

**UNITED STATES
SECURITIES AND EXCHANGE COMMISSION**

WASHINGTON, D.C. 20549

FORM 8-K/A

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

Date of Report (Date of earliest event reported): **July 8, 2019**



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.)

1 Cedar Brook Drive, Cranbury, NJ 08512
(Address of Principal Executive Offices, and Zip Code)

609-662-2000
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, \$0.01 Par Value	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 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

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. ☐

Explanatory Note.

This Form 8-K/A is being filed solely to amend the Current Report on Form 8-K filed by Amicus Therapeutics, Inc. (the “Company”) on July 8, 2019 to correct a rendering error in Exhibit 99.1 attached thereto.

Item 8.01. Other Events.

On July 8, 2019, the Company updated and released presentation materials it plans to use in meetings with investors and analysts. A copy of this presentation is attached hereto as Exhibit 99.1 and is incorporated herein by reference.

Item 9.01 Financial Statements and Exhibits.**Exhibits:**

<u>Exhibit No.</u>	<u>Description</u>
99.1	<u>Investor Presentation - July 2019</u>

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: July 8, 2019

By: /s/ Ellen S. Rosenberg
Name: Ellen S. Rosenberg
Title: Chief Legal Officer and Corporate Secretary



General Corporate & Gene Therapy Overview:

At the Forefront of the Human Genome Medicine Revolution

July 2019



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, actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in our business, including, without limitation: the potential that results of clinical or preclinical studies indicate that the product candidates are unsafe or ineffective; the potential that it may be difficult to enroll patients in our clinical trials; the potential that regulatory authorities, including the FDA, EMA, and PMDA, may not grant or may delay approval for our product candidates; the potential that we may not be successful in commercializing Galafold in Europe, Japan, the US and other geographies or our other product candidates if and when approved; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; the potential that we may not be able to manufacture or supply sufficient clinical or commercial products; and the potential that we will need additional funding to complete all of our studies 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 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, 2018. 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.

A RARE COMPANY.

Amicus is one of the world's leading fully-integrated, global rare disease biotechnology companies

Galafold™
(migalastat)

First Oral Precision
Medicine for Fabry Disease



Gene Therapy
PLATFORM

Protein Engineering
& Glycobiology



World Class
Biologics
Capabilities

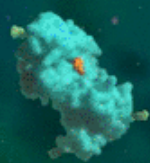


600+
EMPLOYEES
in 27 Countries



AT-GAA

Phase 3 in
Pompe Disease



GLOBAL
COMMERCIAL
ORGANIZATION

~\$580M+
Cash
as of 6/30/19
(Proforma)

**Global Drug
Development**



**Robust R&D
Engine**

Nearly 50+ Lysosomal
Disorders and More
Prevalent Rare Diseases



A RARE OPPORTUNITY.

With a recently launched, highly successful commercial precision medicine for Fabry disease, a late stage novel Enzyme Replacement Therapy with Breakthrough Therapy Designation in Phase 3 studies for Pompe disease (AT-GAA) and now the largest number of Gene Therapy programs for rare diseases in the industry

Galafold

Precision Medicine
for Fabry Disease

Pompe Biologic

Phase 3 with
Breakthrough Therapy
Designation

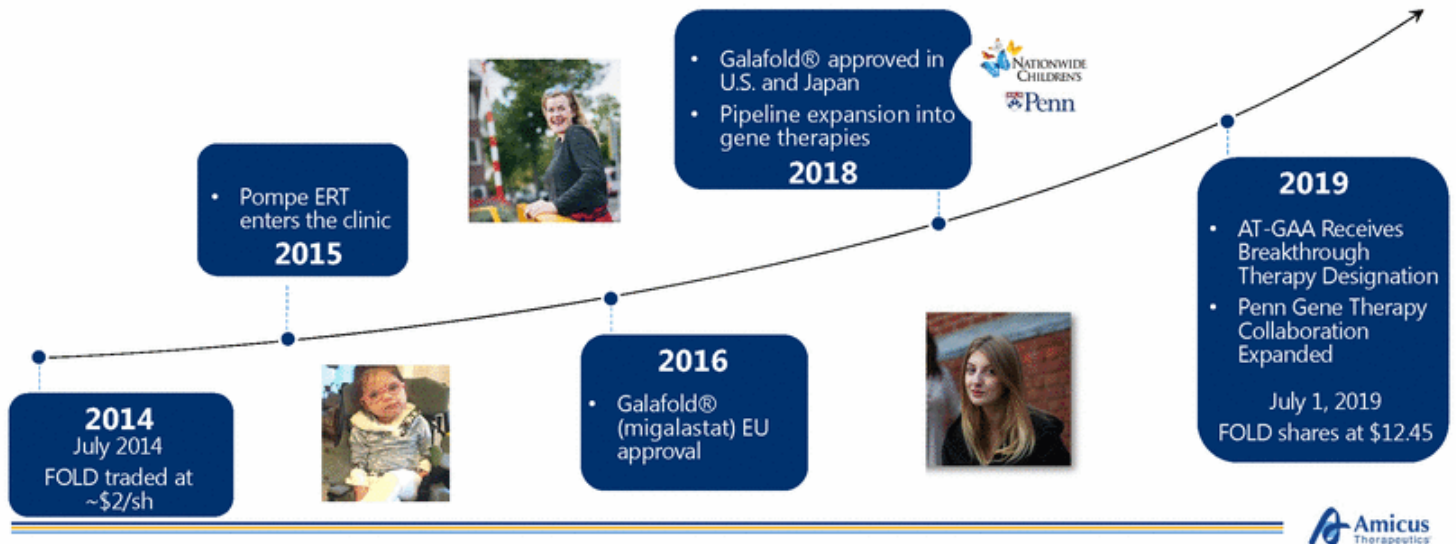
Gene Therapy Portfolio

Industry's Largest for
Rare Disease

*Amicus has built a deep and highly distinct **"Rare Portfolio"** of products, technologies and programs*

