AT THE FOREFRONT OF
THERAPIES FOR RARE DISEASES

41ST Annual J.P. Morgan Healthcare Conference

January 9, 2023

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 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 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, 2021 and the Quarterly Report filed on Form 10-Q for the quarter ended September 30, 2022. 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.

Our Passion is for Patients

Our Mission:

We seek to deliver the highest quality therapies for people living with rare diseases

Our Vision:

Be a leader in rare disease drug development and commercialization leveraging our global capabilities in bringing life-changing therapies to patients

Definition:

\əˈmēkəs (noun) *Latin* Friend

Amicus Therapeutics

A Rare Company

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

Galafold® (migalastat)

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 **2H2023**

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

$355M

Cash as of 9/30/22

Amicus Therapeutics

2022: A Year in Headlines

2022 Galafold® (migalastat) Operational Revenue Growth of 16%

Issuance of New U.S. Composition of Matter Patent for Galafold® (migalastat), Strengthening Patent Protection Through 2038

Positive Long-Term Data from Phase 1/2 Study of AT-GAA in Pompe Disease

Successful Company Leadership Transition

Growing Demand for AT-GAA in the United Kingdom through the MHRA's EAMS Program

Research Collaboration for Next-generation Pharmacological Chaperones in Fabry Disease

Long-term Efficacy of migalastat on Fabry-Associated Clinical Events, including Renal, Cardiac, and Cerebrovascular Outcomes

Type A Meeting Scheduled with FDA to Discuss Inspection Logistics for AT-GAA U.S. Regulatory Approval

Positive CHMP Opinion for Pombiliti™ (cipaglucosidase alfa) in Late-Onset Pompe Disease

Amicus Therapeutics

2023 Strategic Priorities

1. **Sustain double-digit Galafold revenue growth of 12-17% at CER[1]**
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**

[1]CER: Constant Exchange Rates; 2023 Galafold revenue guidance utilizes actual exchange rate as of December 31, 2022

Amicus Therapeutics

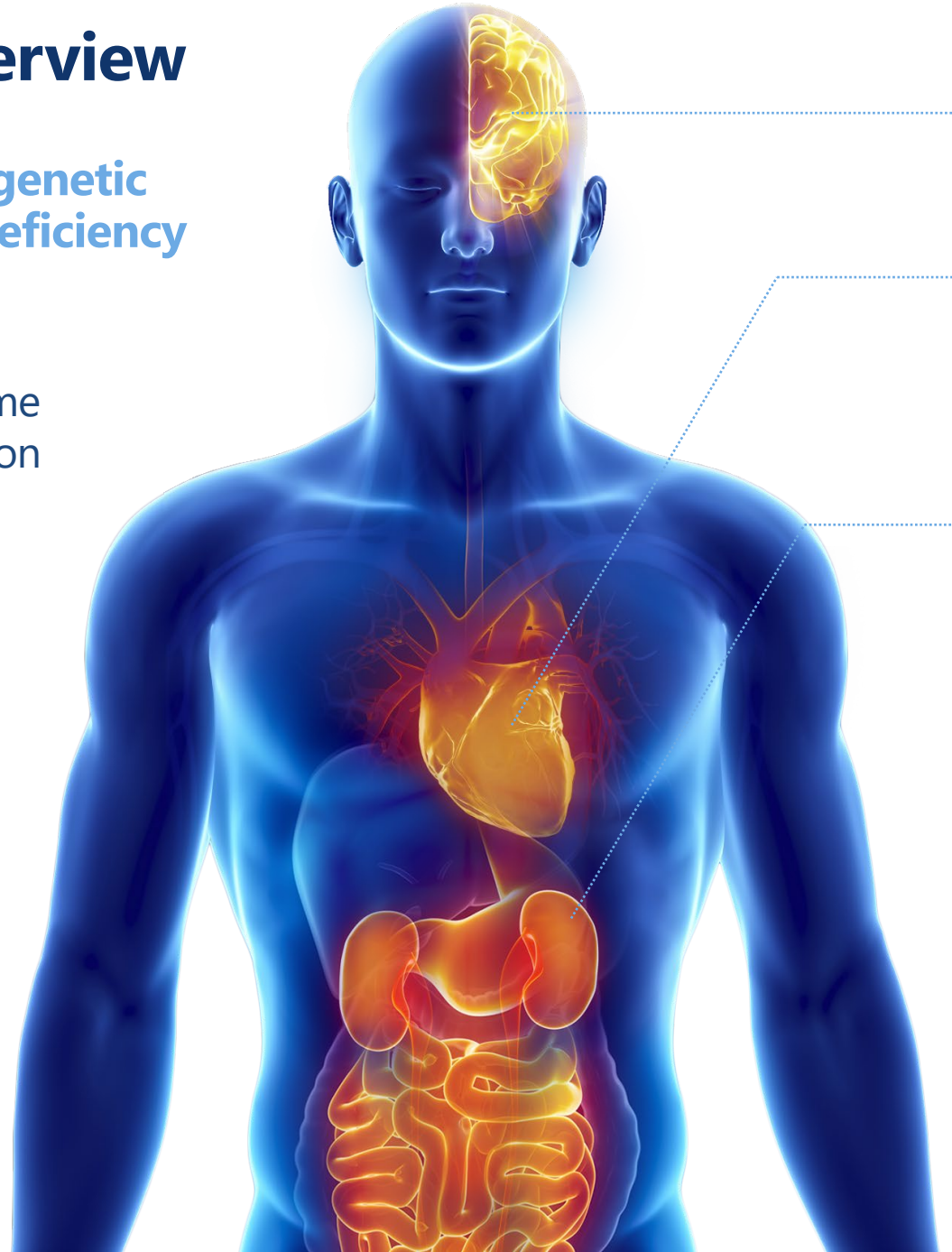
Galafold® (migalastat) Continued Growth

Building a leadership position in the treatment of Fabry disease

Fabry Disease Overview

Fabry is a rare inherited genetic disorder caused by the deficiency of the GLA enzyme

- Deficiency of α-Gal A enzyme leading to GL-3 accumulation
- 1,000+ known variants
- 16,000+ diagnosed WW (51% female/49% male[4])



