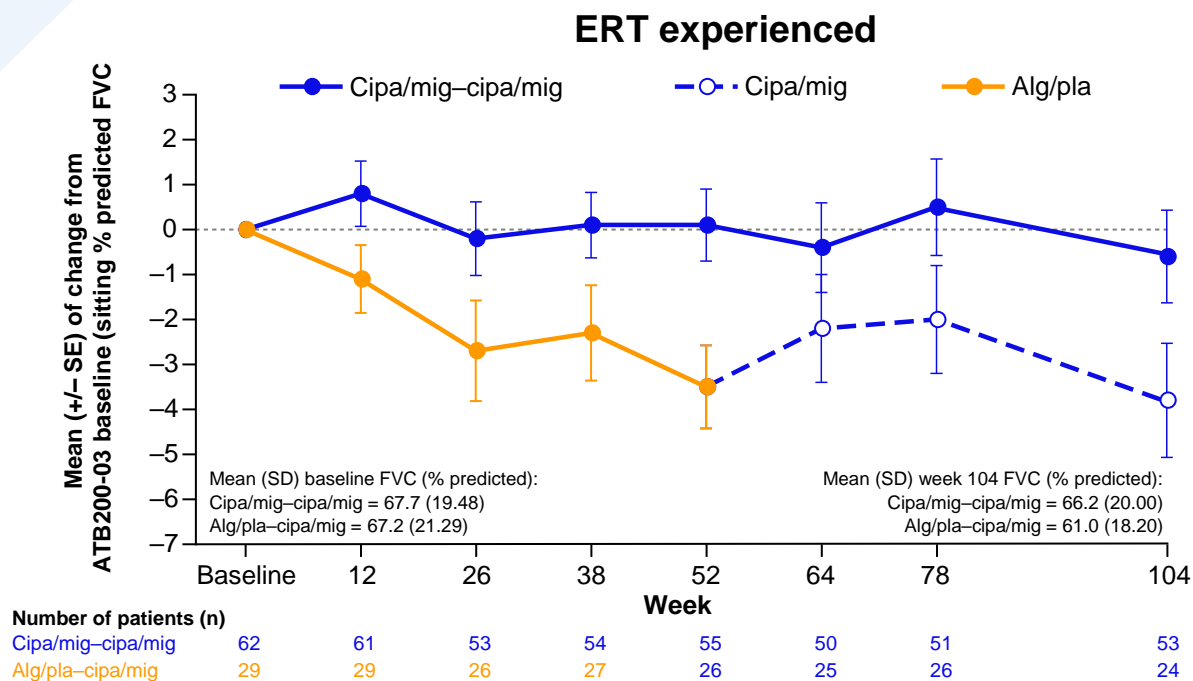
Improvement from the PROPEL baseline in % predicted 6MWD for the cipa/mig group was maintained throughout the OLE for ERT-experienced and ERT-naïve patients



