## 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 12, 2015

### 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:

- o Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- o Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- o Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- o Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

### 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 12, 2015, the Company filed a press release, a copy of which is attached to this Current Report as Exhibit 99.2.

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### Item 9.01. Financial Statements and Exhibits.

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

Number	Description		
99.1 99.2	Presentation Materials Press Release dated January 12, 2015		

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 12, 2015

s /s/ William D. Baird III

William D. Baird III Chief Financial Officer

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### EXHIBIT INDEX

Exhibit Number		Description
99.1 99.2	Presentation Materials Press Release dated January 12, 2015	
		5



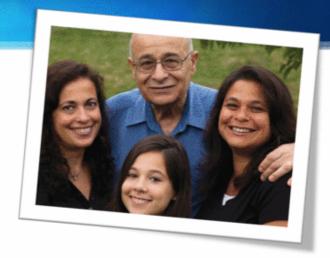
### Safe Harbor

This presentation contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to business, operations and financial conditions of Amicus including but not limited to preclinical and clinical development of Amicus' candidate drug products, cash runway, and the timing and reporting of results from clinical trials evaluating Amicus' candidate drug products. Words such as, but not limited to, "look forward to," "believe," "expect," "anticipate," "estimate," "intend," "plan," "would," "should" and "could," and similar expressions or words, identify forward-looking statements. Although Amicus believes the expectations reflected in such forward-looking statements are based upon reasonable assumptions, there can be no assurance that its expectations will be realized. Actual results could differ materially from those projected in Amicus' forwardlooking statements due to numerous known and unknown risks and uncertainties, including the "Risk Factors" described in our Annual Report on Form 10-K for the year ended December 31, 2013. All forward-looking statements are qualified in their entirety by this cautionary statement, and Amicus undertakes no obligation to revise or update this presentation to reflect events or circumstances after the date hereof.



## Company Mission





Amicus Therapeutics is a biopharmaceutical company at the forefront of developing next-generation medicines to treat a range of rare and orphan diseases, with a focus on improved therapies for Lysosomal Storage Disorders



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### Amicus Value Proposition

Building a Leading Global Rare Disease Company to Transform Lysosomal Storage Disease (LSD) Treatment Paradigm

Fabry franchise, led by novel pre-commercial asset for patients with amenable mutations

Next-generation Pompe ERT to improve uptake and tolerability

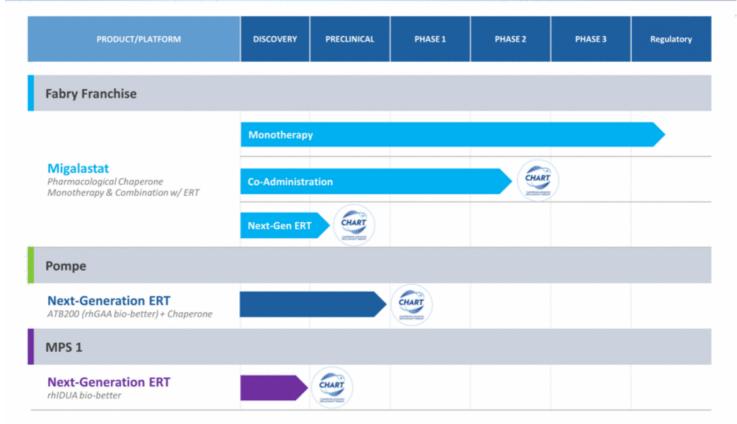
Multiple platform technologies to address current ERT limitations

Financial strength to develop and deliver improved therapies to patients

Experienced leadership team



## Advanced Product Pipeline





## Amicus R&D Engine: Multiple Technology Platforms





## Fabry Disease Overview

## Fatal Lysosomal Storage Disease with Significant Unmet Needs Despite Existing Therapies

- Deficiency of α-Gal A leading to GL-3 accumulation
- >800 known mutations
- Symptoms include pain, gastrointestinal problems, angiokeratomas
- Cardiovascular disease, renal failure, and stroke are leading causes of morbidity and mortality