Creating Shareholder Value

While Amicus shares have appreciated more than ~600% over the past five years, there is now an opportunity for further and substantial shareholder value creation





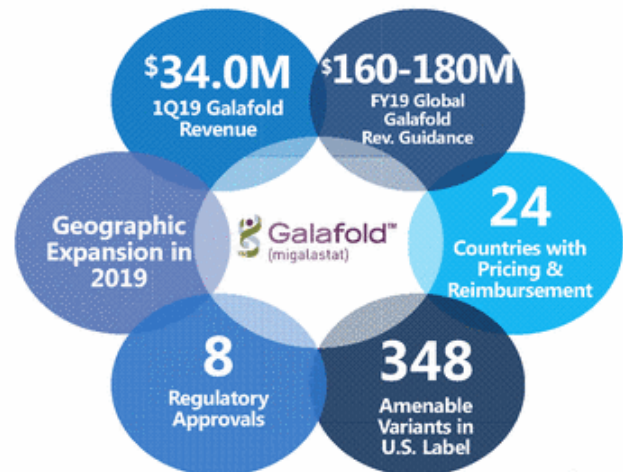
Galafold[®] (migalastat) Global Launch...

...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, 2019)

Galafold is the cornerstone of Amicus' success. It 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 enzyme replacement therapy



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 (>10%) were headache, nasopharyngitis, urinary tract infection, dizziness and pyrexia. For additional information about Galafold, including the full U.S. Prescribing Information, please visit <http://www.amicustherapeutics.com/galafold>. For further important safety information for Galafold, including dosing and method of administration, special warnings, drug interactions and adverse drug reactions, please see the language (s) for Galafold available from the FDA website of www.fda.gov/oc/ohrt.

Galafold Global Launch Momentum (as of March 31, 2019)

Galafold is approved and fully reimbursed in the United States, the EU, Japan and most major countries. It is the one of the most successful new Lysosomal Disorder product launch ever.

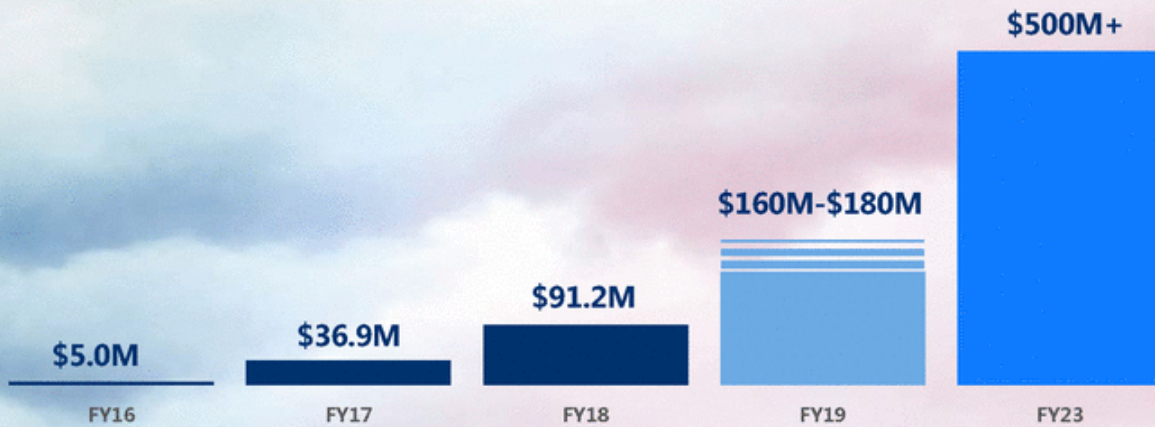
- **Global:** 150+ new patient adds in 1Q19 with continued >90% compliance and adherence. Now estimate ~18% global market share of treated amenable patients*
- **U.S:** 200+ prescription referral forms (PRFs) from 90+ prescribers (as of April 30); shortening time from PRF to shipment
- **International:** strong growth from both switch and previously untreated patients
- **Japan:** Q1 patients ahead of forecast with expanded commercial team
- **Demographics:** balanced mix of males and females, classic and late-onset patients across all markets



*Market share based on reported 2018 global Fabry sales and assumes a 35% amenability rate for Galafold.

Galafold Success and FY19 Galafold Revenue Guidance

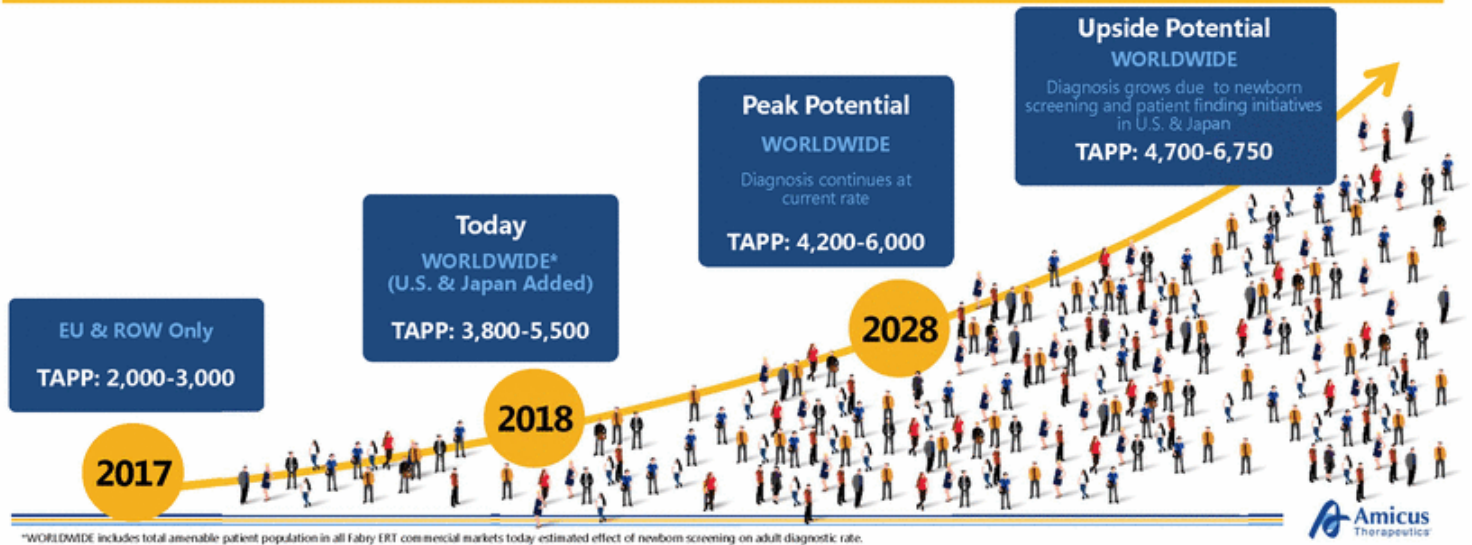
Galafold sales in 2019 represent the first full year of launch in the major geographies in the world and will be between \$160-\$180M, with more than 1,000 Fabry patients on therapy by the end of 2019. Galafold sales of \$500M+ are expected by 2023.



