



1Q21 Financial Results Conference Call & Webcast

May 10, 2021



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, and in particular the potential goals, progress, timing, and results of preclinical studies and clinical trials, and revenue goals, including as they are impacted by COVID-19 related disruption, are based on current information. The potential impact on operations and/or revenue 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 or to treatment sites. 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, UK, 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, commercialization and manufacturing. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. With respect to statements regarding corporate financial guidance and financial goals and the attainment of such goals and 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, 2020 and on Form 10-Q for the quarter ended March 31, 2021, to be filed today. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, and we undertake no obligation to revise or update this news release to reflect events or circumstances after the date hereof.

Non-GAAP Financial Measures

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

A RARE COMPANY

A leading fully integrated, global rare disease biotechnology company



First Oral Precision Medicine for Fabry Disease



Gene Therapy PLATFORM
Protein Engineering & Glycobiology



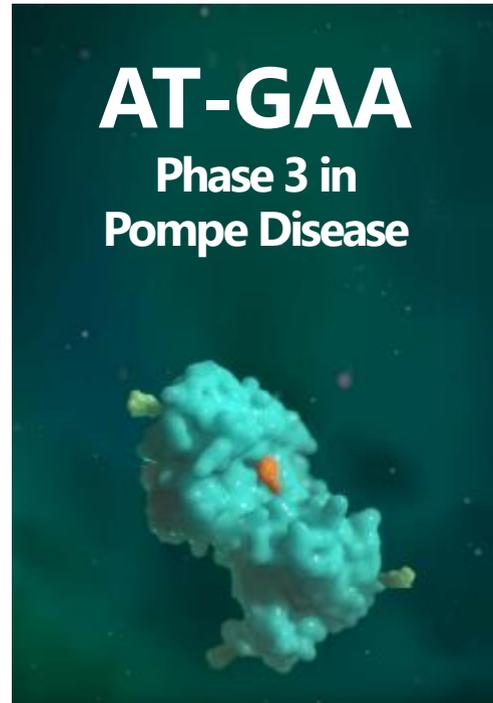
World Class **BIOLOGICS** Capabilities



EMPLOYEES in 27 Countries



AT-GAA
Phase 3 in Pompe Disease



\$417.4M Cash as of 3/31/21

Two Clinical-Stage Gene Therapies



GLOBAL COMMERCIAL ORGANIZATION



Robust R&D Engine

Nearly 50+ Lysosomal Disorders and More Prevalent Rare Diseases



2021 Key Strategic Priorities

- 1** **Achieve double-digit Galafold growth and revenue of \$300M to \$315M**
- 2** **Report data from the AT-GAA Phase 3 PROPEL study and complete BLA and MAA filings for regulatory approvals**
- 3** **Advance clinical studies, regulatory discussions and scientific data across industry leading gene therapy pipeline**
- 4** **Further manufacturing capabilities and capacity to build world-class technical operations to support all gene therapy programs**
- 5** **Maintain strong financial position**



Galafold[®] (migalastat) Global Launch...