- 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

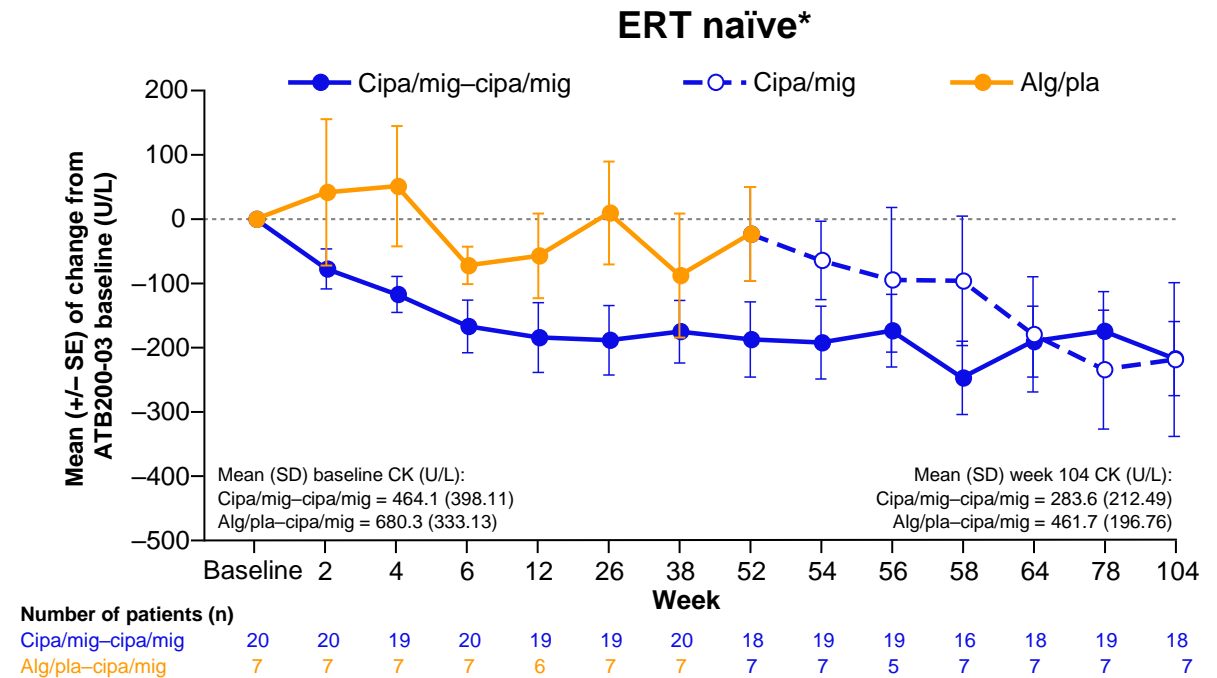
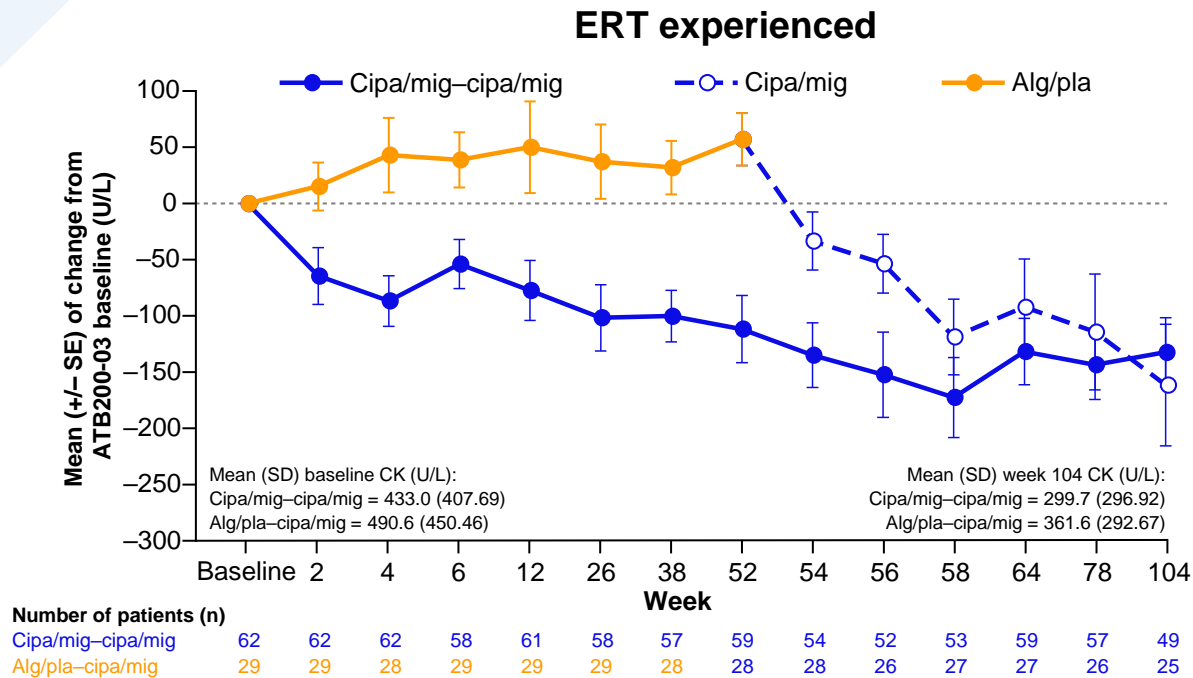
Sitting % predicted FVC remained stable in ERT-experienced and ERT-naïve patients throughout the OLE for both PROPEL treatment groups