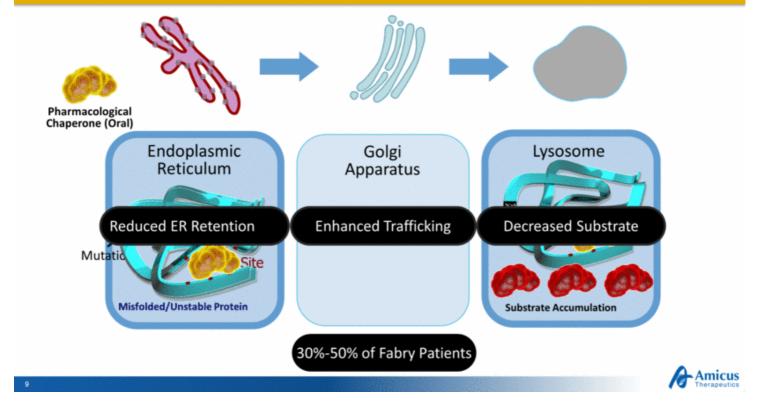


Amicus



## Chaperone Monotherapy: Personalized Medicine Approach

Unique Mechanism of Action with Orally Bioavailable Small Molecule for Fabry Patients with Amenable Mutations

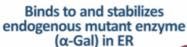


### Fabry Franchise

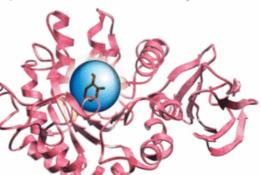
### Migalastat is Designed to Stabilize a Patient's Own Enzyme or an Infused ERT



**Chaperone + ERT** (Non-Amenable Mutations)







Binds to and stabilizes exogenous enzyme (ERT) in circulation



Increases trafficking and lysosomal levels of active enzyme

Increases tissue uptake and lysosomal levels of active enzyme



**LOWERED SUBSTRATE** 



## Migalastat Monotherapy Experience for Fabry

### 91 Patients Today Take Migalastat as Only Therapy for Fabry Disease<sup>1</sup>



Total patients who have ever taken migalastat:

143

Patients taking migalastat today as only therapy:

91

Total patient years of therapy:

411

Average retention rate into next study:

96%

Maximum years on therapy:

9.0

Information as of January 2015. All patients are receiving investigational drug, migalastat HCl, as part of ongoing clinical trials
\*Retention defined as # of patients who completed a study and chose to enter extension, e.g., Study 01112-mo into 24-mo extension



## Two Successful Global Registration Studies

Positive Results Support Global Approvals of Migalastat for Patients with Amenable Mutations

Data in ERT-naïve (Study 011) and ERT switch (Study 012) patients show:

Reduction in disease substrate

Stability of kidney function

Reduction in cardiac mass (LVMi)

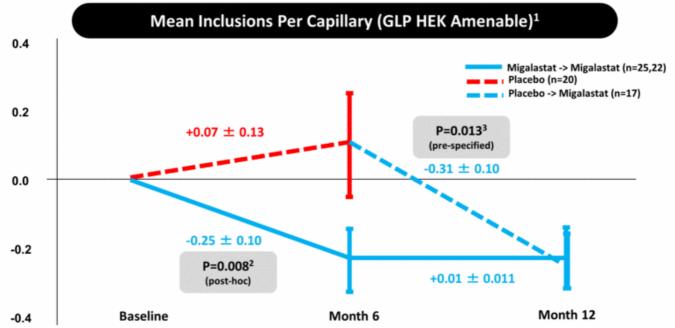
Generally safe and well tolerated

Marketing submissions planned in 2015



## Phase 3 (Study 011) Primary Efficacy Analysis

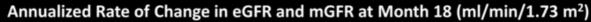
Statistically Significant Reduction in Disease Substrate (Kidney IC GL-3)\*

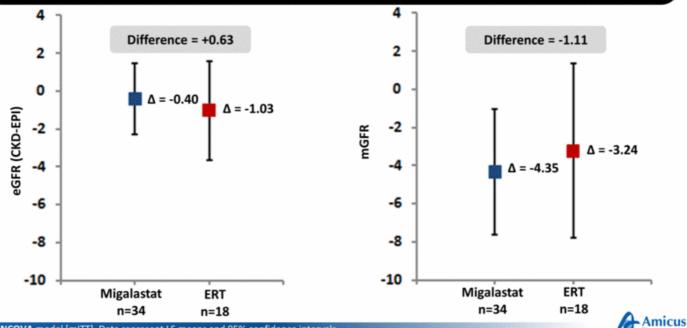


"All patients with evaluable paired biopsies and amenable GLA mutations in GLP-validated HEK assay – post hoc at month 6 and pre-specified at month 12 ¹Data points are baseline corrected; represent mean ± standard error (SEM) change from baseline in the mean number of GL-3 inclusions per capillary after 6 months of treatment with migalastat or placebo. ³Analysis of covariance (ANCOVA) model with covariate adjustment for baseline value and factors for treatment group and treatment by baseline interaction. P-value corresponding to least-square mean difference between migalastat and placebo is displayed. ³MMRM Pbo change M6 to M12.