Total Amenable Patient Population ("TAPP")

Estimate based on 35% - 50% amenability

Fabry disease is increasingly believed to be one of the most prevalent human genetic diseases. The total amenable patient population ("TAPP") has the potential to make Galafold a \$1B+ annual product at peak.





AT-GAA Novel ERT for Pompe Disease

"We encourage and embrace constant innovation"
- Amicus Belief Statement

Pompe Disease Overview

Pompe disease is a severe and fatal muscular dystrophy 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

Respiratory and cardiac failure are leading causes of morbidity and mortality

Age of onset ranges from infancy to adulthood

Deficiency of GAA leading to glycogen accumulation and cellular dysfunction

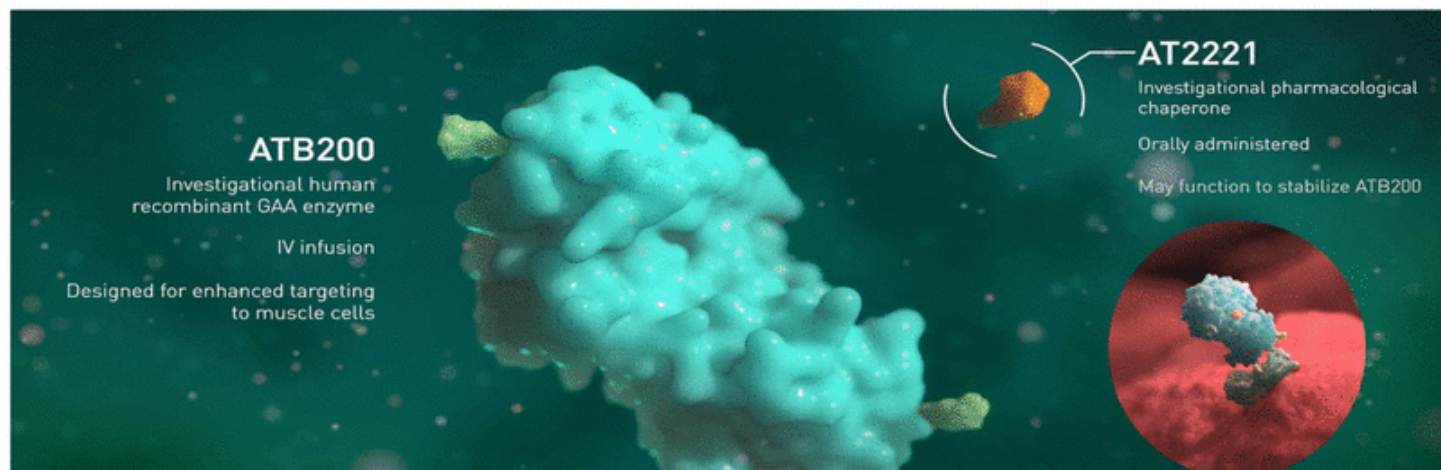
Symptoms include muscle weakness, respiratory failure, and cardiomyopathy

~\$900M+ Global Pompe ERT sales in FY18²

1. National Institute of Neurological Disorders and Stroke (NIH). 2. Sanofi Press Release & 10-K

AT-GAA: Foundation in Protein Engineering

Amicus scientists specializing in protein engineering and glycobiology created a uniquely glycosylated and highly phosphorylated ERT (AT-GAA) that significantly enhances targeting to key muscles affected in patients.



Pompe Patient Experience in Phase 1/2 Clinical Study (ATB200-02)

Phase 1/2 results showed strong and durable effects in patients out to two years, leading to dramatic improvements in muscle strength and function, as well as significant improvements in key biomarkers of disease

6-Minute Walk Test (m)

Cohort	Baseline (n=10)	Change at Month 24 ^{a,b} (n=8) Mean (SD)
Cohort 1 ERT-Switch Ambulatory	397.2 (96.8)	+53.6 (36.4)
Cohort	Baseline (n=5)	Change at Month 21 (n=5) Mean (SD)
Cohort 3 ERT-Naïve	399.5 (83.5)	+54.8 (34.7)

FVC (% Predicted)

Cohort	Baseline (n=9*)	Change at Month 24 ^{a,b,c} (n=7) Mean (SD)
Cohort 1 ERT-Switch Ambulatory*	52.6 (14.7)	-0.6 (2.8)
Cohort	Baseline (n=5)	Change at Month 21 (n=5) Mean (SD)
Cohort 3 ERT-Naïve	53.4 (20.3)	+6.1 (9.7)