...taking a leadership role in the
treatment of Fabry disease

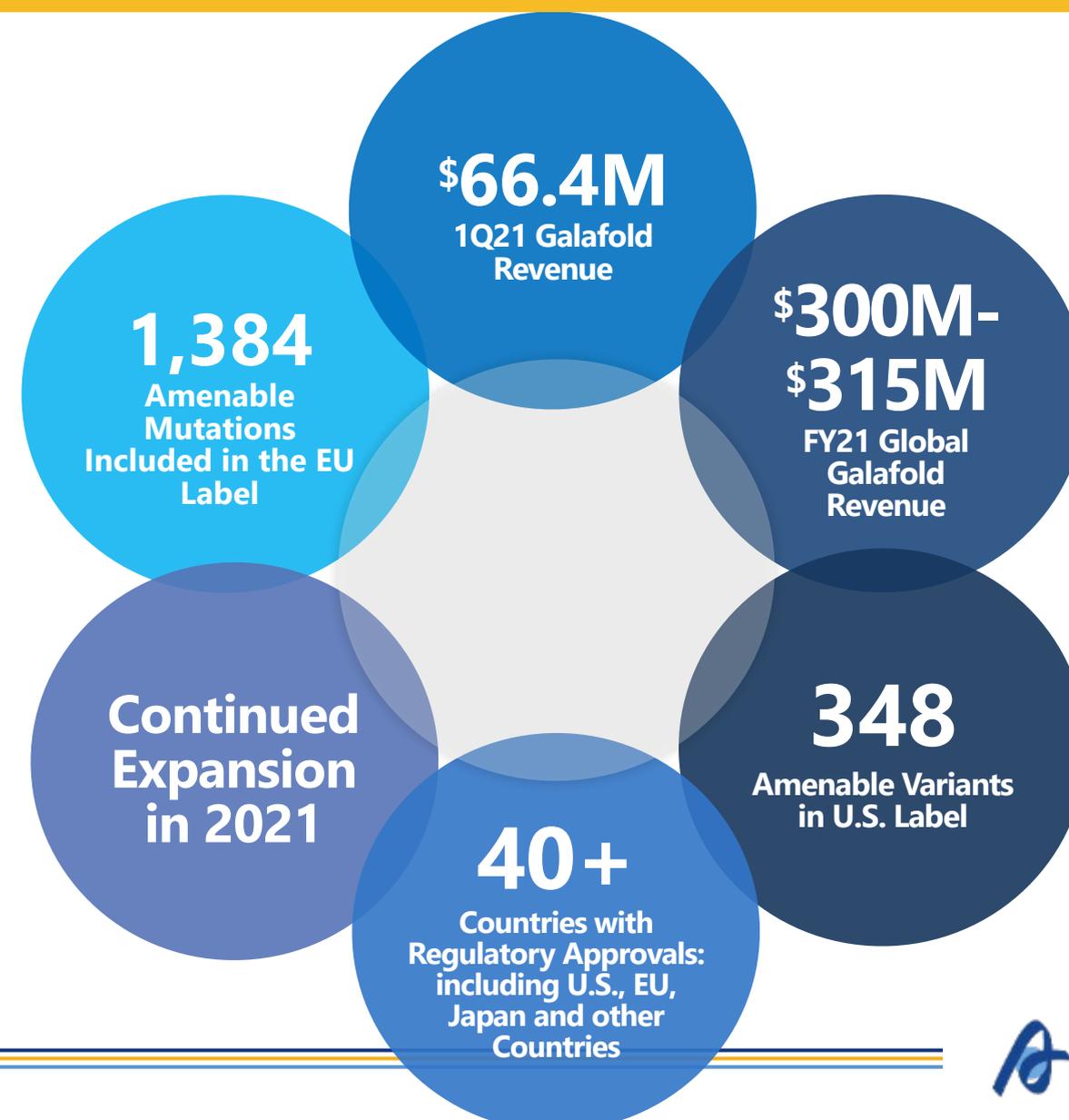
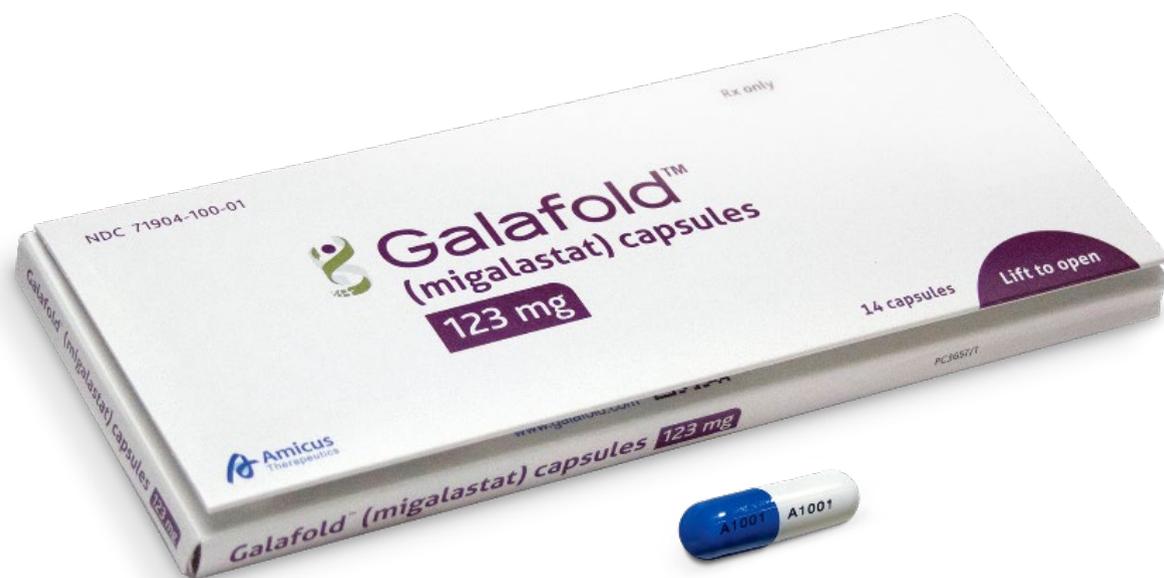
“We push ideas as far and as fast as possible”