## Phase 3 (Study 012) Primary Efficacy Analysis

Met Co-Primary Endpoints Showing Comparability of Kidney Function in Patients Switched from ERT to Migalastat

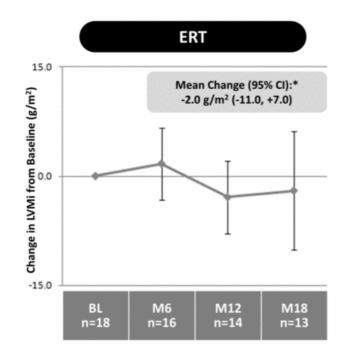


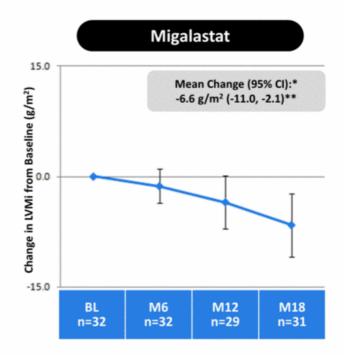


<sup>1</sup> ANCOVA model [mITT]. Data represent LS means and 95% confidence intervals

## Phase 3 (Study 012) Cardiac Data

Reductions in LVMi Observed in Patients Switched from ERT Through Month 18 \*



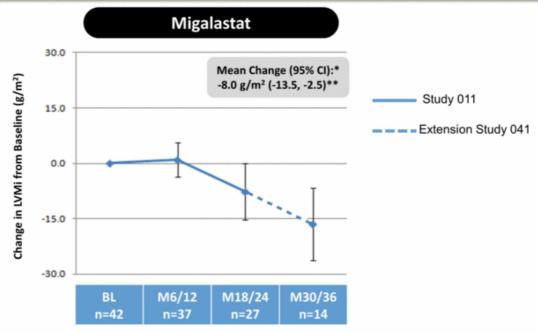


<sup>\*</sup>Mean change to month 18 (mITT; amenable mutations) \*\*Statistically significant (95% CI does not overlap zero)



## Phase 3 (Study 011+041) Cardiac Data

New Data Show Migalastat Has Persistent and Increasing Positive Effect on LVMi Over Longer Periods of Time (Up to 36 Months)



<sup>\*</sup>Mean change to last available time point (average 22 months) in all patients with amenable mutations with baseline and post-baseline values.

Sample size differences due to subjects not yet reaching a given timepoint or due to missing Echos

Note: Mean and 95% confidence intervals on change from baseline are plotted

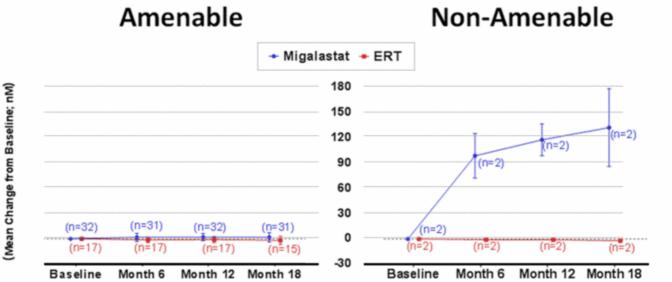


<sup>\*\*</sup>Statistically significant (95% CI does not overlap zero)

# Phase 3 Validation of Personalized Medicine Approach

Lyso-Gb3 Data Validate Pharmacogenetic Approach to Identify Patients Who Respond to Migalastat