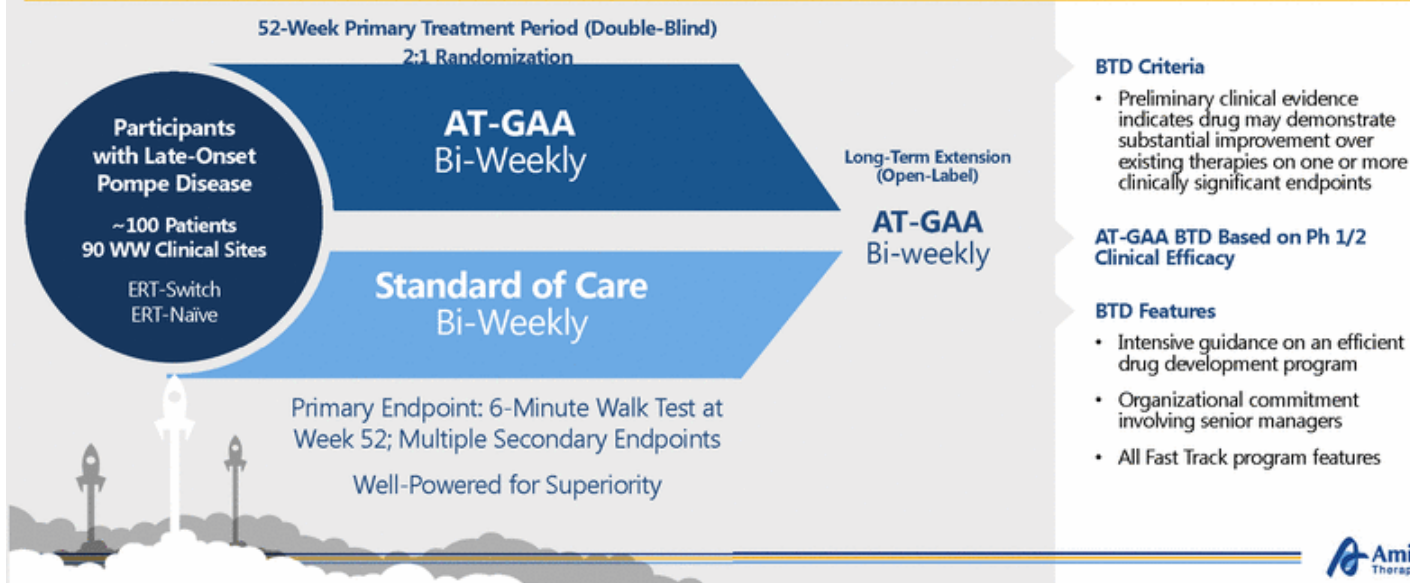
*One patient in Cohort 1 discontinued from study (withdrew consent) before Month 24. ^bAt the time of this interim analysis, 1 patient in Cohort 1 had not reached Month 24. ^cBaseline FVC missing for 1 patient in Cohort 1.



PROPEL (ATB200-03) Study Design

PROPEL

AT-GAA is the first ever second-generation product for any Lysosomal Disorder to earn Breakthrough Therapy Designation ("BTD") from the FDA; Phase 3 enrollment is expected to complete in 2019 with data in 1H 2021



Pompe Biologics Manufacturing

Amicus and partner WuXi Biologics have successfully produced AT-GAA at 1,000L commercial scale, demonstrating unique capabilities in Amicus biologics process science, manufacturing and quality control

- Agreements on biocomparability with key regulators (FDA, BfARM)
- PROPEL participants now treated with drug manufactured at 1000L
- Current bioreactor capacity to supply global population

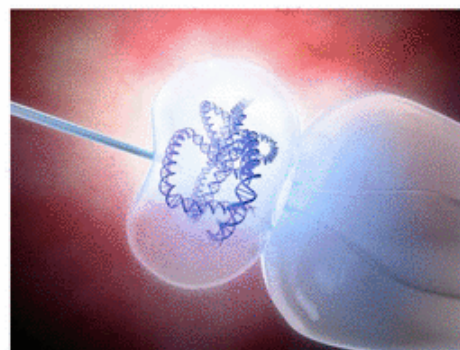
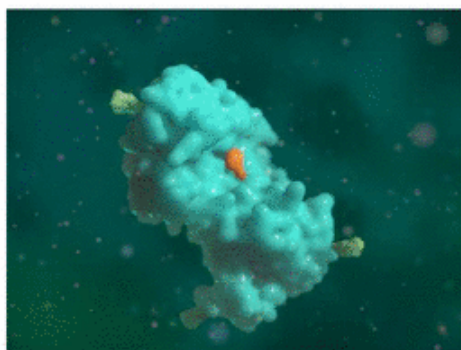
AT-GAA Treatment Opportunity

Potential to become the standard of care for all persons living with Pompe with \$1B+ in annual sales at peak

- Phase 1/2 study data on AT-GAA demonstrated profound improvement in functional outcomes for Pompe patients
- Enrollment of pivotal Phase 3 PROPEL Trial on track to complete enrollment of at least 100 patients by year end
- Ongoing studies intended to support approval in all patients
- AT-GAA plus Pompe gene therapy program can potentially build the largest and most valuable Pompe franchise in the industry

AT-GAA Treatment Opportunity

Any Pompe Gene Therapy will have to address both muscular and CNS aspects of disease and compare favorably on clinical endpoints to the then approved standard of care. AT-GAA has the potential to be standard of care well into the 2030s





Gene Therapy Pipeline

"We have a duty to obsolete our own technologies"
- Amicus Belief Statement

A Natural Evolution: Chaperones to Optimized ERT to Gene Therapy

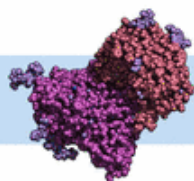
Amicus' Shift Towards Gene Therapy is Built Upon Years of Experience in Developing Genetic Medicines Designed to Deliver Deficient Proteins to Target Cells and Organelles