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

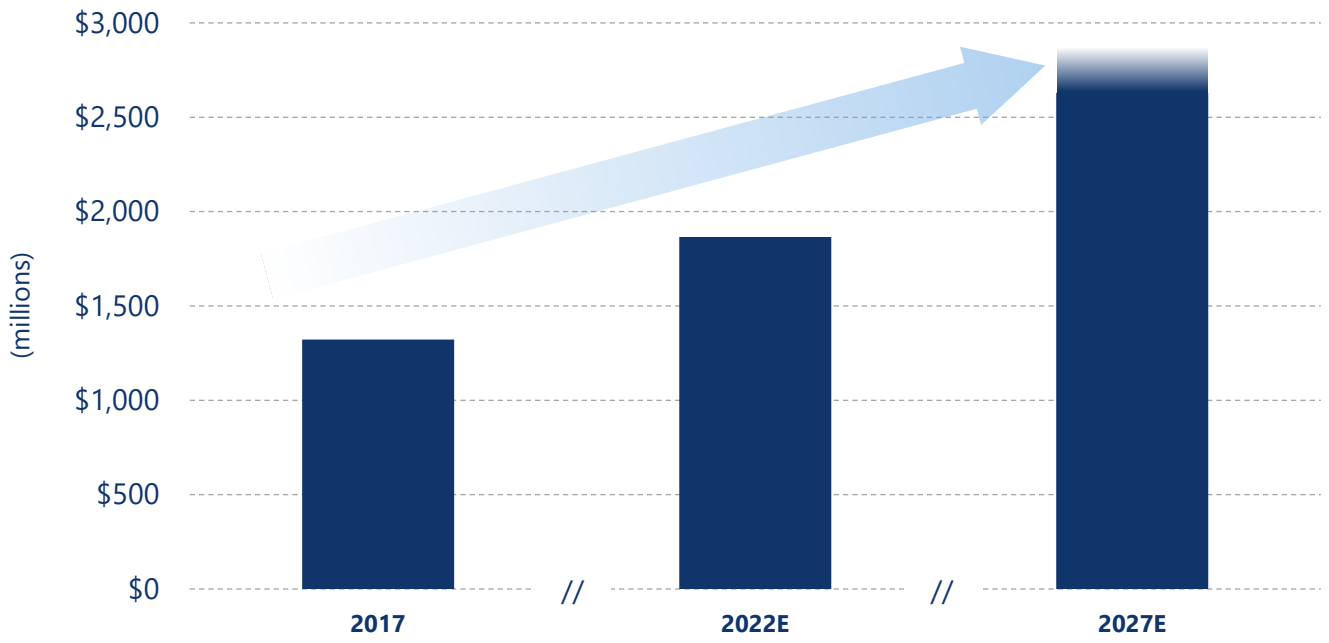
1. Desnick R, *et al.* Ann Intern Med. 2003
2. Yousef Z, *et al.* Eur Heart J. 2013
3. Germain D. Orphanet J Rare Dis. 2010
4. Data on file

Amicus Therapeutics

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 2027[1]



- Believed to be significantly underdiagnosed
  - Newborn screening studies suggest Fabry is one 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+ naïve patients treated

[1]Global market measured by reported sales of approved therapies for Fabry disease – 2027 sales projected using ~8% CAGR

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 (≥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.

[1] Preliminary and unaudited

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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

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
- Healthy mix of switch (55%) and previously untreated patients (45%)[1]
- Compliance and adherence >90%
- Growing prescriber base

Sustained Growth in 2023 Driven by:

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

[1]Data on file

Galafold Studies and Real-World Evidence

Growing body of evidence for Galafold on compliance, impacts on quality of life, long-term efficacy and importance of early treatment

ELSEVIER

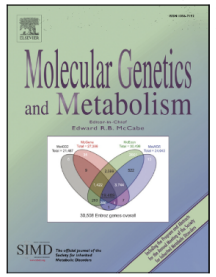
Molecular Genetics and Metabolism Reports

MGM Reports

Long-term follow-up of renal function in patients treated with migalastat for Fabry disease

Daniel G. Bichet[a,*], Roser Torra[b], Eric Wallace[c], Derralynn Hughes[d], Roberto Giugliani[e], Nina Skuban[f,1], Eva Krusinska[f,2], Ulla Feldt-Rasmussen[g], Raphael Schiffmann[h], Kathy Nicholls[i]

Patient reported quality of life and medication adherence in Fabry disease patients treated with migalastat: A prospective, multicenter study



Jonas Müntze, Kolja Lau, Markus Cybulla, Eva Brand, Tereza Cairns, Lora Lorenz, Nurcan Üçeyler, Claudia Sommer, Christoph Wanner, Peter Nordbeck

Therapeutics

OPEN ACCESS

Original research

Long-term multisystemic efficacy of migalastat on Fabry-associated clinical events, including renal, cardiac and cerebrovascular outcomes

Derralynn A Hughes ,[1] Daniel G Bichet,[2] Roberto Giugliani,[3] Robert J Hopkin,[4] Eva Krusinska,[5] Kathleen Nicholls,[6] Iacopo Olivotto,[7] Ulla Feldt-Rasmussen ,[8] Norio Sakai,[9] Nina Skuban,[5] Gere Sunder-Plassmann,[10] Roser Torra,[11] William R Wilcox[12]

Hindawi

Case Report

The Benefits of Early versus Late Therapeutic Intervention in Fabry Disease

Mónica Furlano,[1] Elisabet Ars,[2] Anna Matamala,[3] Vicens Brossa,[4] Joan Martí,[5] Maria del Prado-Venegas,[6] Jaume Crespi,[7] Esther Roe,[8] and Roser Torra[1]

Amicus Therapeutics

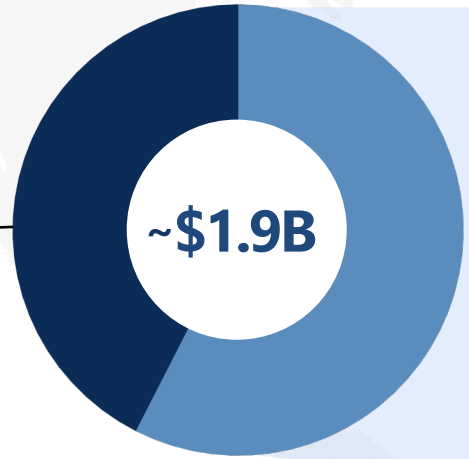
Galafold Long-Term Growth Opportunity

Galafold™ (migalastat hydrochloride)

In the next 5 years, the Fabry market is expected to surpass $2.5B with ~$1B estimated to have amenable mutations

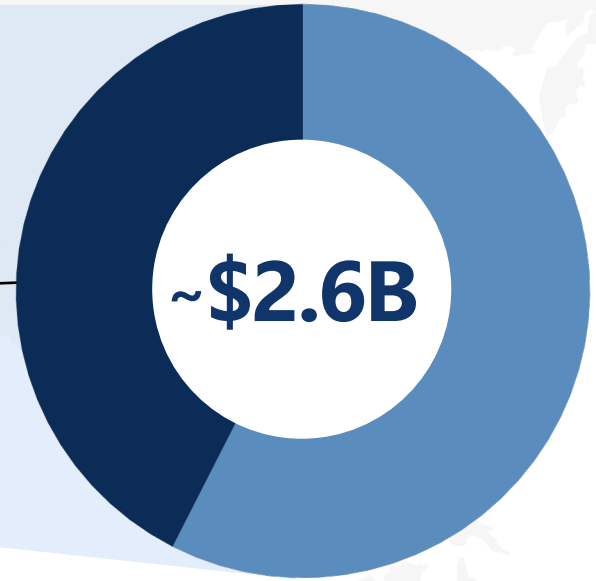
Fabry Market Today

Market Amenable to Galafold[1]



