

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION**

Washington, D.C. 20549

**FORM 8-K**

**CURRENT REPORT**

**Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934**

Date of Report (Date of earliest event reported): **January 8, 2018**

**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**  
(IRS Employer Identification No.)

**1 Cedar Brook Drive, Cranbury, NJ**  
(Address of Principal Executive Offices)

**08512**  
(Zip Code)

Registrant's telephone number, including area code: **(609) 662-2000**

(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))

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.

**Item 8.01. Other Events.**

The senior management of Amicus Therapeutics, Inc. (the "Company") is using the presentation attached as Exhibit 99.1 to this Current Report in its current meetings with investors and analysts. In addition, on January 8, 2018, the Company filed a press release, a copy of which is attached to this Current Report as Exhibit 99.2.

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

(d) Exhibits: The Exhibit Index annexed hereto is incorporated herein by reference.

<u>Exhibit No.</u>	<u>Description</u>
99.1	<a href="#">Presentation Materials — 36th Annual J.P. Morgan Healthcare Conference (January 2018)</a>
99.2	<a href="#">Press Release — 2018 Guidance</a>

**SIGNATURES**

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: January 8, 2018

By: /s/ ELLEN S. ROSENBERG  
Name: Ellen S. Rosenberg  
Title: General Counsel and Corporate Secretary

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36<sup>th</sup> Annual  
J.P. Morgan  
Healthcare  
Conference

John F. Crowley, Chairman and Chief Executive Officer  
January 9, 2018

## Safe Harbor

*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 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, 2016 and Quarterly Report on Form 10-Q for the quarter ended September 30, 2017. 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 presentation to reflect events or circumstances after the date hereof.*

# Amicus Founding Beliefs

## WE BELIEVE...

**In the Fight to Remain at the Forefront of Therapies for Rare and Orphan Diseases**

- We seek to deliver the highest quality therapies for persons living with these diseases
- We support the disease communities - and their families
- We are passionate about what we do
- We encourage and embrace constant innovation
- We have a duty to obsolete our own technologies
- We push ideas as far and as fast as possible
- We take smart risks
- We work hard
- We keep asking the tough questions
- We will never be constrained by prior thinking
- We learn from our mistakes
- We think differently - very differently

## WE BELIEVE...

**In Our Future to Build Long-term Value for Our Stakeholders**

- We are all owners of this business
- We are business led and science driven
- Maximizing value for our shareholders is the foundation of our future successes
- Our medicines must be fairly priced and broadly accessible
- We build strategic partnerships
- We will not lie, cheat or steal
- We take full responsibility for our actions

## WE BELIEVE...

**In Each Other to Foster Teamwork and Respect for Each Individual's Contribution**

- Our passion for making a difference unites us
- Diversity of experience and thought is essential
- We communicate openly, honestly and respectfully
- Our families are part of the Amicus experience
- Work-life balance keeps us healthy

# Amicus Founding Beliefs

WE BELIEVE...

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WE BELIEVE...

**We push ideas as far and as fast as possible**

**We encourage and embrace constant innovation**

**We have a duty to obsolete our own technologies**

**We are business led and science driven**

**Our passion for making a difference unites us**

Orphan Diseases

- We encourage and embrace constant innovation
- We have a duty to obsolete our own technologies
- We push ideas as far and as fast as possible
- We take smart risks
- We work hard

Maximizing value for our shareholders is the foundation of our business

- We build strategic partnerships

- Our passion for making a difference unites us
- Openness, transparency and thought is essential

- We communicate openly

- Work-life balance keeps us healthy

## Amicus Mission

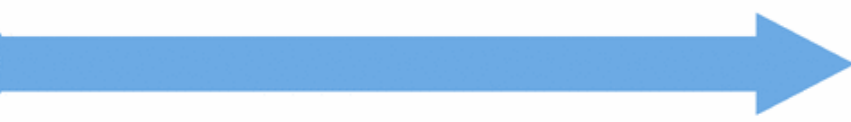
*We seek to deliver the highest quality therapies for persons living with rare metabolic diseases*

# Amicus Vision: Delivering for Patients and Shareholders

To build a top-tier, fully integrated, global biotechnology whose medicines treat 5,000+ patients with \$1B+ in worldwide sales revenue by 2023



>350 Patients\* | ~\$36M Global Sales



5,000 Patients\* | \$1B Global Sales

\*Clinical & commercial, all figures approximate





# Amicus Strategy

## Strategic Goals:

Create...

Manufacture...

Test...

Deliver...

...Great Medicines

## Critical Initiatives:

Invest in core internal scientific technologies

Actively in-license complementary products and technologies in rare, metabolic diseases

Strengthen and expand relationships with WuXi Biologics and other core manufacturing partners

Build internal capabilities and capacity for biologics manufacturing

Complete build-out of global commercial and development footprint with world-class teams

Apply highest levels of business ethics and social responsibility

# Amicus Today



**Galafold**  
(migalastat)

FIRST ORAL PRECISION MEDICINE  
FOR FABRY DISEASE

**ATB200/AT2221**

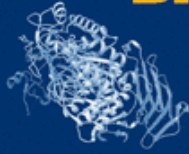
NOVEL TREATMENT  
PARADIGM for  
Pompe Completed Phase 1/2

PRECLINICAL  
**PIPELINE**

of products for rare  
metabolic diseases

**BIOLOGICS**  
PLATFORM

Protein Engineering  
& Glycobiology



CHAPERONE-ADVANCED  
REPLACEMENT THERAPY

**SMALL  
MOLECULE**

Pharmacological  
Chaperones

**~400**  
**EMPLOYEES**  
globally

**~\$359M**  
cash

**GLOBAL  
FOOTPRINT**  
in 27 countries

# Our Passion for Making a Difference Unites Us



## Excellence in Execution in 2017

Successful Achievement of FOUR Key Strategic Priorities in 2017 to Build a Top Global Biotechnology Company Focused on Rare Metabolic Diseases

**1 Advance International Galafold Launch (Target 300 Patients)**



**2 Submit Japanese and U.S. NDAs for Migalastat**



**3 Establish Definitive Proof of Concept for ATB200/AT2221**



**4 Maintain Financial Strength**



## 2018 Key Strategic Priorities

Focused on FIVE Key Strategic Priorities in 2018

- 1 Double Galafold (migalastat) revenue to \$75-\$85M
- 2 Secure approvals for migalastat in Japan and the U.S.
- 3 Achieve clinical, manufacturing and regulatory milestones to advance ATB200/AT2221 toward global regulatory submissions and approvals
- 4 Develop and expand preclinical pipeline to ensure at least one new clinical program in 2019
- 5 Maintain Financial Strength

# Building a World Class Organization

Global Organization of ~400 Employees Dedicated to Create, Manufacture, Test, and Deliver Medicines for Rare Metabolic Diseases