**Pharmacological
Chaperones**



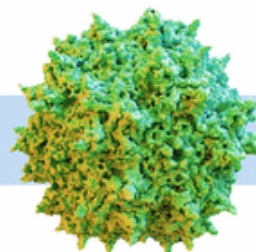
Stabilize
"naturally produced" enzymes

**Next-Generation
ERTs**



Stabilize and target
"externally produced" enzymes

**Gene
Therapies**



Stabilize and target
"internally produced" enzymes

Expansion in Gene Therapy

In less than a year, Amicus has assembled the largest portfolio of gene therapy programs for rare diseases in the entire industry

	DISCOVERY	PRECLINICAL	PHASE 1/2	PHASE 3	REGULATORY	COMMERCIAL
Fabry Franchise						
Galafold®(migalastat) monotherapy						
Fabry Gene Therapy	PENN					
Pompe Franchise						
AT-GAA (Novel ERT + Chaperone)						
Pompe Gene Therapy	PENN					
Batten Franchise – Gene Therapies						
CLN6 Batten Disease	NCH					
CLN3 Batten Disease	NCH					
CLN8 Batten Disease	NCH					
CLN1 Batten Disease	NCH					
Next Generation Research Programs and CNS Gene Therapies						
CDKL5 Deficiency Disorder GTx / ERT	PENN					
Niemann-Pick Type C (NPC)	NCH / PENN					
Tay-Sachs Disease	NCH					
Other	NCH / PENN					
MPS Franchise - Gene Therapies						
Next Generation MPSIIIA	PENN					
MPSIIIB	PENN					

Validated Gene Therapy Platform

The core science for these neurological Lysosomal programs leverage the AAV technologies and platforms utilized in the neuromuscular space at NCH and have robust pre-clinical proof of concept for their application in Batten disease

Clinically validated AAV gene therapy approach by Dr. Brian Kaspar and team at NCH and Sanford

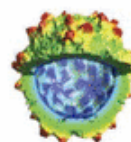
- Nationwide Children's Hospital Center for Gene Therapy (NCH)
- Intrathecal delivery with robust expression throughout CNS

Preclinical safety and efficacy studies replicated across multiple diseases at NCH

- SMA, ALS, CLN6, CLN3, CLN8

Amicus applying platform to Multiple Types of Batten disease and other Neurologic LSDs

- Two clinical programs in CLN6 and CLN3 Batten disease show initial safety in 15 patients; promising results in first two patients in CLN6
- Active preclinical programs in CLN8 and CLN1 Batten disease with other neurologic LSDs in earlier preclinical development



Foust, Kaspar et al, 2009

Source: Likhite 2018, 16th International Conference on Neuronal Ceroid Lipofuscinoses, IND-enabling Preclinical Studies for Batten Disease Gene Therapy



Batten Disease Overview

Batten disease is a devastating early childhood disease that is 100% fatal in children

Disease Overview

- A group of disorders known as neuronal ceroid lipofuscinoses (NCLs), collectively referred to as Batten disease
- Mutation in one of 13 different CLN genes leads to lysosomal dysfunction
- Signs and symptoms include:
 - Loss of speech, ambulation, vision and cognition



AAV-CLN6 Batten Disease Gene Therapy: Phase 1/2 Study

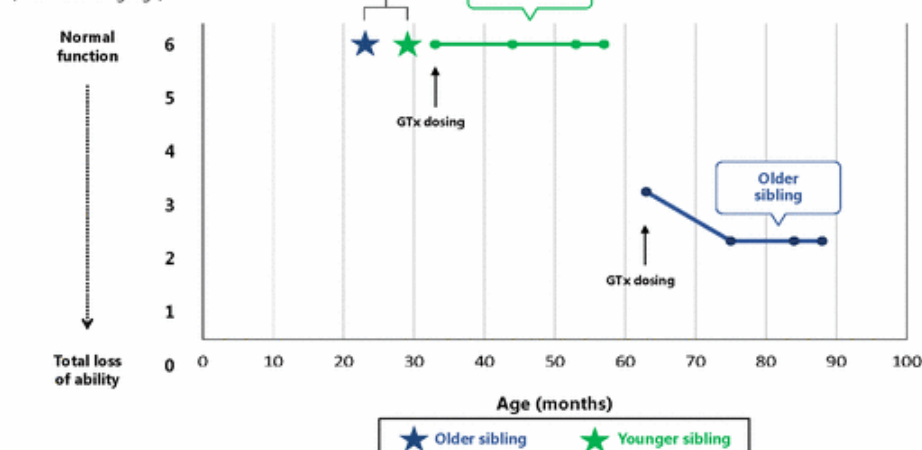
The Amicus CLN6 Batten disease program is one of the most advanced AAV Gene Therapy programs in development today

- ➔ 1st Patient Dosed >3 Years Ago
- ➔ 12 Children Treated
- ➔ Favorable Safety Profile
(exposure from ~7 to 41 months)

Efficacy Data: Matched Sibling Case Report

Encouraging Interim Efficacy Data in First Two Patients Treated with Gene Therapy with Two Years of Follow-up

Hamburg Score (Motor and Language)



- Two siblings (same genotype) treated with gene therapy at ages 2.8 and 5.3 years, respectively
- Two years post treatment, Hamburg motor and language scores indicate no disease progression in the younger sibling
- Disease progression in older sibling has shown evidence of stabilization to date

Upcoming CLN6 Batten Disease Interim Clinical Data

Important data in 7 patients at ~2 years following gene therapy administration will be reported in 3Q19

- 1 Safety
- 2 Hamburg Motor + Language (study patients)
- 3 Hamburg Motor + Language (natural history)
- 4 Matched Sibling Pairs (treated vs. natural history*; treated vs. treated**)

Key question for next CLN6 data set:

"As compared with natural history and sibling pair data, has the fundamental course of disease been profoundly changed, especially in younger children before severe neurodegeneration has occurred?"