- Amicus Belief Statement

Galafold Snapshot (as of March 31, 2021)

Galafold is an orally delivered small molecule precision medicine with a unique mechanism of action for Fabry patients with amenable variants that replaces the need for intravenously delivered ERT

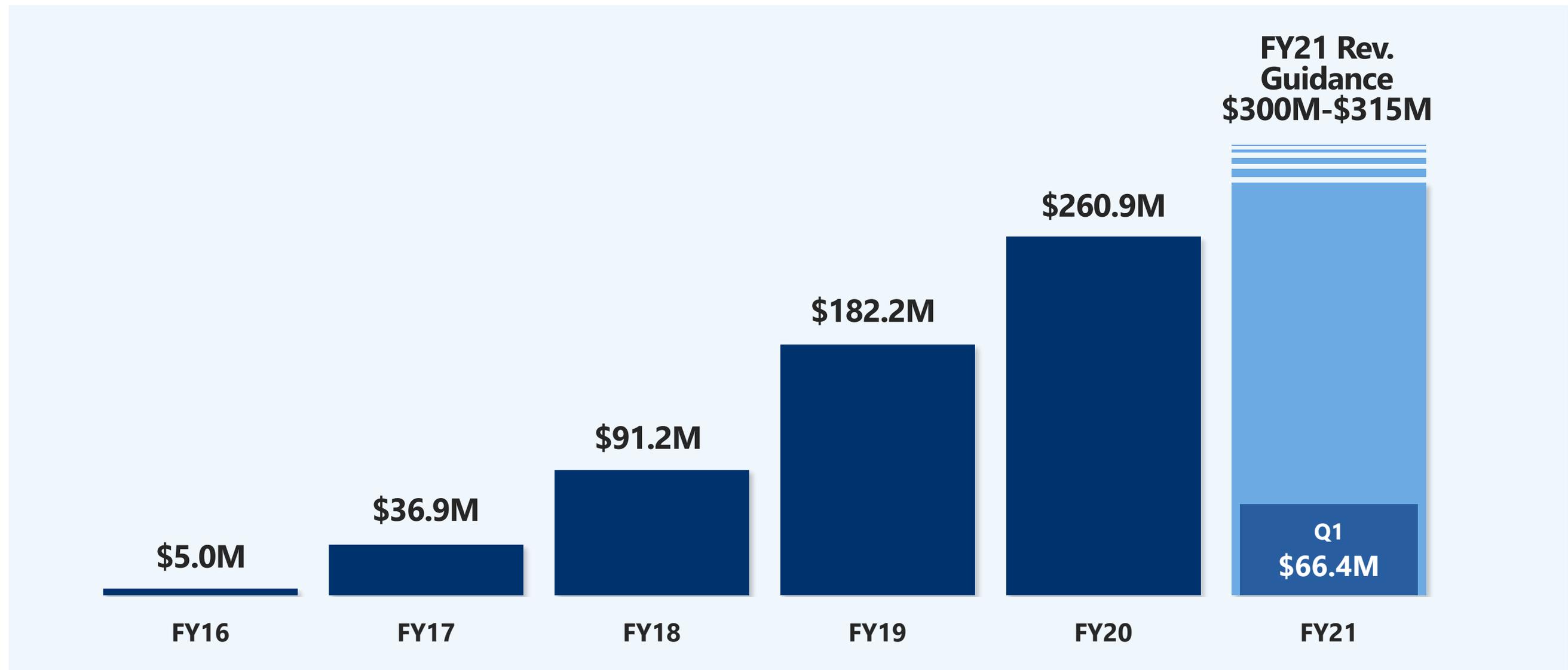
One of the Most Successful Rare Disease Launches