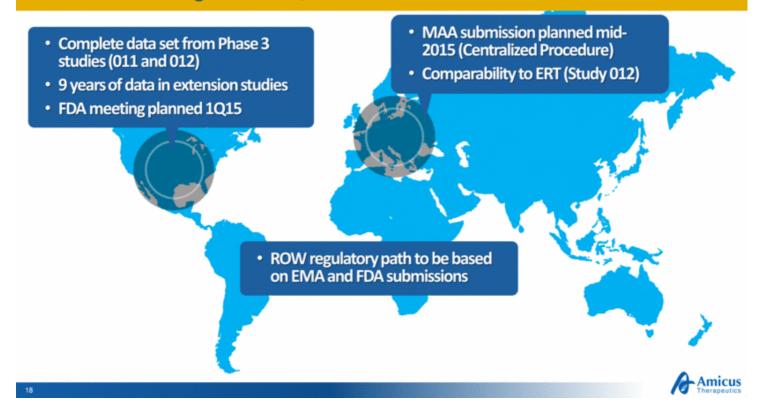




## Global Regulatory Strategy

MAA Submission on Track for Mid-2015

FDA Meeting Planned 1Q15 to Discuss Fastest Path to NDA Submission



## Fabry Commercial Opportunity

### Significant Commercial Opportunity with Large and Growing ~\$1B Market Today

Undiagnosed Patients

Diagnosed Untreated Patients

ERT-Treated Pts

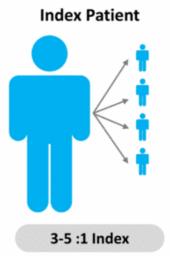
- \$993M in FY13 global ERT sales (Fabrazyme and Replagal)
- 5-10K diagnosed WW (51% female/49% male¹)
- <50% of diagnosed patients are currently treated with ERT</p>
- 30-50% of Fabry patients with amenable mutations



## Significant Underdiagnosis of Fabry Disease

Large Number of Patients Identified Through Newborn Screening Suggests Fabry Could Be One of the More Prevalent Human Genetic Diseases

Newborn Screening Study	# Newborns Screened	# Confirmed Fabry Mutations	% Amenable
Burton, 2012, US	8,012	7 [1: ~1100]	TBD
Mechtler, 2011, Austria	34,736	9 [1: ~3,800]	100%
Hwu, 2009, Taiwan	171,977	75 [1: ~2300]	75%
Spada, 2006, Italy	37,104	12 [1: ~3100]	86%
Historic published incidence		1:40,000 to 1	:60,000



### **Majority of Newly Diagnosed Patients Have Amenable Mutations**

Burton, LDN WORLD Symposium, 2012 Feb. Mechtler et al., The Lancet, 2011 Dec. Hwu et al., **Hum Mutation**, 2009 Jun Spada et al., **Am J Human Genet.**, 2006 Jul



## Fabry Franchise Strategy

### **Our Vision is to Develop Next Generation Therapies for All Fabry Patients**

### **Amenable Mutations**

### **Non-Amenable Mutations**

Migalastat Monotherapy

Migalastat Co-Administration Migalastat Co-Formulation

Product

Novel small molecule chaperone

Advantages

Oral therapy, broad tissue distribution

2015 Milestones EU and US marketing applications

Chaperone + marketed ERT; labelexpansion

Stabilized ERT for better targeting and tissue uptake

Ph 2/3 study start

Chaperone + nextgeneration ERT

Optimized and stabilized ERT for max tissue uptake

Cell line optimization



## Key Milestones – Fabry Franchise

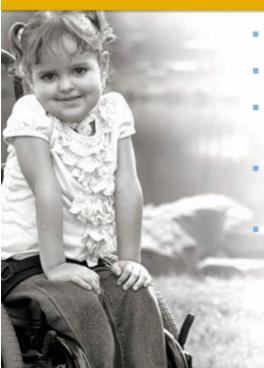
Timing	Milestone	
1Q15	Additional 011 and Phase 2 extension data	
1Q15	Scientific Presentations at LDN WORLD	
1Q15	FDA Regulatory Interaction	
Mid-2015	MAA Submission	
2H15	Phase 2 Co-Administration Study Initiation	
2H15	Internal Development of Next-Gen ERT Cell Line	



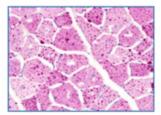


## Pompe Disease Overview

## Severe, Fatal, Progressive Neuromuscular Disease with Significant Unmet Need Despite Availability of ERT



- Deficiency of GAA leading to glycogen accumulation
- Age of onset ranges from infancy to adulthood
- Symptoms include muscle weakness, respiratory failure and cardiomyopathy
- Respiratory and cardiac failure are leading causes of morbidity and mortality
- Incidence 1:28,000<sup>1</sup>