# Galafold™ (Migalastat) Precision Medicine for Fabry Disease

*"We push ideas as far and as fast as possible"*

- Amicus Belief Statement

# Fabry Disease Overview

**Fabry Disease is a Fatal Genetic Disorder that Affects Multiple Organs and is Believed to be Significantly Underdiagnosed**

## Leading Causes of Death:

**TRANSIENT ISCHEMIC ATTACK (TIA) & STROKE<sup>1</sup>**

**HEART DISEASE<sup>2</sup>**

**KIDNEY DISEASE<sup>3</sup>**

## Life-Limiting Symptoms:

**GASTROINTESTINAL<sup>3</sup>**

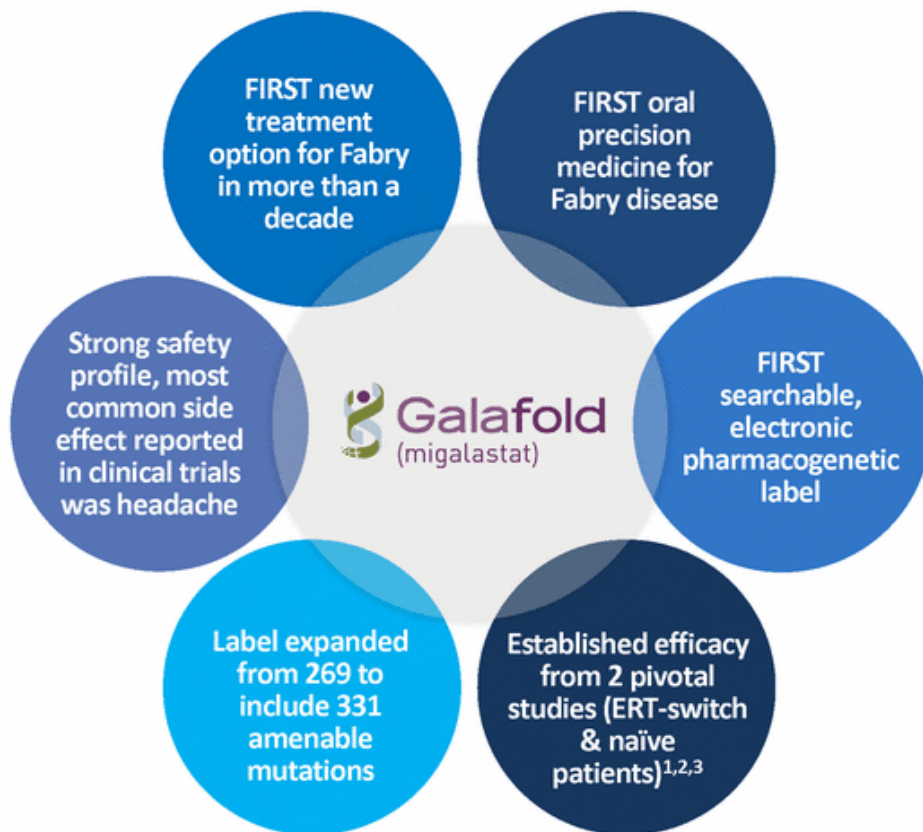
## Key Facts:

- $\alpha$ -Gal A enzyme deficiency leads to substrate (GL-3) accumulation
- >1,000 known mutations
- ~10K diagnosed WW (51% female/49% male<sup>4</sup>)
- Newborn screening studies suggest prevalence of ~1:1000 to ~1:4000

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. Fabry Registry 2011



# First Oral Precision Medicine for Fabry Disease



Galafold Indicated for Long-Term Treatment of Adults and Adolescents Aged  $\geq 16$  years with a Confirmed Diagnosis of Fabry Disease and Who Have an Amenable Mutation<sup>3</sup>

Approved in 6 Major Markets<sup>4</sup>

Pricing & Reimbursement Secured in 15 Countries

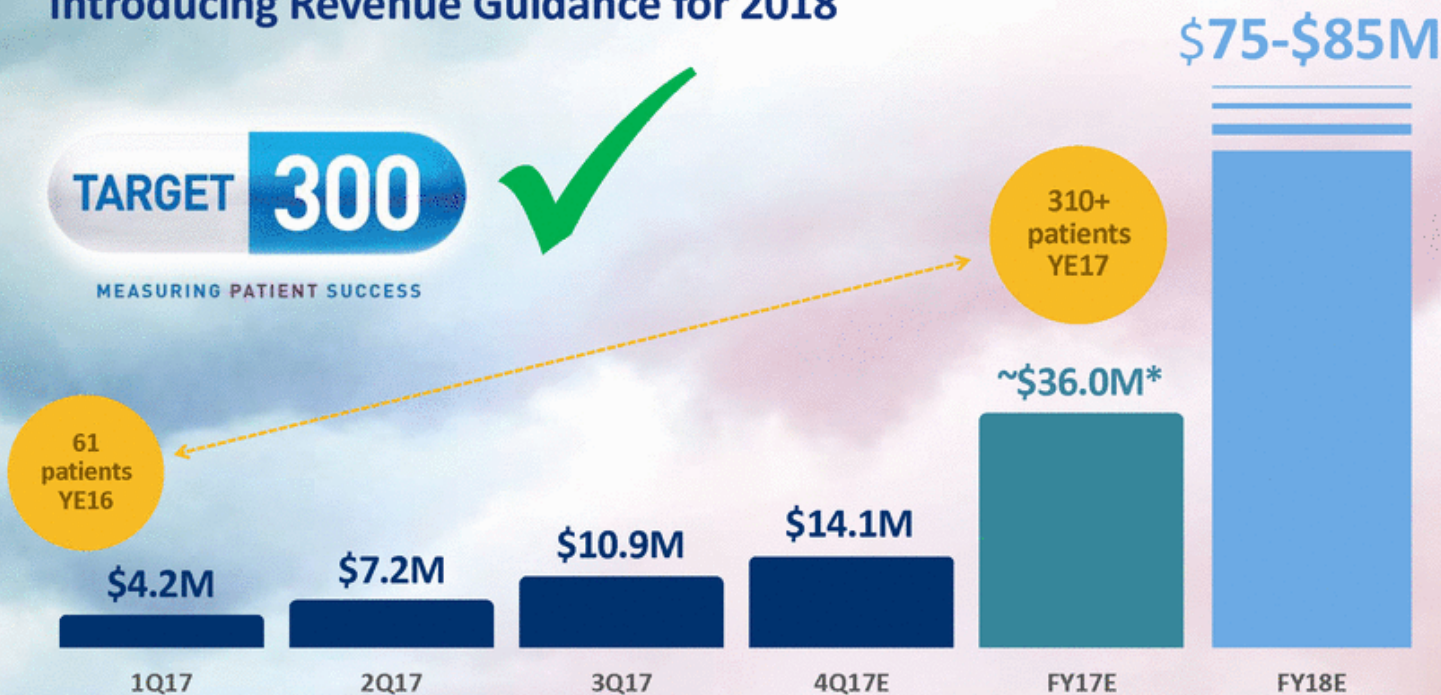
Approvals Pending in Japan, U.S. and Other Geographies