Galafold is indicated for adults with a confirmed diagnosis of Fabry Disease and an amenable mutation/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 Success and FY21 Revenue Guidance

Galafold momentum remains on track to achieve full year 2021 revenue guidance



Outlook for 2021

Continued double-digit Galafold revenue growth to \$300M-\$315M in 2021

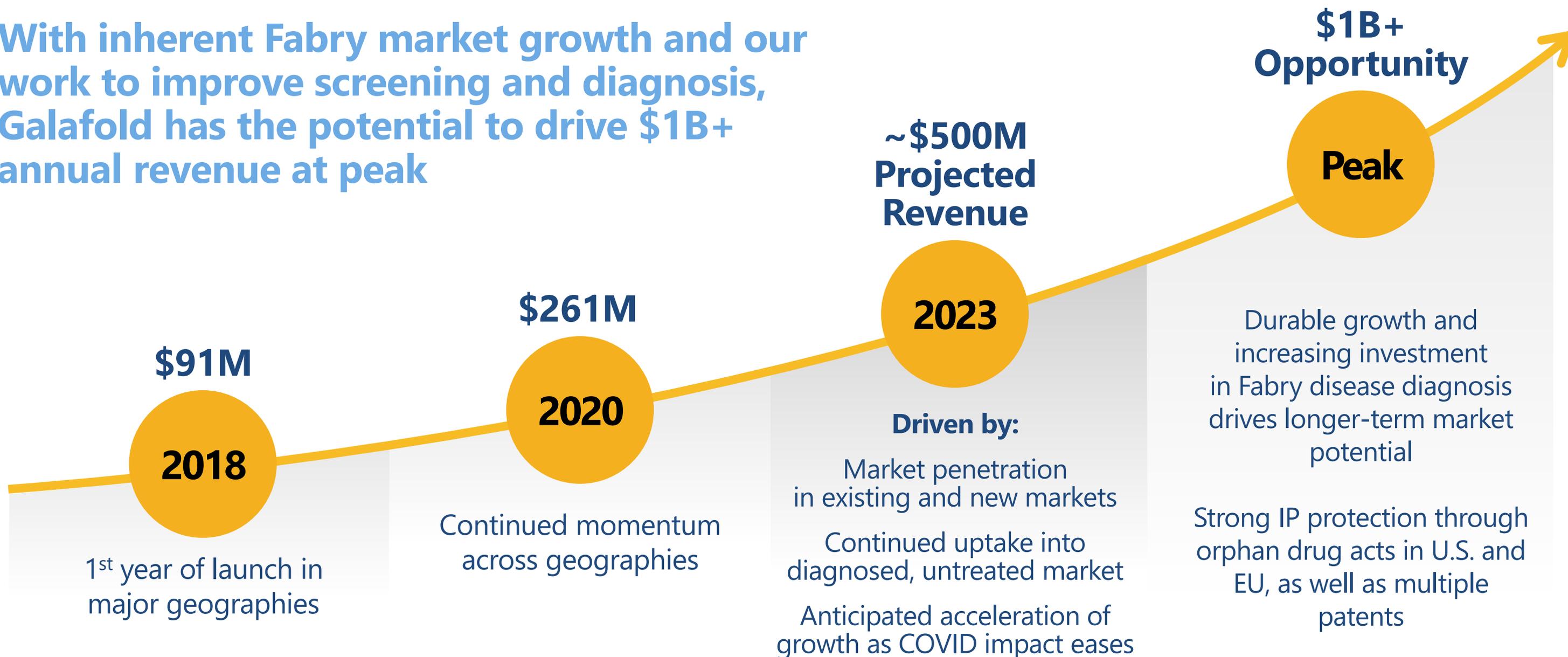


Galafold Continues
Strong Launch
Performance &
Cornerstone of
Amicus Success

- Global demand remains strong with continued growth anticipated in 2021 and beyond
- Number of patients on Galafold were ahead of the Company's internal expectations at end of Q1
- In 2021, project double-digit revenue growth with net new patient starts expected to be greater than in 2020
- COVID continues to impact time between patient identification and treatment initiation
- Expect higher patient adds and revenue growth in the second half of 2021 as COVID impact eases
- Continue to see >90% compliance and adherence rates globally

Galafold Opportunity

With inherent Fabry market growth and our work to improve screening and diagnosis, Galafold has the potential to drive \$1B+ annual revenue at peak