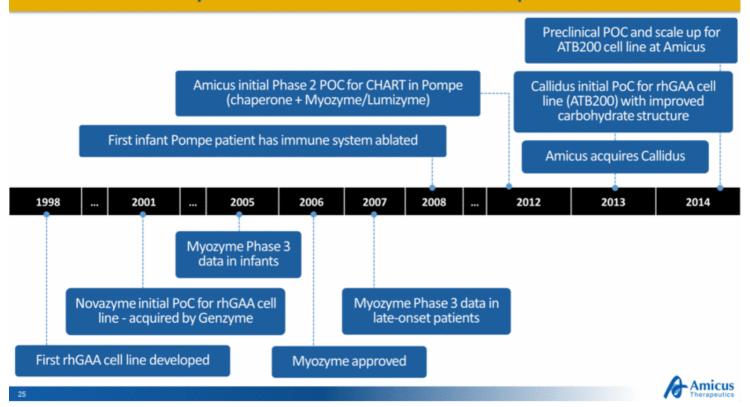
Elevated Glycogen in Muscle

Amicus

Evidence Report - Newborn Screening for Pompe Disease - June 2013 - HRSA.gov

## Select Milestones in Pompe Drug Development

A Decade After Initial Clinical Studies of Myozyme, Researchers Still Working to Develop Next-Generation Treatment for Pompe Patients



## **Current Pompe ERT Limitations**

### Significant Unmet Needs Remain Due to Limitations of First-Generation Pompe ERT

"...Biologic drugs, including enzyme-replacement therapies, can elicit anti-drug Abs (ADA) that may interfere with drug efficacy and impact patient safety." – Journal of Immunology 2014

"...recurrent injections of rhGAA during ERT can elicit high titer antibody formation against GAA; this reduces the efficacy of ERT and may prompt infusion associated reactions (IAR) that may be lifethreatening." – Doerfler, et al. WORLD 2014

### The NEW ENGLAND JOURNAL of MEDICINE

"...14% of pts on [Lumizyme] treatment have declining 6-minute walk test and 36% have declining forced vital capacity." - van der Ploeg, et al. 2010



"All 18 patients who enrolled in the initial [infantile-onset Pompe] study survived significantly longer and with fewer ventilation events ... However, morbidity and mortality remain substantial, with a 28% mortality rate and a 51% invasive ventilation rate at age 36 months." - Kishnani, et al. 2009



## Three Challenges with Pompe ERT Today

Activity/ Stability

Rapid denaturation of ERT in pH of blood<sup>1</sup>

Protein Aggregation

Tolerability / Immunogenicity Infusion-associated reactions in >50% of late-onset patients<sup>3</sup>

Antibody titers shown to affect treatment outcomes<sup>4,5</sup>

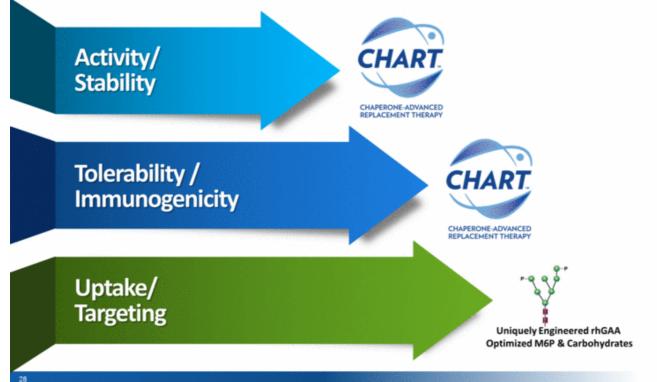
Uptake/ Targeting Low M6P receptor uptake into skeletal muscle<sup>2</sup> Vast majority of rhGAA not delivered to lysosomes<sup>2</sup>

<sup>1</sup>Khanna *et al.*, **PLoS ONE**, 2012; <sup>2</sup>Zhu *et al.*, Amer. Soc. Gene Therapy, 2009 June; <sup>3</sup>Banati *et al.*, **Muscle Nerve**, 2011 Dec.; <sup>4</sup>Banugaria *et al.*, **Gen. Med.**, 2011 Aug.; <sup>5</sup>de Vries *et al.*, **Mol Genet Metab.**, 2010 Dec.