1. Germain, DP et al., New England Journal of Medicine. 2. Hughes, et al., Journal of Medical Genetics. 3. For important safety information for Galafold visit [www.ema.europa.eu](http://www.ema.europa.eu).  
4. EU, Australia, Canada, Israel, Switzerland, South Korea

# FY17 Galafold Success and FY18 Galafold Revenue Guidance

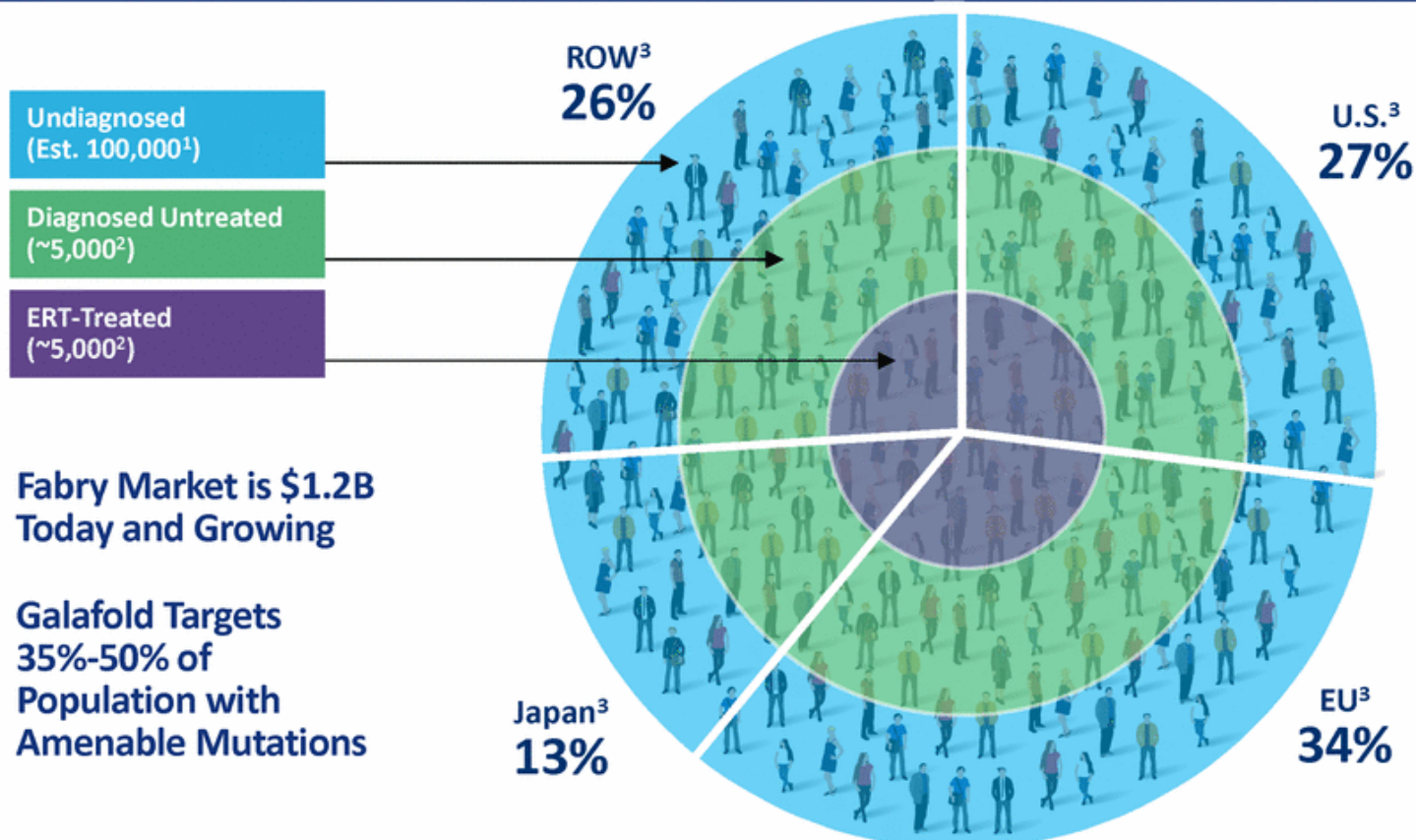
International Launch Success in 2017 Positions for Significant Growth in 2018 and Beyond