AT-GAA: Next Potential Standard of Care for Pompe Disease

“We encourage and embrace constant innovation”

- Amicus Belief Statement

Pompe Disease Overview

Pompe disease is a severe and fatal neuromuscular disease and one of the most prevalent lysosomal disorders with very high unmet medical need



5,000 – 10,000+ patients diagnosed WW¹; newborn screening suggests underdiagnosis

Age of onset ranges from infancy to adulthood

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 glycogen accumulation and cellular dysfunction

Symptoms include muscle weakness, respiratory failure and cardiomyopathy

~\$1B+ global Pompe ERT sales²

Phase 3 PROPEL Study

Primary, Key Secondary and Biomarker Endpoint Heat Map

Endpoints across motor function, pulmonary function, muscle strength, PROs and biomarkers favored AT-GAA over alglucosidase alfa in both the overall and ERT experienced populations

Overall Population

	Alglucosidase alfa	AT-GAA
Motor Function		6MWD
		GSGC*
Pulmonary Function		FVC*
Muscle Strength		Lower MMT
PROs		PROMIS-Physical
		PROMIS-Fatigue
Biomarker		Hex4*
		CK*

ERT Experienced Population

	Alglucosidase alfa	AT-GAA
Motor Function		6MWD*
		GSGC*
Pulmonary Function		FVC*
Muscle Strength		Lower MMT
PROs		PROMIS-Physical
		PROMIS-Fatigue
Biomarker		Hex4*
		CK*

AT-GAA: Next Steps



AT-GAA for Pompe
Advances Toward
Approval

- Rolling BLA submission expected to complete in 2Q
- Other key regulatory submissions for approval throughout 2021 including MAA in Europe
- Potential for early approval under EAMS framework with Priority Innovative Medicines Designation in U.K.
- 150+ patients worldwide now being treated with AT-GAA including adults, adolescents and infants
- Pediatric study for Pompe patients aged 12 to <18 with late-onset Pompe disease ongoing
- Clinical study for Pompe patients with infantile-onset disease expected to begin this year
- Expanded access program for Pompe infantile patients and adult-onset patients open and has enrolled multiple patients with Pompe. Further expanded access for all Pompe patients being considered.



Next Generation Gene Therapy Platform

“We have a duty to obsolete our own technologies”

- Amicus Belief Statement

A RARE PORTFOLIO

	DISCOVERY	PRECLINICAL	PHASE 1/2	PHASE 3	REGULATORY	COMMERCIAL
Fabry Franchise						
Galafold® (migalastat) Monotherapy ODD						
Fabry Gene Therapy	PENN					
Pompe Franchise						
AT-GAA (Novel ERT + Enzyme Stabilizer) ODD BTD						
Pompe Gene Therapy	PENN					
Batten Franchise – Gene Therapies						
CLN6 Batten Disease ODD RPD PRIME	NCH					
CLN3 Batten Disease ODD RPD	NCH					
CLN1 Batten Disease	PENN					
Next Generation Research Programs and CNS Gene Therapies						
CDKL5 Deficiency Disorder GTx / ERT	PENN					
Angelman Syndrome	PENN					
Others	NCH / PENN					
MPS Franchise						
Mepsevii™ (vestronidase alfa) <i>(Japan Only)*</i>						
Next Generation MPSIIIA	PENN					
MPSIIIB	PENN					

LEGEND

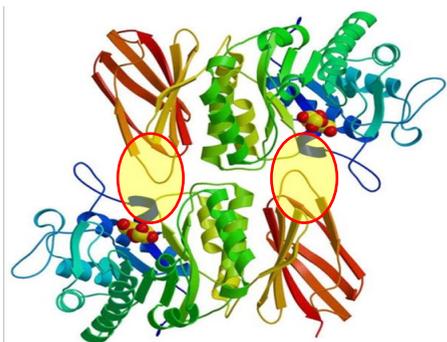
- **ODD** - Orphan Drug Designation
- **RPD** - Rare Pediatric Disease Designation
- **PRIME** - Priority Medicines Designation
- **BTD** - Breakthrough Therapy Designation

*Exclusive license from Ultragenyx for Japanese rights to Mepsevii™, investigator-sponsored trial in Japan underway