## Amicus Biologics Platform Technologies

Multiple Complementary Amicus Platform Technologies Address The Challenges with Existing ERTs Today





## **Amicus Biologics Capabilities**

## ATB200 Successfully Manufactured at Clinical Scale While Maintaining Optimized Carbohydrate Structure



- Cell line scaled to 250 L
- 2 engineering batches completed in 2014
- IND-enabling tox underway



# Amicus Pompe ERT: Highly Differentiated Approach

Amicus to advance ATB200 + Chaperone into Phase 2 in 2015 **Potential Solution for Key ERT Limitations** 

Pompe ERT Challenges	IGF2-GAA	Neo-GAA	ATB200 + Chaperone
Stability & Activity			(Chaperone)
Targeting & Uptake	(IGF2 Tag)	(M6P)	<b>√ √</b> (M6P)
Tolerability & Immunogenicity			(Chaperone)
Development Stage	Phase 3	Phase 1	Late Preclinical



## Pompe: Multiple Milestones to Clinic

Timing	Milestone
1Q15	Initiate GMP Batch
3Q15	Tox Studies
Mid-2015	Pre-IND Meeting
2H15	Phase 1/2 study initiation



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## Financial Summary

### **Strong Balance Sheet to Fund Operations into 2017**

Financial Position	Dec. 31, 2014		
Cash:	\$169.1M		
2015 Net Cash Spend Guidance:	\$73M-83M		
Capitalization			
Shares Outstanding:	95,556,277		







#### Amicus Therapeutics Provides Full-Year 2015 Strategic Outlook and Financial Guidance

### MAA Submission for Migalastat Monotherapy for Fabry Disease on Track for Mid-2015

Next-Generation Pompe ERT Set to Enter Clinic in 2H15

FY15 Cash Spend Guidance of \$73-\$83 Million — Current Cash Expected to Fund Operations into 2017

**CRANBURY, NJ, January 12, 2015** — Amicus Therapeutics (Nasdaq: FOLD), a biopharmaceutical company at the forefront of therapies for rare and orphan diseases, today provided its full-year 2015 strategic outlook and financial guidance.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc., stated, "2014 was a transformational year for Amicus. We enter 2015 with great momentum and equally high expectations. We expect many important firsts for Amicus in 2015 including our first regulatory submissions for our novel oral Fabry monotherapy as well as our first human clinical study of our highly differentiated, proprietary next-generation ERT for Pompe disease. These milestones will significantly advance our goal of building one of the world's leading biotechnology companies focused on the treatment of rare and orphan diseases and improving the lives of people living with rare disorders around the world. I am very optimistic and look forward to leading Amicus for many years to come as we fulfill this great vision."

### **Key 2015 Highlights**

- · Migalastat monotherapy FDA interaction planned in 1Q15. MAA submission on track for mid-2015 to seek marketing approval for Fabry patients with amenable mutations.
- · Initiation of longer-term Phase 2 Fabry co-administration study anticipated in 2015 in support of Fabry franchise strategy to develop migalastat in combination with ERT for Fabry patients with non-amenable mutations.
- · Next-generation Pompe ERT (ATB200 + chaperone) set to enter clinic in 2H15. Additional details on Pompe program to be highlighted at 33rd Annual J.P. Morgan Healthcare Conference.
- Strong balance sheet supports current development programs, biologics manufacturing capabilities, and initial commercial infrastructure to establish leading global rare disease company
  - · Full-year 2015 net cash spend expected to range between \$73 million and \$83 million
  - · Current cash (\$169.1 million at December 31, 2014) projected to fund operating plan into 2017

Mr. Crowley will discuss Amicus' corporate objectives and key milestones in a presentation at the 33rd Annual J.P. Morgan Healthcare Conference on Tuesday, January 13, 2015 at 10:30 a.m. PT (1:30 p.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.amicustherapeutics.com/events.cfm, and will be archived for 90 days.

#### **Highlights and Milestones by Program**

#### **Fabry Franchise**

Amicus is preparing to submit marketing applications for the oral pharmacological chaperone migalastat HCl ("migalastat") as a monotherapy for Fabry patients who have amenable mutations. Positive Phase 3 data in both treatment naïve and ERT switch patients have shown that treatment with migalastat has resulted in reductions in disease substrate, stability of kidney function and improvement in a key cardiac parameter (left ventricular mass index, or LVMI) in patients with amenable mutations. For all other Fabry patients who do not have amenable mutations and cannot take monotherapy, Amicus is advancing migalastat in combination with ERT.