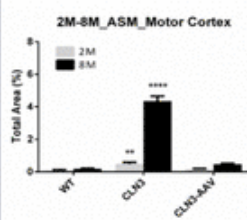
*study participants with untreated CLN6 siblings as natural history controls

**siblings are clinical study participants who received CLN6 gene therapy

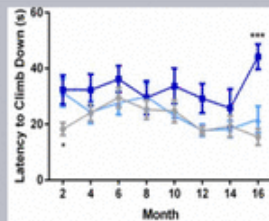
CLN3 Batten Disease: Preclinical and Clinical Summary

Amicus' second clinical stage Gene Therapy in CLN3 Batten disease has successfully completed dosing in three children in Cohort 1 (low dose) and dosing for up to an additional three patients in Cohort 2 (high dose) will be completed in 2H 2019

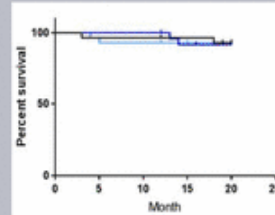
Preclinical Data in KO Mice



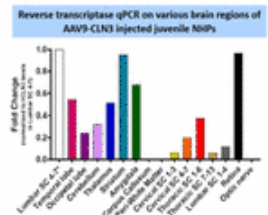
Reduction of storage material in mouse model



Improvement of motor function and cognitive behavior in mouse model



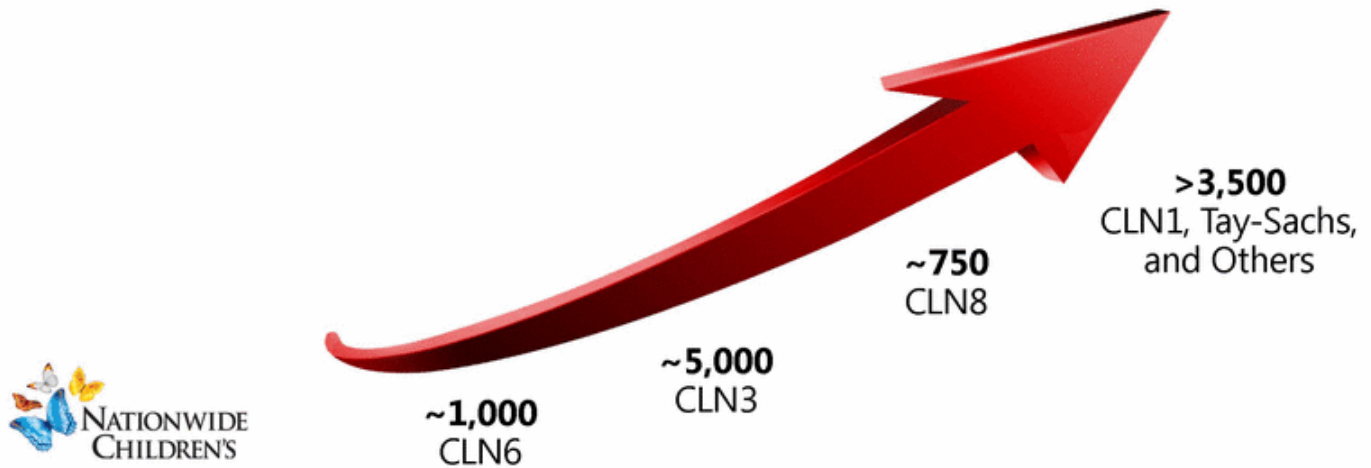
Comparable survival in mouse model



Widespread gene expression in brain of NHPs

Addressable Patient Populations in Neurologic LDs*

The portfolio of NCH/Celenex programs has the potential to reach \$1B+ in peak annual recurring revenue



*Estimated addressable U.S., EU, Japan, and other major, reimbursable markets based on published incidence and prevalence

Amicus Establishes Gene Therapy Portfolio

The original Amicus/Penn Gene Therapy collaboration with Dr. Jim Wilson's lab was announced in October 2018 and focused on combining Penn's gene therapy experience and technologies with Amicus expertise in protein engineering. It centered on programs in Pompe, Fabry and CDKL5 Deficiency Disorder

Increased Protein Expression

Novel untranslated sequences to avoid inhibition of initiation and drive efficient protein synthesis

Increased Protein Secretion

Effective signal sequences to increase protein expression & secretion

Improved Protein Targeting & Stabilization

Targeting moieties
Protein design



Penn



Penn Collaboration Overview

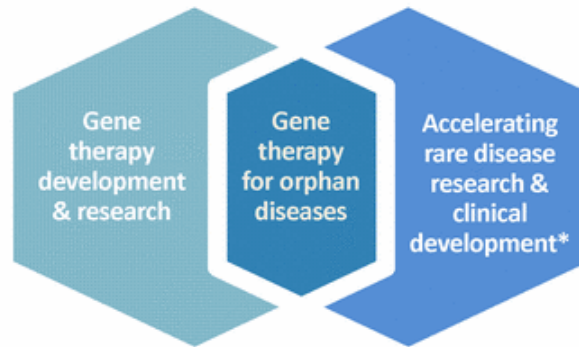
Combines Amicus Expertise in Protein Engineering with Penn's AAV Vector Technology, Manufacturing and Immunology Capabilities to Improve Safety and Efficacy and Speed Development

- **Dr. James Wilson and Gene Therapy Program (GTP) at Penn: Renowned center of excellence**

- >20 years of gene therapy experience
- Proven platform with numerous clinical programs across multiple disease indications
- Leader in next generation AAV technologies