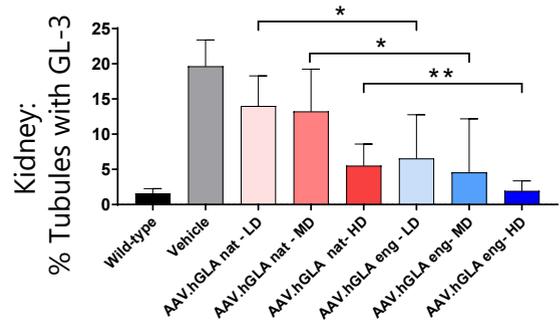
Amicus Protein Engineering Expertise & Technologies for Gene Therapy

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

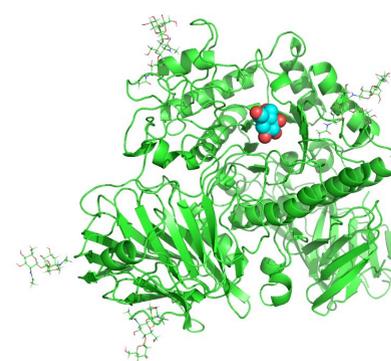
Fabry Gene Therapy



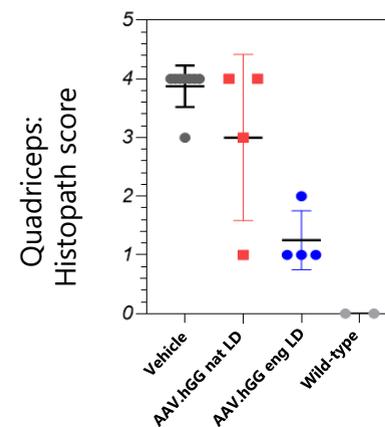
- Proprietary AAV capsid
- Pantropic capsid and ubiquitous promoter
- Engineered hGAL transgene at dimer interface designed for improved stability and optimized cross correction
- Preclinical data demonstrate robust substrate reduction across all Fabry disease relevant tissues, including first evidence of dorsal root ganglia storage reduction



Pompe Gene Therapy



- Proprietary AAV capsid
- Pantropic capsid and ubiquitous promoter
- Engineered hGAA transgene with cell receptor binding motif designed for improved uptake and optimized cross correction
- Preclinical data demonstrate robust glycogen reduction in all key Pompe disease tissues, including reduction in neurons of central nervous system





Financial Summary

“We are business led and science driven”
- Amicus Belief Statement

1Q2021 Select Financial Results

1Q2021 revenue of \$66.4M primarily from global Galafold sales

<i>(in thousands, except per share data)</i>	Mar. 31, 2021	Mar. 31, 2020
Product Revenue	\$66,402	\$60,525
Cost of Goods Sold	6,539	6,552
R&D Expense	64,117	89,120
SG&A Expense	46,726	40,215
Changes in Fair Value of Contingent Consideration	471	931
Depreciation and Amortization	1,604	1,764
Loss from Operations	(53,055)	(78,057)
Income Tax Expense	(1,582)	(361)
Net Loss	(65,664)	(88,948)
Net Loss Per Share	(0.25)	(0.35)

Financial Outlook: Key Takeaways

- Reaffirming full-year Galafold revenue guidance of \$300 million to \$315 million
- Non-GAAP operating expense guidance for 2021 is expected to remain flat at \$410 million to \$420 million
 - Driven by disciplined expense management and continued investment in the global Galafold launch, AT-GAA clinical studies and pre-launch activities and advancing our gene therapy pipeline
- Current cash position is sufficient to achieve self-sustainability without the need for any future dilutive financing



Closing Remarks

“We believe in our future to build long-term value for our stakeholders”

- Amicus Belief Statement

Thank You

"Our passion for making a difference unites us"

-Amicus Belief Statement



Appendix

Reconciliation

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

	Three Months Ended	
	March 31,	
	2021	2020
Total operating expenses - as reported GAAP	\$ 112,918	\$ 132,030
Research and development:		
Share-based compensation	6,305	5,253
Selling, general and administrative:		
Share-based compensation	14,049	7,343
Changes in fair value of contingent consideration payable	471	931
Depreciation and amortization	1,604	1,764
Total operating expense adjustments to reported GAAP	22,429	15,291
Total operating expenses - as adjusted	\$ 90,489	\$ 116,739