### Anticipated 2015 Fabry Franchise Milestones:

- · FDA meeting to discuss migalastat monotherapy in 1Q15
- · Migalastat monotherapy MAA submission in mid-2015
- $\cdot \quad \text{Initiation of longer-term Phase 2 study of oral migal a state co-administered with currently marketed ERTs in 2H15}$
- · Internal development underway of next-generation ERT (bio-better Fabry ERT cell line for co-formulation with migalastat)

### Next-Generation ERT for Pompe Disease (ATB200 + Chaperone)

Amicus is leveraging its biologics capabilities to develop a bio-better, uniquely engineered rhGAA enzyme with an optimized carbohydrate structure (designated ATB200) to enhance uptake, in combination with a pharmacological chaperone to improve activity and tolerability. In preclinical studies ATB200 has demonstrated greater enzyme uptake into tissues and further substrate reduction compared to the current standard of care Pompe ERT (Myozyme/Lumizyme). Clinical studies of pharmacological chaperones in combination with currently marketed ERTs have established initial human proof-of-concept that a chaperone can stabilize enzyme activity and potentially improve ERT tolerability.

### Anticipated 2015 Pompe Program Milestones:

- · GMP batch of ATB200 in 1Q15
- · Pre-IND meeting in mid-2015
- · Phase 1/2 study initiation in 2H15

### 2015 Financial Guidance

Cash, cash equivalents, and marketable securities totaled \$169.1 million at December 31, 2014 compared to \$82.0 million at December 31, 2013. The Company's balance sheet was strengthened during 2014 with a \$40 million at-the-market (ATM) financing as well as a \$103.5 million public offering. Amicus expects full-year 2015 net cash spend between \$73 million and \$83 million. The current cash position is projected to fund operations into 2017.

#### **Company Leadership**

Amicus also announced that Bradley L. Campbell has been promoted to President and Chief Operating Officer. Mr. Campbell will continue to report to Mr. Crowley and will also continue to oversee a broad range of activities at Amicus including: Research and Development; Technical (Manufacturing) Operations; Program Management; and Patient Advocacy and Public Policy. He was previously named Chief Operating Officer in 2013 and prior to that he has held a number of positions at Amicus, including Chief Business Officer.

#### **About Amicus Therapeutics**

Amicus Therapeutics (Nasdaq:FOLD) is a biopharmaceutical company at the forefront of therapies for rare and orphan diseases. The Company is developing novel, first-in-class treatments for a broad range of human genetic diseases, with a focus on delivering new benefits to individuals with lysosomal storage diseases. Amicus' lead programs in development include the small molecule pharmacological chaperone migalastat as a monotherapy for Fabry disease, as well as next-generation enzyme replacement therapy (ERT) products for Fabry disease, Pompe disease, and MPS-1.

### **Forward-Looking Statements**

This press release contains, and the accompanying conference call will contain, "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to preclinical and clinical development of Amicus' candidate drug products, the timing and reporting of results from preclinical studies and clinical trials evaluating Amicus' candidate drug products, financing plans, and the projected cash position for the Company. Words such as, but not limited to, "look forward to," "believe," "expect," "anticipate," "estimate," "intend," "potential," "plan," "targets," "likely," "may," "will," "would," "should" and "could," and similar expressions or words identify forward-looking statements. Such forward-looking statements are based upon current expectations that involve risks, changes in circumstances, assumptions and uncertainties. The inclusion of forward-looking statements should not be regarded as a representation by Amicus that any of its plans will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong. They can be affected by inaccurate assumptions Amicus 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 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 the business of Amicus, including, without limitation: the potential that results of clinical or pre-clinical 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 may not grant or may delay approval for our product candidates; the potential that preclinical and clinical studies could be delayed because we identify serious side effe

all of our studies and, our dependence on third parties in the conduct of our clinical studies. 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 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, 2013. 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 Amicus undertakes no obligation to revise or update this news release to reflect events or circumstances after the date hereof. This caution is made under the safe harbor provisions of Section 21E of the Private Securities Litigation Reform Act of 1995.

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