## Introducing Revenue Guidance for 2018



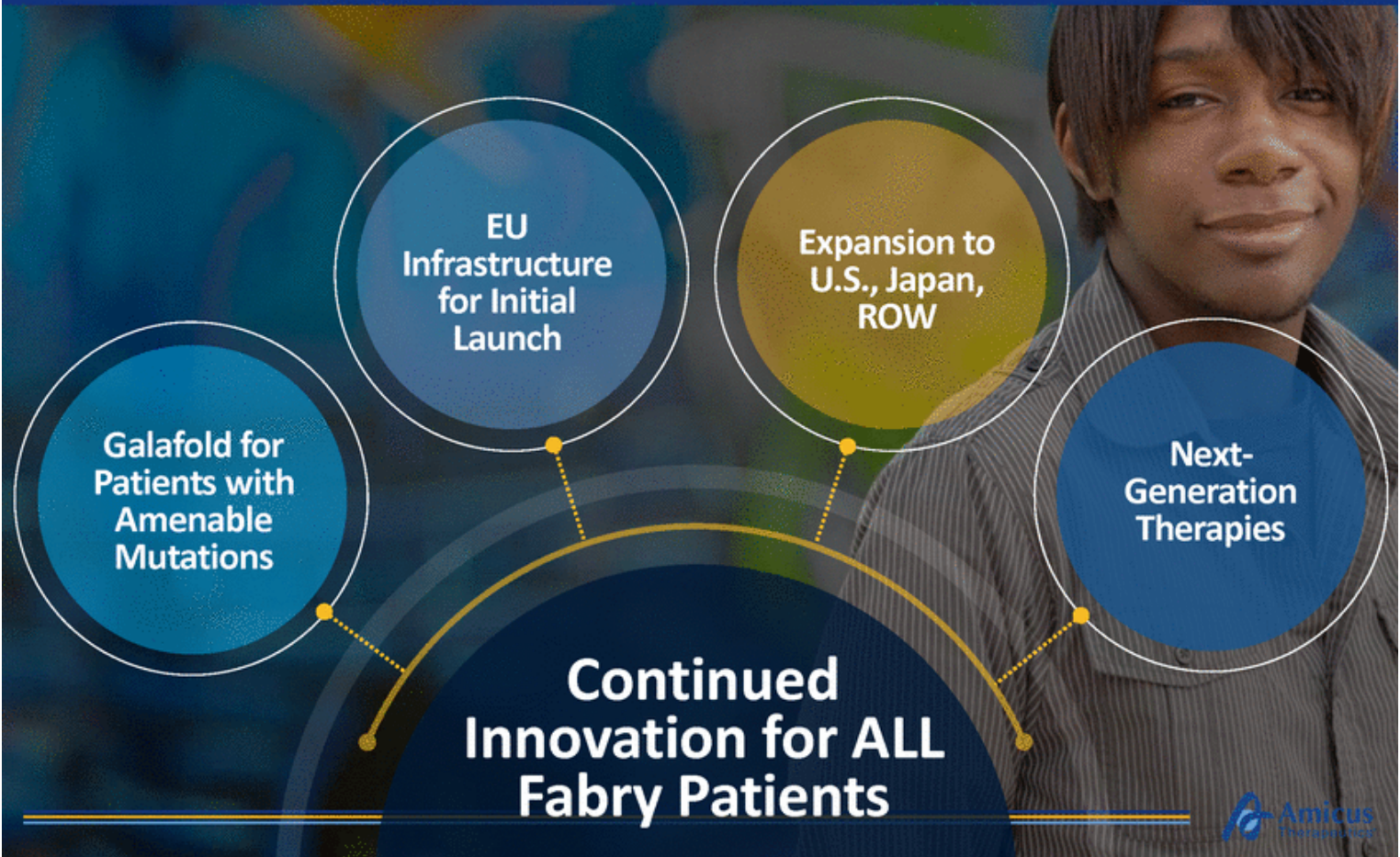
\*Unaudited preliminary financials

# Galafold \$500M+ Global Peak Revenue Opportunity



1. Bokhari SRA, Bhimji SS. Fabry Disease. December 2017 2. Sanofi presentation February 2017 3. Fabry registries, Sanofi investor reports, Shire investor reports

# Fabry Franchise Strategy



# Fabry Precision Medicine Driven by a Patient's Genotype

**Amicus Therapeutics is Committed to Delivering the Highest Quality Therapies**

**Migalastat**  
Oral Precision Medicine



**~\$1.2B**  
Global Fabry Market Today



Growing to  
**~\$2B**  
Global Fabry Market



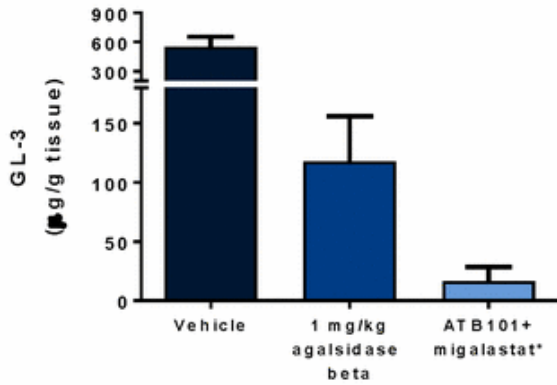
**Novel ERT Co-Formulated with Migalastat**



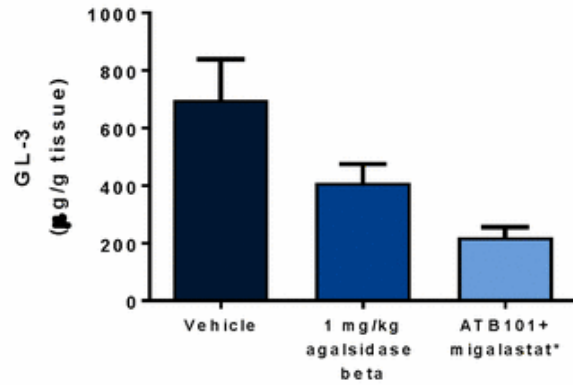
# Amicus Proprietary ERT Preclinical Proof of Concept

## ATB101 Co-formulated with Migalastat Results in Significantly Greater Substrate Reduction In Fabry KO Model

### Heart



### Kidney



Notes: \*3 mg/kg ATB101 + 10 mg/kg AT1001; Data from Gla KO mice administered two bi-weekly doses; p<0.05



# ATB200 Novel ERT for Pompe Disease

*"We encourage and embrace constant innovation"*

- Amicus Belief Statement

# Pompe Disease Overview

**Pompe Disease is a Fatal Neuromuscular Disorder that Affects a Broad Range of People**



5,000 – 10,000 patients diagnosed WW<sup>1</sup>

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

Symptoms include muscle weakness, respiratory failure, and cardiomyopathy

~\$800M+ Global Pompe ERT sales in FY16<sup>2</sup>

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

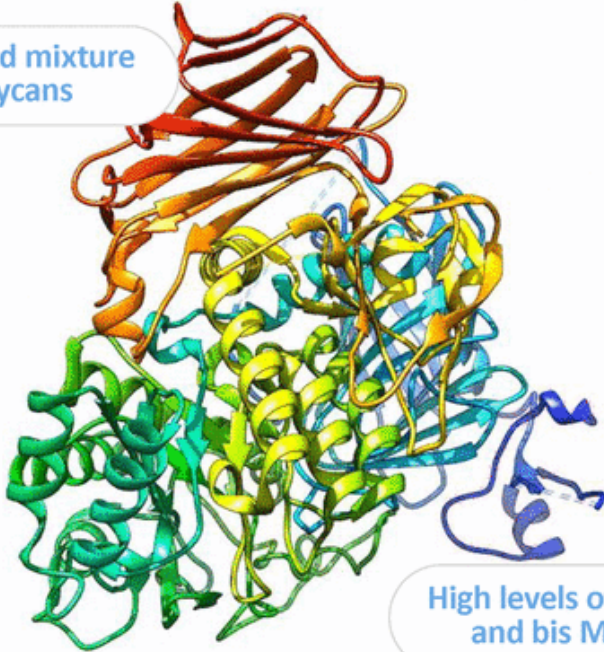


# ATB200 + Chaperone: A Differentiated Treatment Paradigm

## Application of Platform Technologies for Potential New Treatment Paradigm

### ATB200 (Novel ERT)

Optimized mixture of glycans



High levels of M6P and bis M6P



CHAPERONE-ADVANCED  
REPLACEMENT THERAPY

Chaperone  
addition



\*Artist rendering, not actual product image

## Phase 1/2 Data Presented at World Muscle Society

*"I believe that the results from this Phase 1/2 clinical study show striking improvements in functional measures and key biomarkers during the first six months of treatment, in addition to continued, further benefit out to nine months. I am especially intrigued by the six-minute walk distance and other motor function tests in the ERT-switch patients who historically have declining motor function following two or more years of treatment. These clinical data are compelling and suggest that ATB200/AT2221 has the potential to shift the treatment paradigm for Pompe disease."<sup>1</sup>*

**Mark Roberts, MD**

Department of Neurology, Salford Royal NHS Foundation Trust and  
Principal Investigator in the ATB200-02 study



**The 22nd International Annual  
Congress of the World Muscle Society**

PALAIS DU GRAND LARGE  
CONGRES SEMINAIRES

Casino Barrière de Saint-Malo

# 6-Minute Walk Test (6MWT) and Forced Vital Capacity (FVC) (as of 10/4/17)

Improvements in Key Functional Measure in both ERT-Naïve and ERT-Switch at Month Six with Continued Benefit Out to Month Nine