**GENE
THERAPY
PROGRAM**



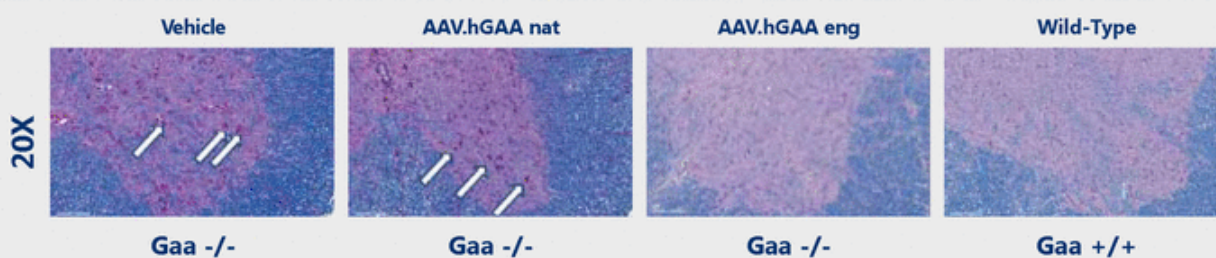
**the
orphan
disease center**

* therapeutic approach agnostic

Preclinical Pompe Gene Therapy Results

Within 7 months, the combined Amicus/Penn efforts to develop an advanced Gene Therapy in Pompe disease yielded striking results presented at the ASGCT meeting in April 2019

Glycogen Luxol/PAS – Spinal Cord



Key findings:

- Improved cellular uptake and glycogen reduction observed with engineered AAV-hGAA
- Robust glycogen reduction in CNS observed only with engineered AAV-hGAA

Amicus Gene Therapy Expansion: Key Takeaways

In May 2019, Amicus and Penn announced a major expansion of the collaboration



GENE
THERAPY
PROGRAM



Worldwide Rights to Penn's Next Generation Gene Therapy Technologies for the Majority of Lysosomal Disorders

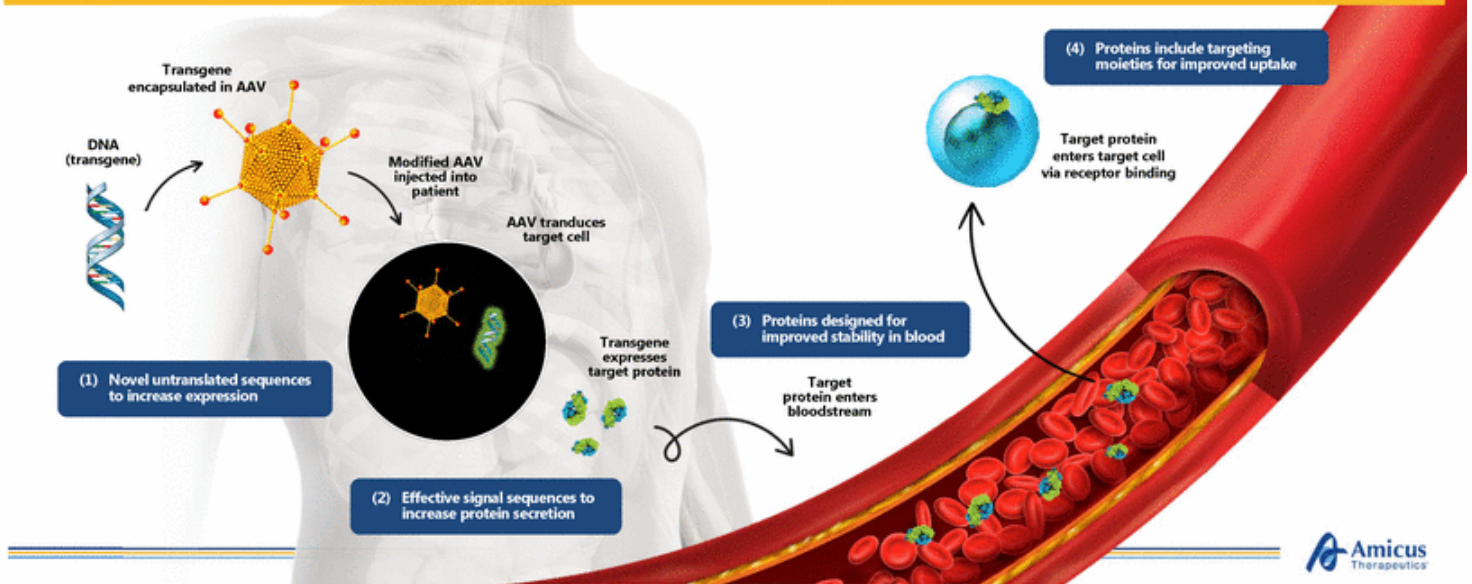
Current Collaboration Extended to Include Three New Indications: Niemann-Pick Type C, Next Generation MPS IIIA, and MPS IIIB

Partnership Encompasses 12 Additional Rare Diseases, including Rett Syndrome, Angelman Syndrome, Myotonic Dystrophy and Select Other Muscular Dystrophies

Amicus to Invest \$10M / Year for 5 Years for Research to Improve Safety, Efficacy and Manufacturability of Next Generation Vectors with Option to Extend

Amicus Approach: Engineered Transgenes for Optimal Cross-Correction

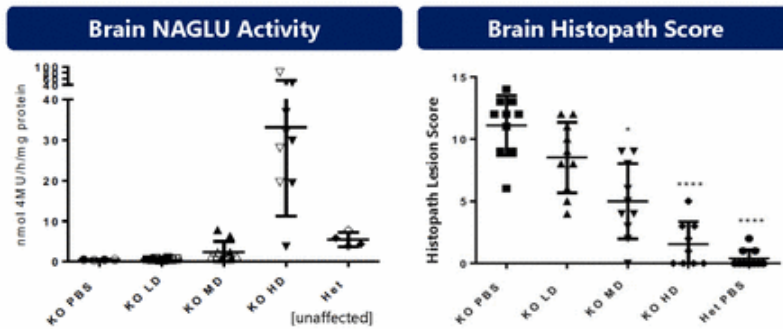
Amicus' unique technologies for protein engineering in Gene Therapy represent a new major platform technology and a groundbreaking advancement in the field