Estimated Fabry Market in 2027[2]

Market Amenable to Galafold: ~$1.1B[1]



Galafold has successfully switched 80%-90% of treated amenable patients in its most mature markets

[1]Assuming 35%-50% amenability [2]Assuming ~7% CAGR

Amicus Therapeutics

Amicus Therapeutics®

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

NBS studies shows higher incidence than medical literature suggests (~1:10,000 to ~1:30,000)

Age of onset ranges from infancy to adulthood

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

Deficiency of GAA leading to lysosomal glycogen accumulation and cellular dysfunction

Symptoms include muscle weakness, respiratory failure, and cardiomyopathy

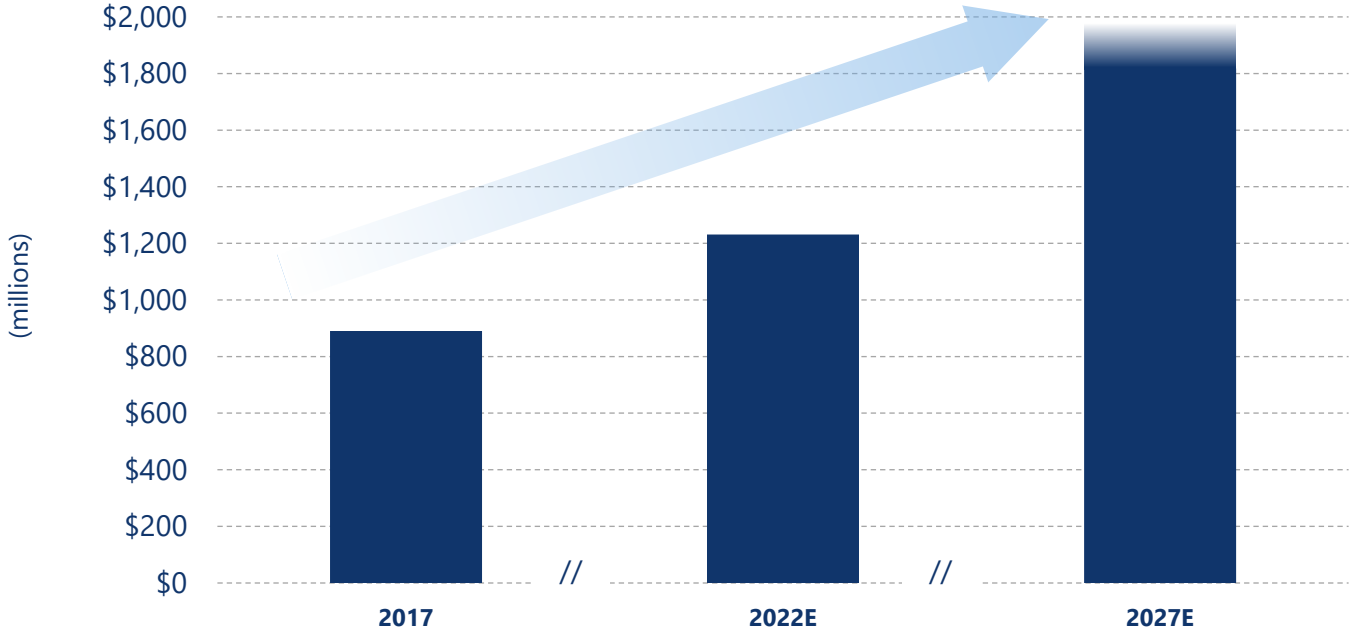
Respiratory and cardiac failure are leading causes of morbidity and mortality

NBS: Newborn Screening

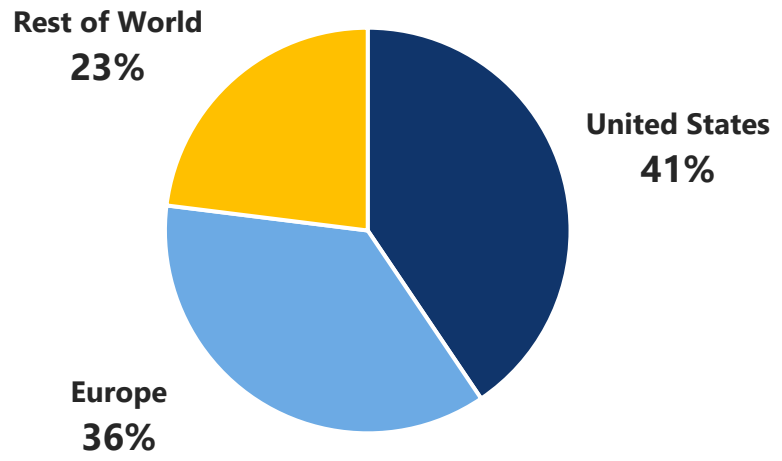
Global Pompe Market

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 2027[1]



Global Pompe Market Sales Split YTD 9M 2022[2]



- An estimated 3,500-4,000 Pompe patients globally are being treated by ERT[3]