## 6-Minute Walk Test (m): Month 6 and 9

Cohort	Change at Month 6 (n=9) Mean (SD)	Change at Month 9 (n=8) Mean (SD)
Cohort 1 ERT-Switch Ambulatory	<b>+35.3</b> (40.1)	<b>+37.2</b> (33.8)
Cohort	Change at Month 6 (n=5) Mean (SD)	Change at Month 9 (n=2) Mean (SD)
Cohort 3 ERT-Naïve	<b>+41.8</b> (29.4)	<b>+74.9</b> (4.0)

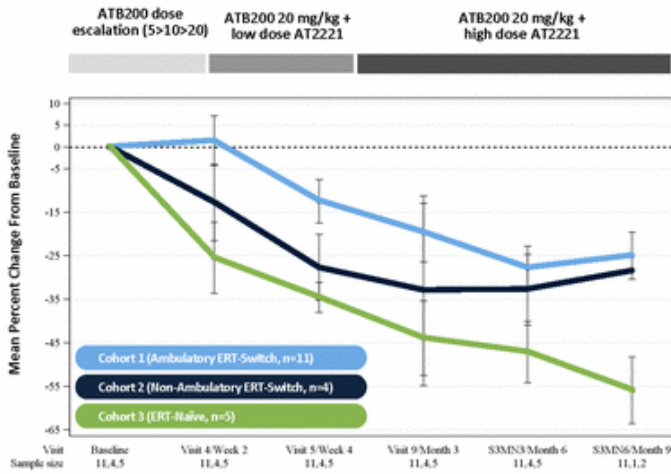
## FVC (% Predicted): Month 6 and 9

Cohort	Change at Month 6 (n=8) Mean (SD)	Change at Month 9 (n=7) Mean (SD)
Cohort 1 ERT-Switch Ambulatory	<b>-1.0</b> (4.2)	<b>-2.0</b> (3.6)
Cohort	Change at Month 6 (n=5) Mean (SD)	Change at Month 9 (n=2) Mean (SD)
Cohort 3 ERT-Naïve	<b>+4.2</b> (5.6)	<b>+5.0</b> (1.4)

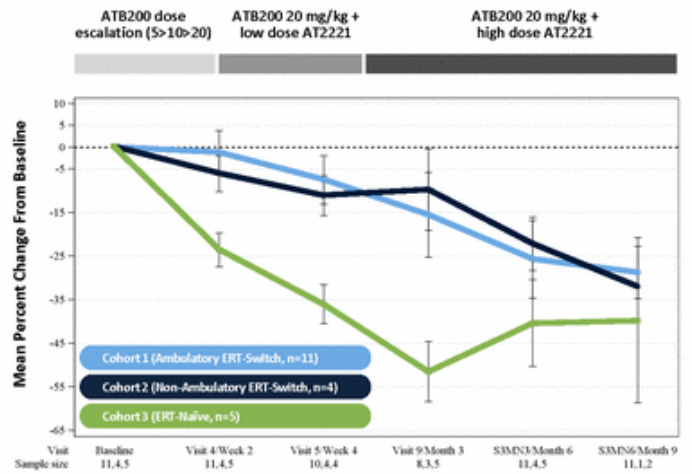
# Biomarkers up to Week 58 (N=20)\* (as of 10/4/17)

Persistent and Durable Improvement in Biomarkers of Muscle Damage (CK, ALT, AST) and Disease Substrate (Hex4) Across All Three Cohorts for up to 58 Weeks on ATB200/AT2221

## Muscle Damage Biomarkers (% Change from Baseline for CK)



## Disease Substrate Biomarker (% Change from Baseline for Hex 4)



### SAFETY

- Adverse events (AEs) generally **mild and transient**
- Very **low rate** of infusion associated reactions (IARs) (<1%) after **400+** total infusions

\*Reported through interim data analysis (maximum 58 weeks); Missing values either unable to be analyzed or not yet analyzed



# Pompe Development Pathways

**Our Goal: To Work with Global Regulators to Ensure That as Many People Living with Pompe Have Access to This Novel Treatment Paradigm as Quickly as Possible**

## Potential Pathways Include:\*



\*Subject to ongoing discussions with regulatory authorities, update anticipated 1H18

# Key Clinical & Manufacturing Activities 2018

## Significant Clinical and GMP Manufacturing Activities Ongoing in 2018 to Lay Foundation for Most Successful and Fastest Approval Pathways

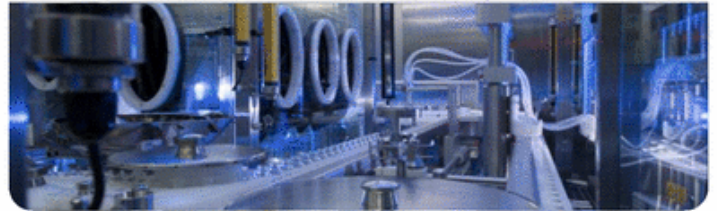
### CLINICAL

- Additional Phase 1/2 extension data
- Additional 4-6 patients added to Phase 1/2 study
- Retrospective natural history of ERT-treated patients
- Prospective data collection on current ERT-treated patients
- Initiation of larger registration-directed study



### MANUFACTURING

- Final regulatory agreement on comparability between 1,000L and 250L GMP scale
- Completion and release for clinic of 1,000L GMP commercial scale material
- Continued capacity to ensure sufficient medicines to supply patient population
- Announce plan for long term commercial manufacture and capacity



# Biologics Manufacturing Capabilities

## Scaling up Manufacturing to Meet the Needs of the Pompe Community

**1000L**

(Registration & Commercial)

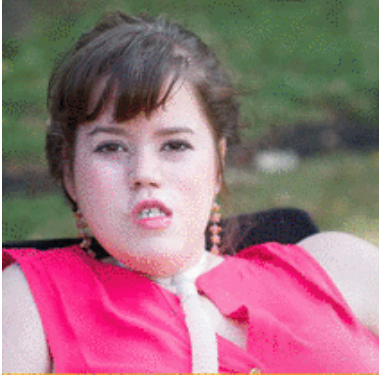
All engineering runs complete

GMP production commenced

Analytical and *in vivo* comparability studies completed between 250L and 1000L

FDA agreement on comparability between 250L GMP scale and 1000L engineering batches

FDA agreement on testing strategy for demonstrating comparability between 250L scale and 1000L GMP batches