MPS IIIB Program Summary

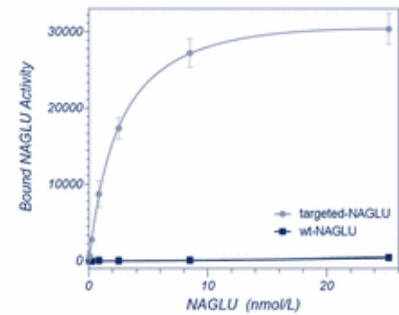
The Amicus protein engineering technologies have now been applied to Penn's MPS IIIB program to improve cellular uptake and cross-correction of affected cells

AAV-NAGLU Candidate (Unmodified WT): KO Mouse*



- AAV-NAGLU candidate (unmodified); 3 single ICV doses (LD, MD, HD)
- 4-mo old KO mice (n=10); Sacrificed 90-days post injection

NAGLU Binding to Intended Receptor**



Potential to engineer NAGLU transgene for improved receptor binding and cellular uptake

Preclinical Advancements in GTx to come in 2H 2019

Multiple preclinical pipeline advancements in Gene Therapy lie ahead in 2H 2019

Gene Therapy Program Milestones

- ☒ Positive initial preclinical data for Pompe gene therapy
- ☒ Selection of CMO partners for initial manufacturing supply
- ☒ Complete enrollment in low-dose cohort in CLN3 Batten disease Phase 1/2 study
- ☐ Complete enrollment in high-dose cohort in CLN3 Batten disease Phase 1/2 study
- ☐ Additional 2-year data from CLN6 Batten disease Phase 1/2 study
- ☐ Selection of Amicus late process development and manufacturing facilities
- ☐ Additional preclinical data including next-generation gene therapies for Fabry and Pompe
- ☐ Selection of Pompe AAV gene therapy IND candidate

Amicus Global Research and Gene Therapy Center of Excellence in Philadelphia

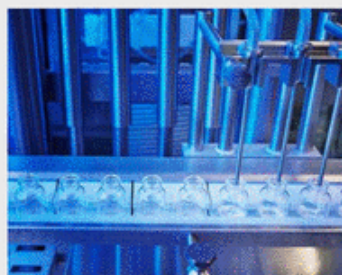
The 75,000 sq. ft. Amicus Global Research & Gene Therapy Center of Excellence in Philadelphia will be fully operational in 2H 2019 and is adjacent to Penn's campus



Artist renderings

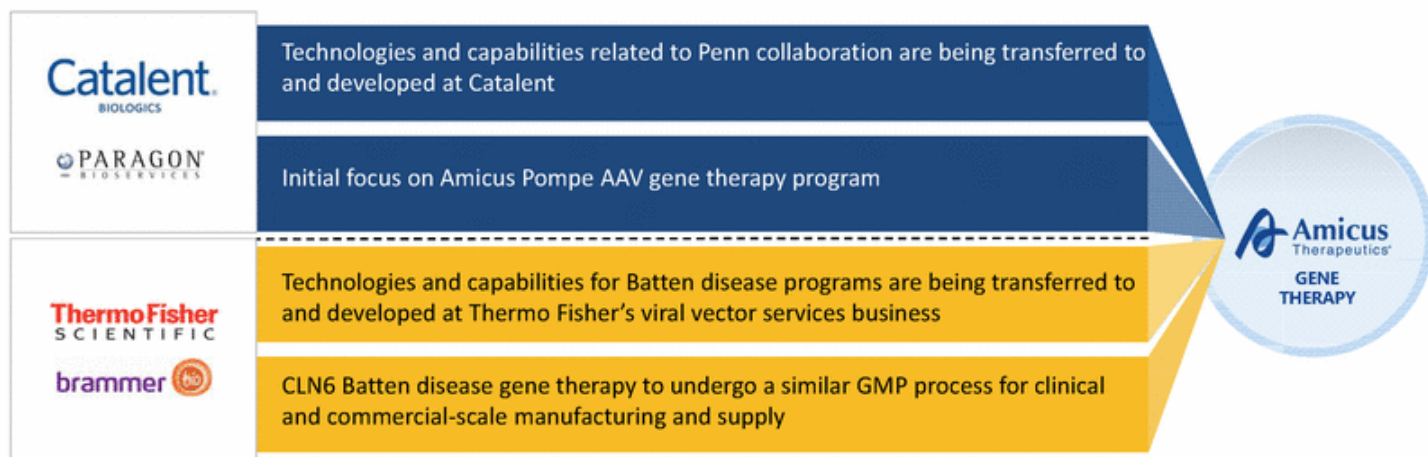
Manufacturing and Process Science

Manufacturing and process science capabilities and capacity in Gene Therapy will be crucial for Amicus success. Our experience in complex biologics manufacturing and quality control provide a great competitive advantage here.



CMO Partnerships

Manufacturing Partnerships Secured with Industry Leading CMOs with Tech Transfer Process Underway



Plasmid Supply Relationships

Amicus has also established long term supply agreements with industry leading plasmid suppliers for research and GMP quality plasmids for all Amicus Gene Therapy programs.



Amicus Manufacturing Center of Excellence

Amicus will build, staff and operate our own state of the art Gene Therapy Manufacturing Center of Excellence. This manufacturing complex will become one of the largest, most flexible and most technically advanced Gene Therapy manufacturing centers in the world



Financial Summary and Guidance

Amicus financial strength provides a great foundation for the future, with ~\$580M cash on hand

FINANCIAL POSITION

Cash (Proforma)	~\$580M
Cash Runway¹	Into 2021
Debt²	\$152.8M

CAPITALIZATION

Shares Outstanding³	~255M
---------------------------------------	-------

FINANCIAL GUIDANCE

FY19 Galafold Revenue Guidance	\$160M-\$180M
---------------------------------------	---------------

¹Based on existing operating plan including proceeds from June 2019 equity offering to invest in manufacturing

²Includes \$3.7 million of convertible debt and \$150 million of straight debt

³Includes shares from June 2019 equity offering

Our Passion for Making a Difference Unites Us

Amicus is now at a major inflection point and positioned to create significant shareholder value ahead while advancing our mission for patients.