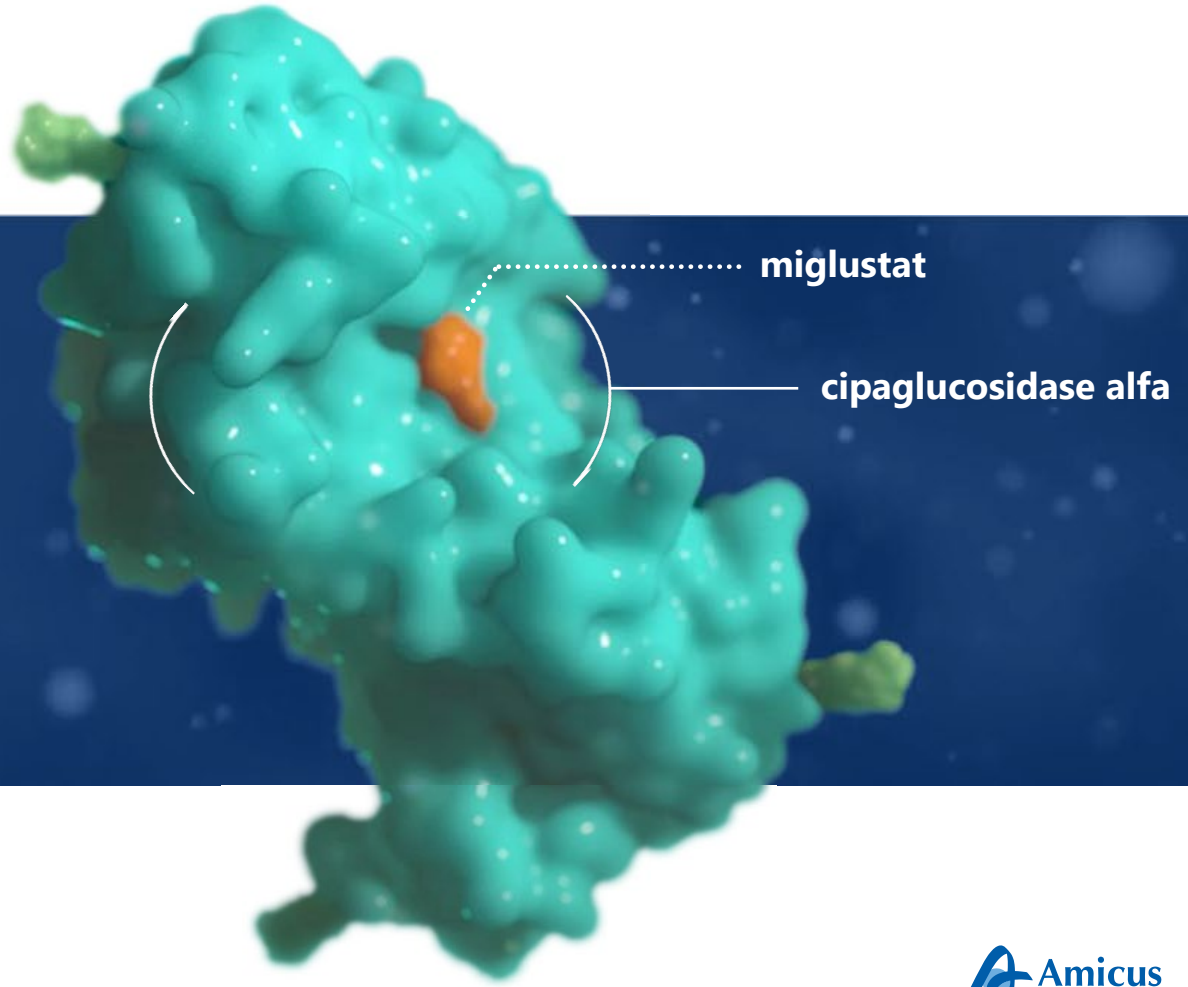
[1] Global market measured by reported sales of approved therapies for Pompe disease – 2027 sales projected using ~8% CAGR
[2] As reported YTD September 2022
[3] Amicus Data on File from Market Mapping

Amicus Therapeutics

AT-GAA: An Innovative Approach to Pompe Disease

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

- AT-GAA is a two-component therapy combining cipaglucosidase 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 glycogen[1]



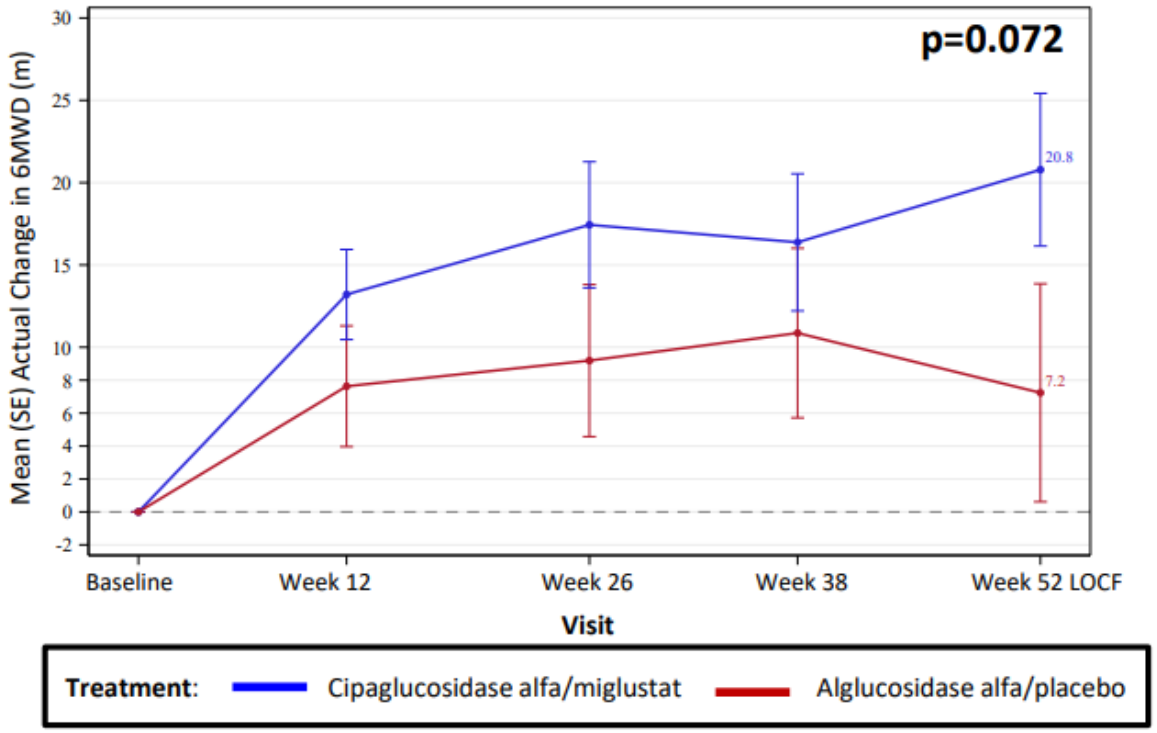
[1]Selvan et al. 2021, J Biol Chem 2021 Jan-Jun;296:100769
ERT: Enzyme Replacement Therapy

Amicus Therapeutics

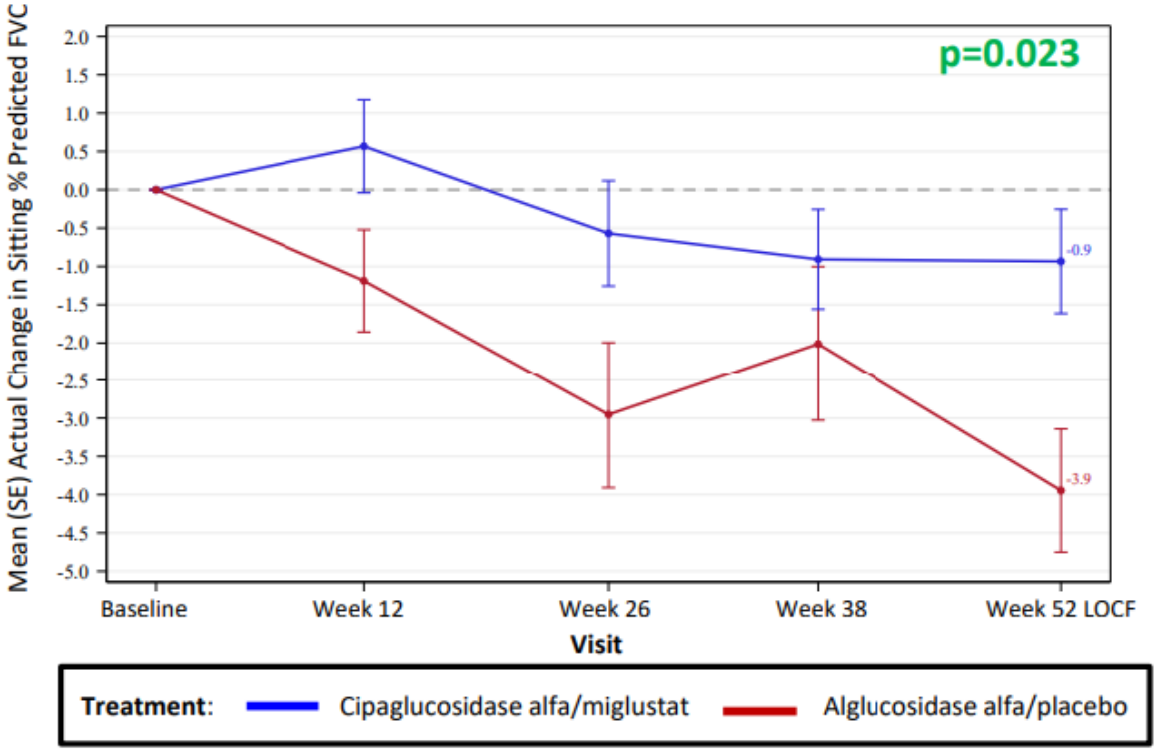
Phase 3 PROPEL Study Results:
Overall Population (n=122*)