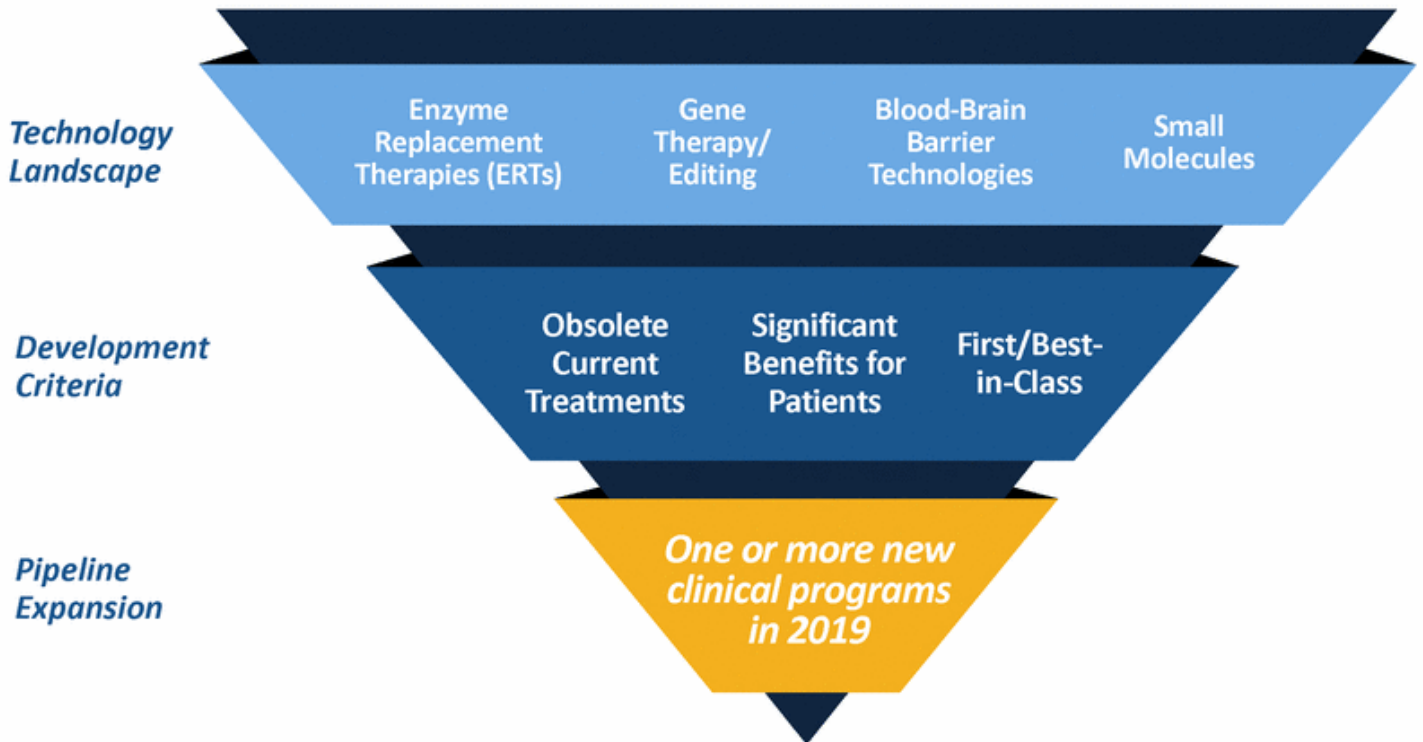
# Pipeline Strategy

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



# Pipeline Strategy

Sharply Focused on Developing Therapies for People Living with Rare Metabolic Diseases





# Financial Summary & Key Milestones

*"We are business led and science driven"*

- Amicus Belief Statement

# Financial Summary & Guidance

**Strong Balance Sheet with \$359M Cash at 12/31/17 and Cash Runway Into 2H19**

## FINANCIAL POSITION

December 30, 2017

<b>Cash</b>	\$359M
<b>Debt</b>	\$250M
<b>Cash Runway</b>	2H19

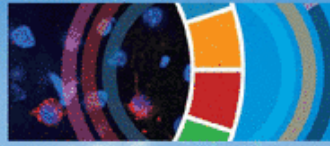
## CAPITALIZATION

<b>Shares Outstanding</b>	167M
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## FINANCIAL GUIDANCE

<b>FY18 Net Cash Spend Guidance</b>	\$230-\$260M
<b>Galafold Revenue Guidance</b>	\$75-\$85M

# WORLDSymposium™ 2018



14TH ANNUAL  
**WORLDSymposium™**  
February 5-9, 2018

*We're Organizing Research on Lysosomal Diseases*

## Pompe

- Pompe preclinical and clinical data, including additional Phase 1/2 data for up to 12 months

## Fabry

- Migalastat long-term data
- ATB101 co-formulated with migalastat preclinical proof of concept



## 2018 Key Strategic Priorities

Focused on FIVE Key Strategic Priorities in 2018

- 1 Double Galafold (migalastat) revenue to \$75-\$85M
- 2 Secure approvals for migalastat in Japan and the U.S.
- 3 Achieve clinical, manufacturing and regulatory milestones to advance ATB200/AT2221 toward global regulatory submissions and approvals
- 4 Develop and expand preclinical pipeline to ensure at least one new clinical program in 2019
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# Amicus Vision: Delivering for Patients and Shareholders

To build a top-tier, fully integrated, global biotechnology whose medicines treat 5,000+ patients with \$1B+ in worldwide sales revenue by 2023



>350 Patients\* | ~\$36M Global Sales



5,000 Patients\* | \$1B Global Sales



\*Clinical & commercial, all figures approximate

# Thank You

*“Our passion for making a difference unites us”*

*-Amicus Belief Statement*





# Appendix



# Fabry Disease Overview

**Fabry Disease is a Fatal Genetic Disorder that Affects Multiple Organs and is Believed to be Significantly Underdiagnosed**

## Leading Causes of Death

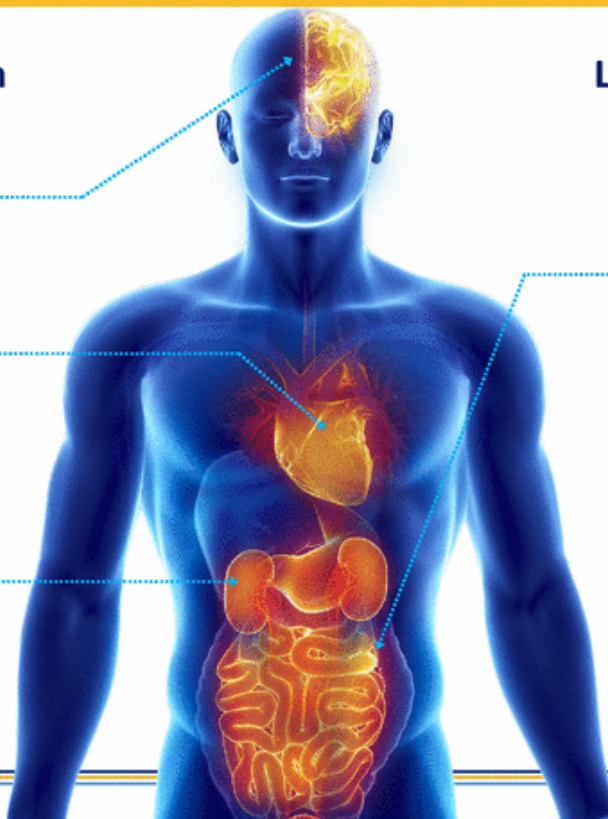
### TRANSIENT ISCHEMIC ATTACK (TIA) & STROKE<sup>1</sup>