- 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

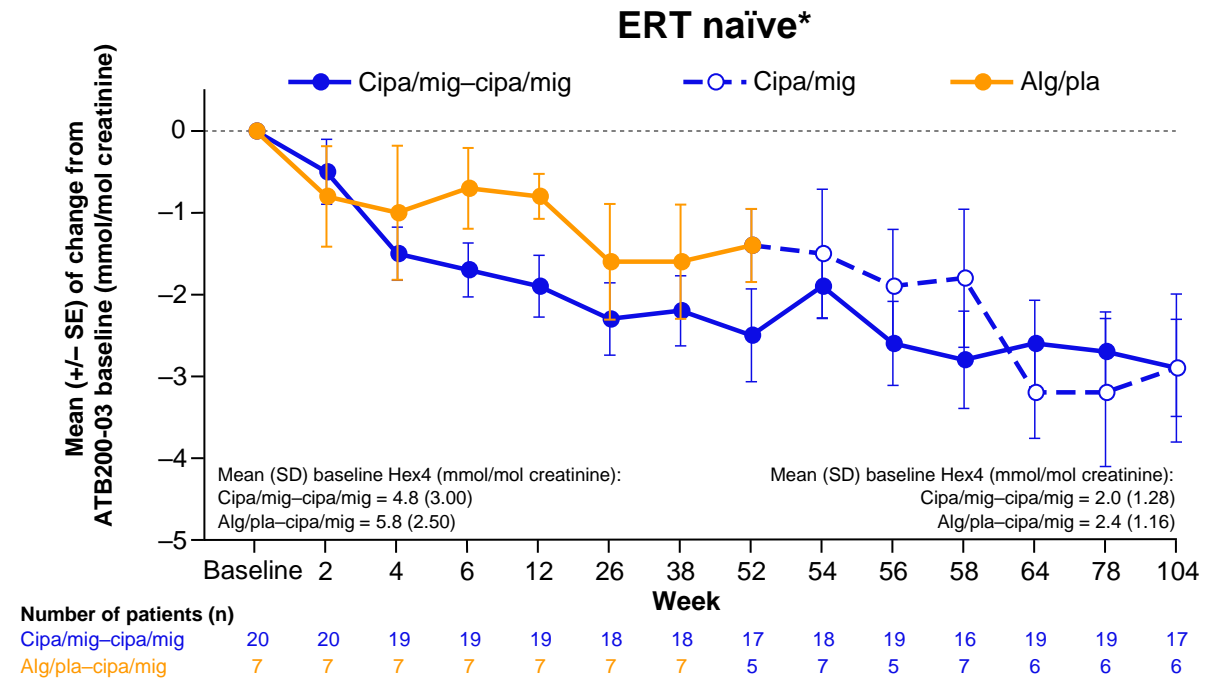
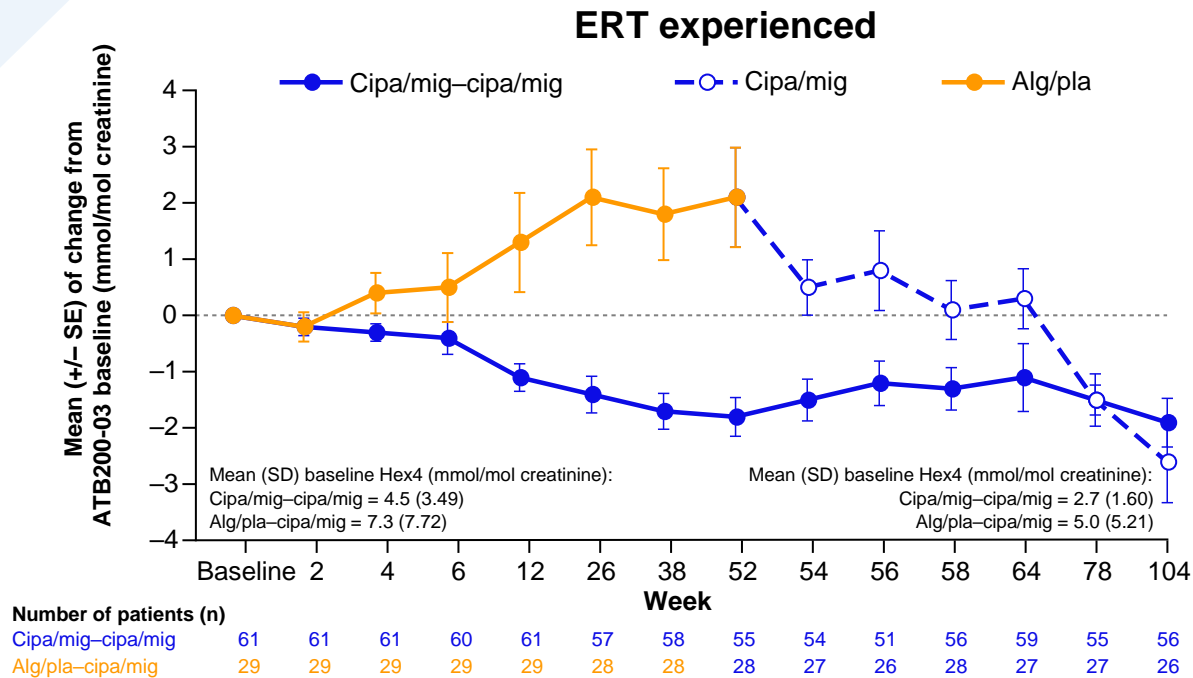


- ERT-experienced and -naïve patients treated with cipa/mig throughout showed a decline in serum CK levels during PROPEL that was maintained throughout the OLE
- ERT-experienced and -naïve patients who received alg/pla in PROPEL showed a slight increase or stability in serum CK levels to week 52, and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