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)



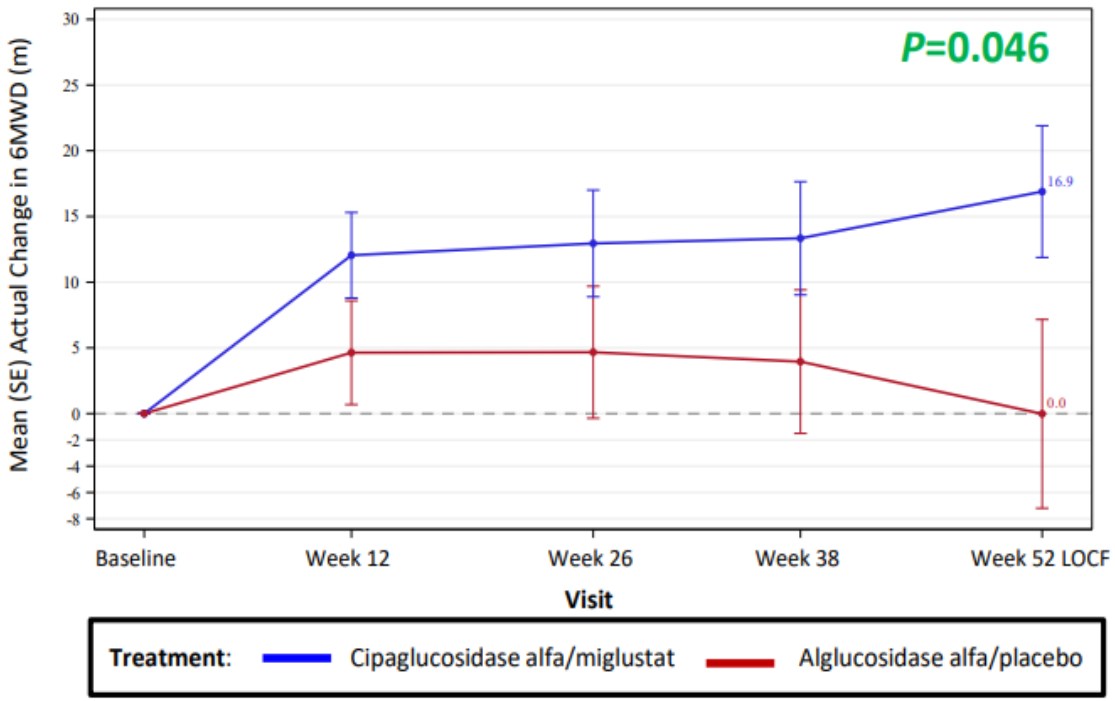
6MWD=6-minute walk distance;; FVC=forced vital capacity; SE=standard error. P values are nominal 2-sided; FVC data normally distributed and P value is from ANCOVA. 6MWD data not normally distributed and P value is for nonparametric ANCOVA; *Results exclude one outlier subject

Amicus Therapeutics

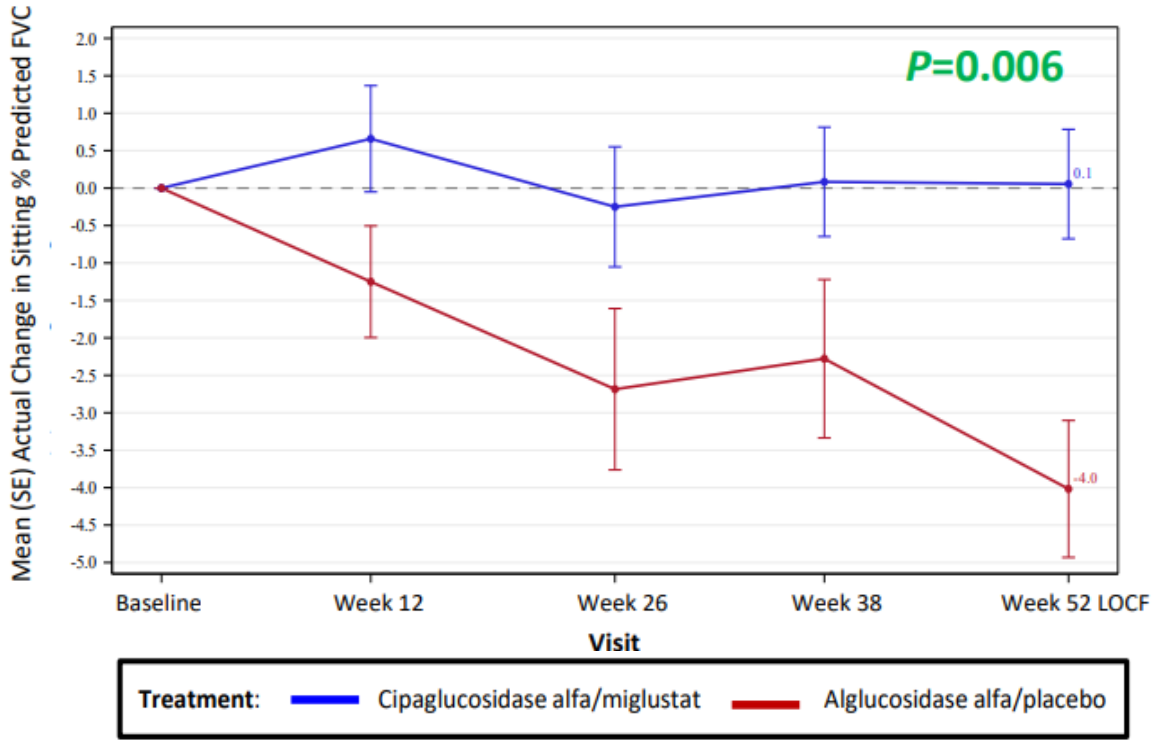
Phase 3 PROPEL Topline Results:
ERT Experienced Population (n=95)