### HEART DISEASE<sup>2</sup>

- Irregular heartbeat (fast or slow)
- Heart attack or heart failure
- Enlarged heart

### KIDNEY DISEASE<sup>3</sup>

- Protein in the urine
- Decreased kidney function
- Kidney failure



## Life-Limiting Symptoms

### GASTROINTESTINAL<sup>3</sup>

- Nausea, vomiting, cramping, and diarrhea
- Pain/bloating after eating, feeling full
- Constipation
- Difficulty managing weight

## Key Facts

- Deficiency of  $\alpha$ -Gal A enzyme leading to GL-3 accumulation
- >900 known mutations
- ~10K diagnosed WW (51% female/49% male<sup>4</sup>)
- Newborn screening studies suggest prevalence of ~1:1000 to ~1:4000

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. Fabry Registry 2011

# Fabry Global Operations Excellence

## FABRY Initial Launch Success

### People

Deep experience in rare disease space

Hire “best and brightest” from range of leading biotech companies

Culture of strong patient focus

### Product

Differentiated safety and efficacy published in seminal journals\*

First-in-class oral therapy for Fabry

Precision medicine based on genotype

### Access

Compelling value proposition led to rapid reimbursement

Specialty distributor with high touch services

Commitment to patient access and support services

### Execution

Clear focus at launch on priority patient segments

Efficient outreach to key Fabry centers

Strong education efforts on importance of genotype

\*New England Journal of Medicine, Journal of Medical Genetics

## Cohort 2: Muscle Strength Testing at Month 6 (n=4) (as of 10/4/17)

### Substantial and Consistent Improvements in Upper Extremity Strength in Non-Ambulatory ERT-Switch Patients at Month 6

Assessment	Muscle Group Tested	Baseline		Change to Month 6	
		Left Mean (SD)	Right Mean (SD)	Left Mean (SD)	Right Mean (SD)
<b>QMT</b> Quantitative Muscle Testing - Dynamometer (pounds force)	Shoulder Adduction*	<b>4.2</b> (6.8)	<b>1.5</b> (1.9)	<b>+2.3</b> (4.4)	<b>+5.8</b> (8.4)
	Shoulder Abduction	<b>9.8</b> (10.9)	<b>6.9</b> (7.6)	<b>+0.3</b> (5.1)	<b>+0.8</b> (1.5)
	Elbow Flex	<b>7.8</b> (8.7)	<b>4.9</b> (5.1)	<b>-0.1</b> (10.0)	<b>+2.4</b> (6.1)
	Elbow Extension	<b>7.3</b> (8.1)	<b>5.0</b> (5.9)	<b>+1.5</b> (3.4)	<b>+4.1</b> (2.1)
<b>MMT</b> Manual Muscle Testing (manual score)	Shoulder Adduction*	<b>1.3</b> (1.2)	<b>1.0</b> (1.0)	<b>+0.7</b> (1.2)	<b>+0.7</b> (1.2)
	Shoulder Abduction**	<b>1.3</b> (1.2)	<b>1.3</b> (1.2)	<b>+0.5</b> (0.7)	<b>0.0</b> (0.0)
	Elbow Flex	<b>2.3</b> (2.5)	<b>2.0</b> (2.0)	<b>+0.7</b> (0.6)	<b>+1.0</b> (1.0)
	Elbow Extension	<b>2.0</b> (2.0)	<b>2.0</b> (2.0)	<b>+0.7</b> (0.6)	<b>+1.0</b> (1.0)

Note: MMT Scoring: 1) Visible muscle movement, but no movement at the joint, 2) Movement at the joint, but not against gravity, 3) Movement against gravity, but not against added resistance, 4) Movement against resistance, but less than normal, 5) Normal strength  
\*N=3 or \*\*N=2 due to assessment not being performed at some visits for some patients



## Amicus Therapeutics Provides Full-Year 2018 Strategic Outlook and Financial Guidance

*310+ People with Fabry Disease Treated with Reimbursed Galafold® at YE17*

*FY18 Galafold Revenue Guidance of \$75M-\$85M*

*Regulatory Agreement on Comparability of 1,000L Scale Engineering Material for Pompe*

*Additional Pompe Phase 1/2 Clinical Study Results to be Presented at WORLDSymposium™ 2018*

**CRANBURY, NJ, January 8, 2018** — Amicus Therapeutics (Nasdaq: FOLD), a global biotechnology company focused on discovering, developing and delivering novel cutting-edge medicines for rare metabolic diseases, today provided its full-year 2018 strategic outlook and financial guidance.

### Key 2017 Accomplishments

- Exceeded “Target 300” goal with more than 310 people treated with reimbursed Galafold™ (migalastat) oral precision medicine for Fabry disease at year-end 2017. Full-year 2017 Galafold revenue totaled approximately \$36 million.
- Completed global regulatory submissions for migalastat in Japan (J-NDA), the U.S. (NDA), and other key geographies
- Established important clinical proof-of-concept for novel, highly differentiated Pompe treatment regimen ATB200/AT2221 on safety, functional outcomes and key disease biomarkers
- Successfully scaled manufacture of Pompe biologic engineering batches at commercial scale (1,000L) with capacity plans to ensure that entire Pompe population can be served as quickly as possible
- Strengthened balance sheet with total cash, cash equivalents and marketable securities of \$359 million at December 31, 2017 and cash runway into the second half of 2019

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. stated, “During 2017 we continued to build a leading global rare disease biotech company while advancing our vision to maximize the impact of our medicines for people living with rare diseases. We exceeded our EU launch and regulatory objectives for our Fabry precision medicine Galafold, and we reported clinical data from our Pompe clinical study that lays the foundation for a potential new treatment paradigm for this muscle disease. Throughout 2018 we are poised to create significant additional value for patients and shareholders across our key programs in Fabry and Pompe, and through our focused commitment to advancing and expanding a pipeline of novel medicines for rare metabolic diseases.”

Amicus is focused on five key strategic priorities in 2018:

- Double global revenue for Galafold (\$75 million - \$85 million)
- Secure approvals for migalastat in Japan and the U.S.
- Achieve clinical, manufacturing and regulatory milestones to advance ATB200/AT2221 toward global regulatory submissions and approvals as soon as possible
- Develop and expand preclinical pipeline to ensure at least one new clinical program in 2019
- Maintain a strong balance sheet

Mr. Crowley will discuss Amicus’ corporate objectives and key milestones in a presentation at the 36th Annual J.P. Morgan Healthcare Conference on Tuesday, January 9, 2018 at 8:00 a.m. PT (11:00 a.m. ET). A live webcast of the presentation can be accessed through the Investors section of the Amicus Therapeutics corporate web site at <http://ir.amicusrx.com/events.cfm>, and will be archived for 90 days.

### Full-Year 2017 Financial Summary and 2018 Guidance

Amicus recorded approximately \$36 million in full-year 2017 revenue from commercial sales and reimbursed expanded access programs for Galafold. For the full-year 2018 the Company anticipates total Galafold revenue of \$75 million to \$85 million.

Cash, cash equivalents, and marketable securities totaled approximately \$359 million at December 31, 2017. As previously announced, the Company strengthened the balance sheet during 2017 with a \$243.0 million in net proceeds from a follow on public offering in July 2017. The Company expects full-year 2018 net cash spend of between \$230 million and \$260 million. The current cash position is anticipated to fund ongoing operations into at least the second half of 2019.

### Program Highlights

#### **Migalastat for Fabry Disease**

Migalastat is an oral precision medicine intended to treat Fabry disease in patients who have amenable genetic mutations. Regulatory authorities in the European Union, Switzerland, Israel, Canada, Australia, and South Korea have granted full approval for migalastat under the trade name Galafold. The EU approval may serve as the basis for regulatory approvals in more than two-thirds of the global Fabry market that is outside the U.S. In the U.S., Amicus submitted a new drug application (NDA) to the FDA in December 2017 to seek approval of migalastat.

Amicus is committed to advancing the highest quality therapies for all people living with Fabry disease. For people with non-amenable mutations who are not eligible for migalastat as an oral precision medicine, the Company has established initial preclinical proof-of-concept for a novel Fabry ERT (ATB101) co-formulated with migalastat as part of our CHART® platform.

#### Global Fabry Updates:

- 310+ patients (naïve and ERT-switch) on reimbursed Galafold as of December 31, 2017
- Total full-year 2017 revenue of \$36 million from global commercial sales and expanded access programs (EAPs)
- Pricing and reimbursement secured in 15 countries
- Approvals secured in EU, Australia, Canada, Israel, South Korea and Switzerland
- Approvals pending in Japan, U.S. and other key geographies

#### Anticipated Milestones:

- FDA acceptance of U.S. NDA for filing (1Q18)
- Regulatory decision on Japanese J-NDA (1H18)
- Total full-year 2018 revenue guidance of \$75 million to \$85 million
- ATB101 co-formulated with migalastat advancing toward the clinic in 2019

#### **ATB200/AT2221 for Pompe Disease**

ATB200/AT2221 is a novel treatment paradigm that consists of ATB200, a unique recombinant human acid alpha-glucosidase (rhGAA) enzyme with optimized carbohydrate structures, particularly mannose-6 phosphate (M6P), to enhance uptake, co-administered with AT2221, a pharmacological chaperone. Throughout 2017, Amicus presented a cascade of positive data from an ongoing global Phase 1/2 clinical study (ATB200-02) to evaluate safety, tolerability, pharmacokinetics (PK), and pharmacodynamics (PD) of ATB200/AT2221 across ambulatory ERT-switch patients (Cohort 1), non-ambulatory ERT-switch patients (Cohort 2), and ERT-naïve patients (Cohort 3).

Amicus continues to assemble the highest quality data package and to scale up manufacturing to meet the needs of the Pompe community. The Company is in the midst of a series of collaborative discussions with U.S. and EU regulators regarding the best and fastest pathway forward for this novel treatment option, and continues to anticipate a Pompe regulatory pathway update in the first half of 2018.

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#### Pompe Program Updates:

- Data collection underway in a retrospective natural history study (POM-002)
- Prospective observational study (POM-003) initiated
- GMP production of ATB200 commenced at the large commercial scale (1,000 Liters)
- FDA agreement reached on comparability between 250L scale and 1000L engineering batches
- FDA agreement reached on testing strategy for demonstrating comparability between 250L scale and 1000L GMP batches

#### Anticipated Upcoming Pompe Program Milestones:

- Expansion of ongoing ATB200-02 clinical study to include four to six additional ambulatory ERT-switch patients
- Additional data from ATB200-02 clinical study at 14<sup>th</sup> Annual *WORLDSymposium*<sup>™</sup> (February 5-9, 2018)
- Final regulatory agreement on manufacturing comparability between 1,000L and 250L GMP scale
- Completion and release for clinic of 1,000L GMP commercial scale material
- Pompe regulatory pathway update (1H18)
- Initiation of larger registration-directed study

#### **EU Important Safety Information**

Treatment with GALAFOLD should be initiated and supervised by specialists experienced in the diagnosis and treatment of Fabry disease. GALAFOLD is not recommended for use in patients with a nonamenable mutation.

- GALAFOLD is not intended for concomitant use with enzyme replacement therapy.
- GALAFOLD is not recommended for use in patients with Fabry disease who have severe renal impairment (<30 mL/min/1.73 m<sup>2</sup>). The safety and efficacy of GALAFOLD in children 0—15 years of age have not yet been established.

- No dosage adjustments are required in patients with hepatic impairment or in the elderly population.
- There is very limited experience with the use of this medicine in pregnant women. If you are pregnant, think you may be pregnant, or are planning to have a baby, do not take this medicine until you have checked with your doctor, pharmacist, or nurse.
- While taking GALAFOLD, effective birth control should be used. It is not known whether GALAFOLD is excreted in human milk.
- Contraindications to GALAFOLD include hypersensitivity to the active substance or to any of the excipients listed in the PRESCRIBING INFORMATION.
- It is advised to periodically monitor renal function, echocardiographic parameters and biochemical markers (every 6 months) in patients initiated on GALAFOLD or switched to GALAFOLD.
- OVERDOSE: General medical care is recommended in the case of GALAFOLD overdose.
- The most common adverse reaction reported was headache, which was experienced by approximately 10% of patients who received GALAFOLD. For a complete list of adverse reactions, please review the SUMMARY OF PRODUCT CHARACTERISTICS.
- Call your doctor for medical advice about side effects.

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).

### **About Amicus Therapeutics**

Amicus Therapeutics (Nasdaq: FOLD) is a global, patient-centric biotechnology company focused on discovering, developing and delivering novel high-quality medicines for people living with rare metabolic diseases. The cornerstone of the Amicus portfolio is migalastat, an oral precision medicine for people living with Fabry disease who have amenable genetic mutations. Migalastat is currently approved under the trade name Galafold™ in the European Union, with additional approvals granted and pending in several geographies. The future value driver of the Amicus pipeline is ATB200/AT2221, a novel, late-stage, potential best-in-class treatment paradigm for Pompe disease. The Company is committed to advancing and expanding a robust pipeline of cutting-edge, first- or best-in-class medicines for rare metabolic diseases.

### **Forward-Looking Statements**

This press release 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

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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, 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 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, 2016 and Quarterly Report on Form 10-Q for the quarter ended September 30, 2017. 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.

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