ERT-experienced patients treated with AT-GAA demonstrated improvements over time in 6MWD and stabilization over time in FVC versus alglucosidase alfa

6MWD (m): Change from baseline (n=65, n=30)



FVC (% predicted): Change from baseline (n=65, n=30)



NOTE: Baseline is Mean (STDEV); CFBL is Mean (SE); P-values are nominal 2-sided; FVC data normally distributed and p–values are from ANCOVA
6MWD data not normally distributed and 6MWD p-value is for non-parametric ANCOVA; 6MWD parametric MMRM p-value was p=0.078

Amicus Therapeutics

AT-GAA: Global Regulatory Status

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

- Pombiliti™ (cipaglucosidase alfa) received a positive CHMP opinion in December 2022
- Miglustat CHMP opinion is expected in 2Q 2023

- PDUFA action deferred due to inability to conduct pre-approval manufacturing inspection[1]
- In discussion with the FDA to develop plans and logistics for inspection

- U.K. MAA submitted via recognition procedure based on CHMP opinion

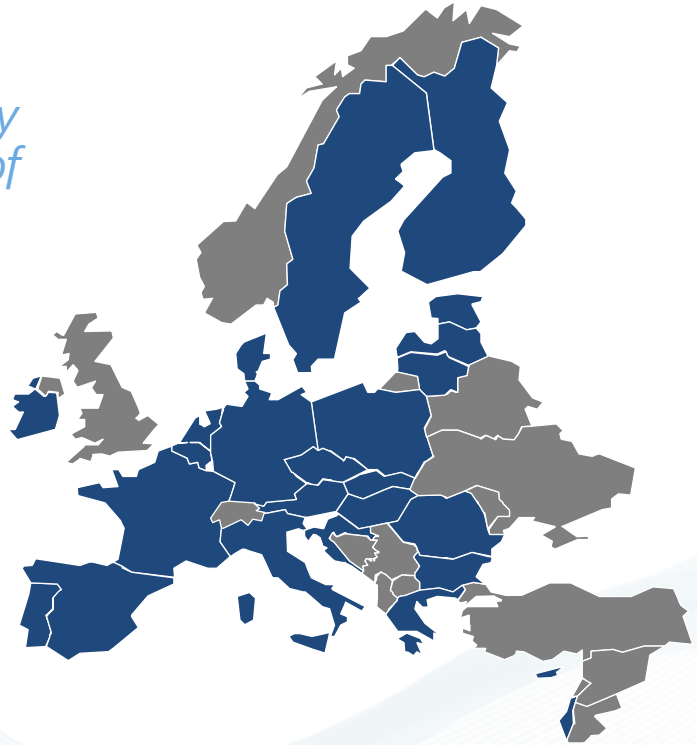
[1] FDA has not provided anticipated action dates as they continue to monitor the public health situation and travel restrictions in China; The Company expects the FDA to approve the NDA and BLA applications together

Amicus Therapeutics

AT-GAA: 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 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 Europe[1]
- Broad experience with AT-GAA from a wide set of KOLs through clinical trials and early access programs
- EU regulatory outcome and label to be leveraged in other ex-U.S. geographies

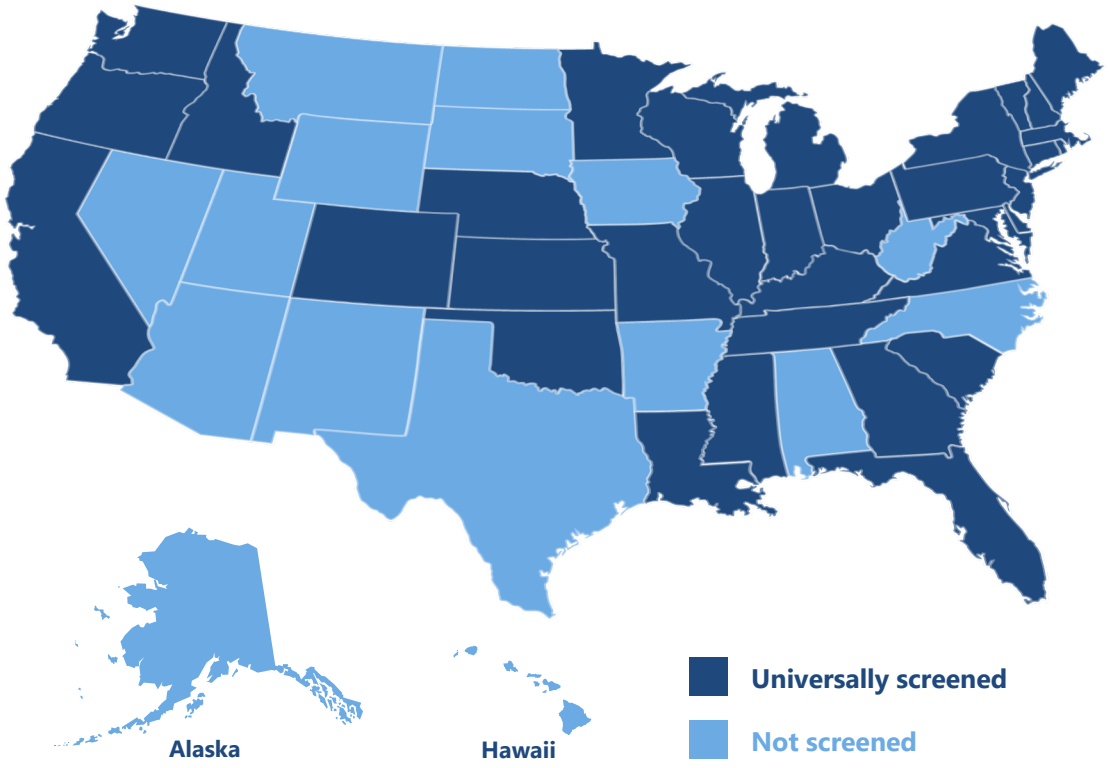


[1] Amicus Data on File from Market Mapping

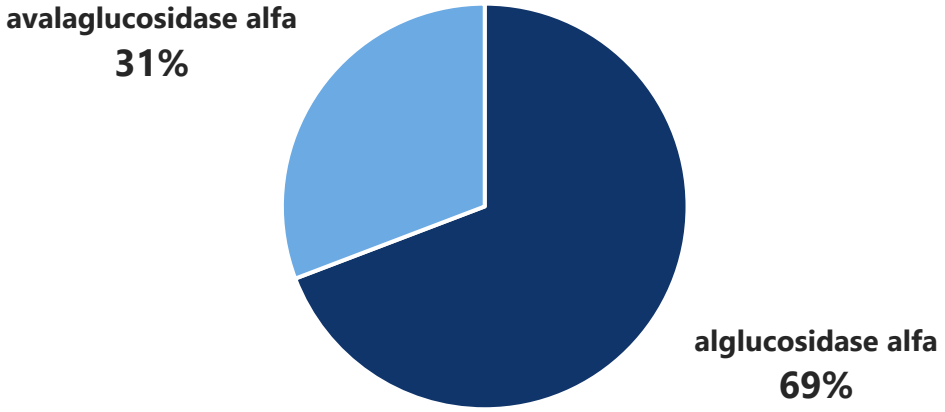
AT-GAA: U.S. Opportunity

U.S. Pompe market currently represents a sizeable market opportunity of >$500M

Pompe disease Newborn Screening in 34 U.S. States[1]



U.S. Pompe Market Sales Split YTD 9M 2022[2]



- >800 patients are estimated to be treated in the USA[3]

[1] https://www.newsteps.org/resources/data-visualizations/newborn-screening-status-all-disorders
[2] As reported in YTD September 2022
[3] Amicus Data on File from Market Mapping

AT-GAA: U.K. Opportunity

U.K. represents the third largest Pompe disease market

- U.K. submission via recognition procedure based on CHMP opinion
- Significant demand through EAMS underscores unmet need:
  - Dozens of patients on treatment today
  - All leading centers have requested access
  - Requests for additional patients being received every month
- >200 people with Pompe disease are estimated to be treated in the U.K.[1]

[1] Amicus Data on File from Market Mapping

AT-GAA: Ongoing Clinical Studies and Expanded Access Mechanisms

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

- Ongoing clinical studies in children and adolescents[1] 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
- ~190 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

[1] Children and adolescents aged 0 to <18 years old

Amicus Therapeutics

AT-GAA Launch Preparations

Experienced and passionate rare disease medical and commercial organization poised for second successful launch

Key Strengths

Team	Highly leverageable team in place, few new hires needed	Great experience and passion	Eagerness to introduce a new therapy upon approvals
Scientific Exchange	Published Phase 3 PROPEL data in *The Lancet Neurology*	Active medical conference and publication schedule	Continued education on biology of disease and diagnosis
Access	Commitment to patient access	Multiple expanded access programs in place	Demonstrating value to payors including parity pricing strategy
Planning	Clear focus on launch	Identification of key Pompe disease treatment centers	Development of educational materials

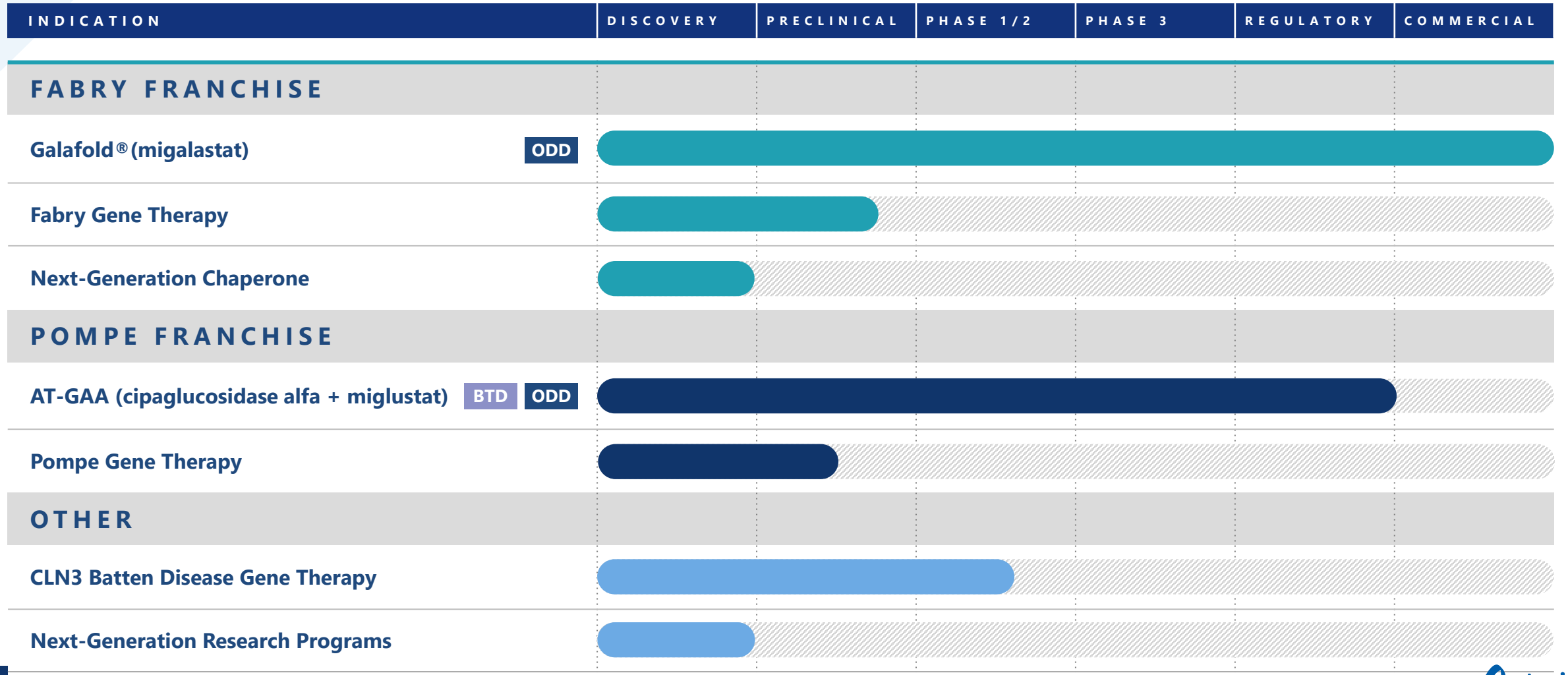
Amicus Therapeutics

Corporate Outlook

Delivering on our mission for patients and shareholders

Amicus Pipeline

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



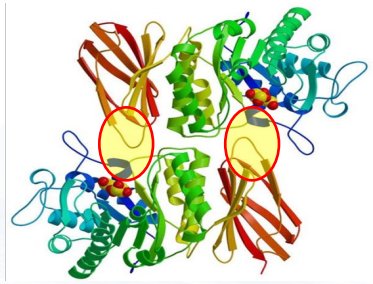
ODD - Orphan Drug Designation **BTD** - Breakthrough Therapy Designation

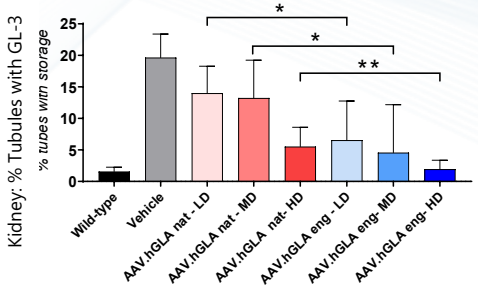
Amicus Therapeutics

Expanding Our Leadership Position in Fabry and Pompe

Differentiated gene therapy approach for greater potency and optimized cross correction through transgene engineering for stability and targeting

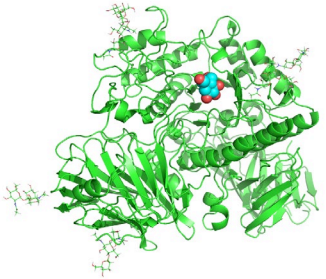
Fabry Gene Therapy

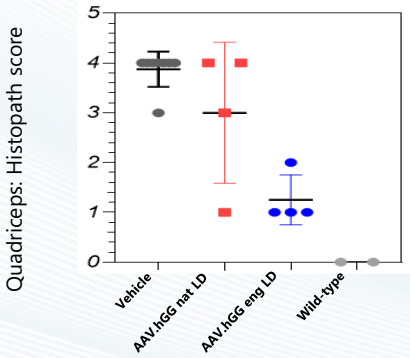




- Proprietary pantropic AAV capsid and ubiquitous promoter
- Engineered hGLA transgene for improved stability and optimized cross correction
- Preclinical data demonstrate robust substrate reduction across all Fabry disease relevant tissues
- Preclinical and manufacturing work underway

Pompe Gene Therapy





- Proprietary pantropic AAV capsid and ubiquitous promoter
- Engineered hGAA transgene for improved uptake and optimized cross correction
- Preclinical data demonstrate robust glycogen reduction in all key Pompe disease relevant tissues
- Preclinical and manufacturing work underway

Amicus Therapeutics

2023 Key Milestones

Galafold: Fabry Disease

- 2023 revenue growth of 12-17% at CER
- First data published from followMe Registry

AT-GAA: Pompe Disease

- Regulatory approvals in multiple key geographies
- Initial sales of AT-GAA in 2H2023
- Long-term data from Phase 3 PROPEL study at WORLD*Symposium*

Financial Strength

- Non-GAAP profitability[1] expected in 2H2023

[1]Based on projections of Amicus non-GAAP Net Income under current operating plans, which includes successful AT-GAA regulatory approvals and continued Galafold growth. We define non-GAAP Net Income as GAAP Net Income excluding the impact of share-based compensation expense, changes in fair value of contingent consideration, depreciation and amortization, acquisition related income (expense), loss on extinguishment of debt, loss on impairment of assets, restructuring charges, and income taxes.

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

>350 Patients*

>2,000 Patients*

Thousands of Patients*

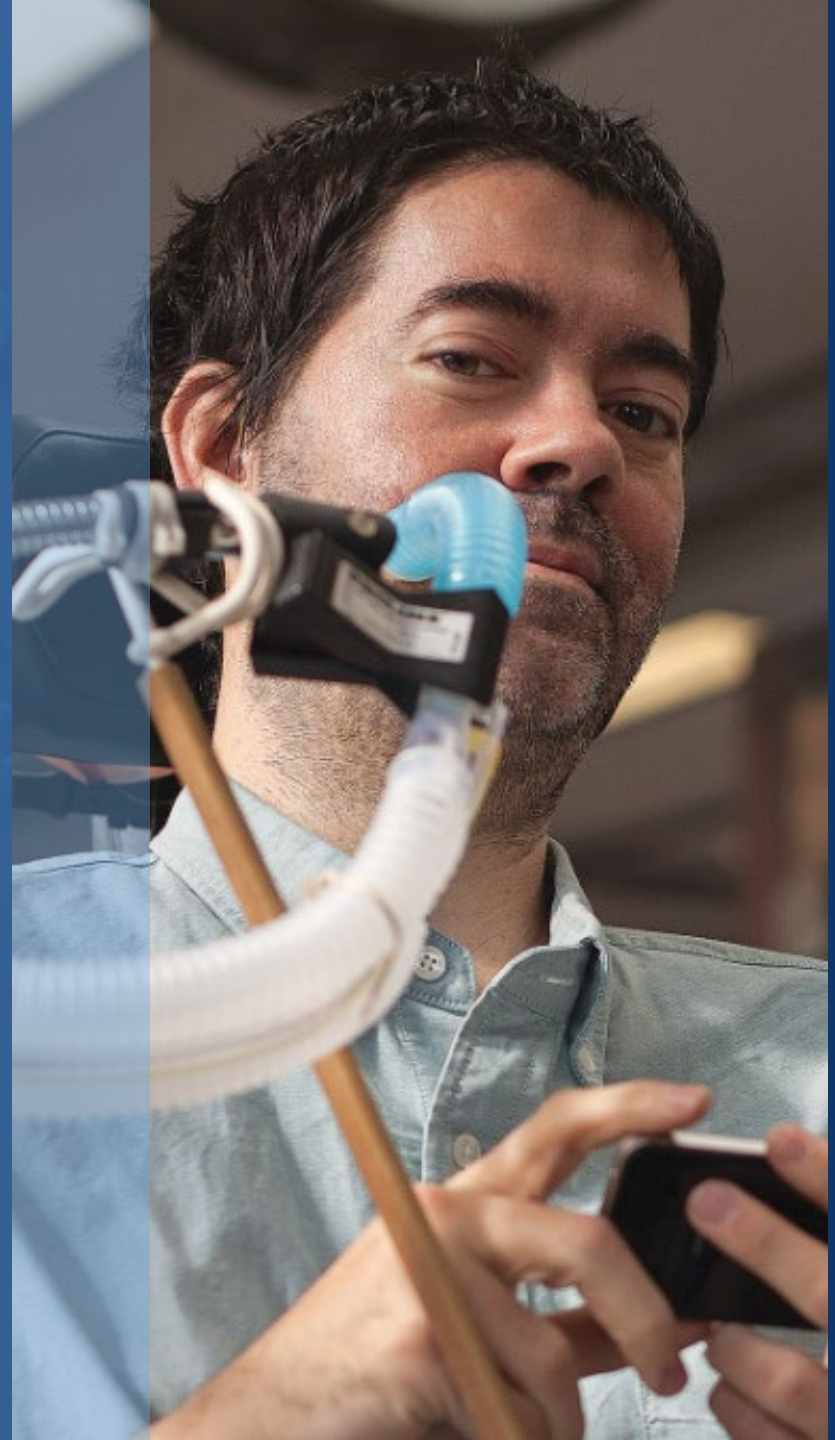
YE17 → YE22 → 2023+

*Clinical & commercial, all figures approximate

Amicus Therapeutics

Amicus Therapeutics®

Thank You



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)

Expanded Access through Jan 2023:
74 patients / **20** countries

Contributions allocated:
$1,677,000 U.S.
$832,976 Intl.

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
496

% female employees
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**.

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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%

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

		Overall population				ERT-experienced			
	Endpoints	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 ($P<0.05$)

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