

**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 7, 2013**

**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))

**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 7, 2013, 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.

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**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 7, 2013

By: /s/ PETER M. MACALUSO  
Name: Peter M. Macaluso  
Title: Secretary

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**EXHIBIT INDEX**

<b>Exhibit No.</b>	<b>Description</b>
99.1	Presentation Materials
99.2	Press Release dated January 7, 2013



## Corporate Overview

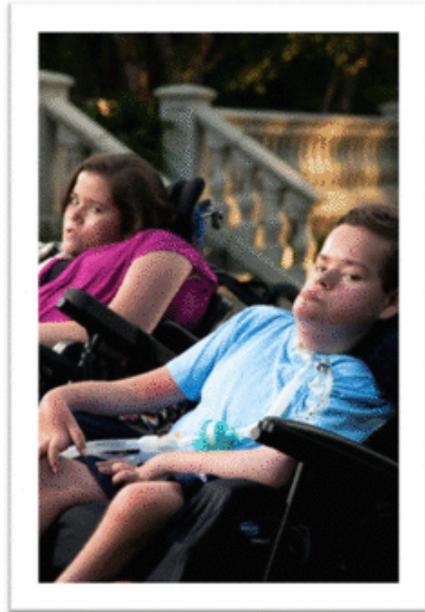
*At the Forefront of Therapies for Rare and Orphan Diseases™*

January 2013

### 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, 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’ forward-looking statements due to numerous known and unknown risks and uncertainties, including the “Risk Factors” described in our Quarterly Report on Form 10-Q for the quarter ended June 30, 2012. 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.



*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*

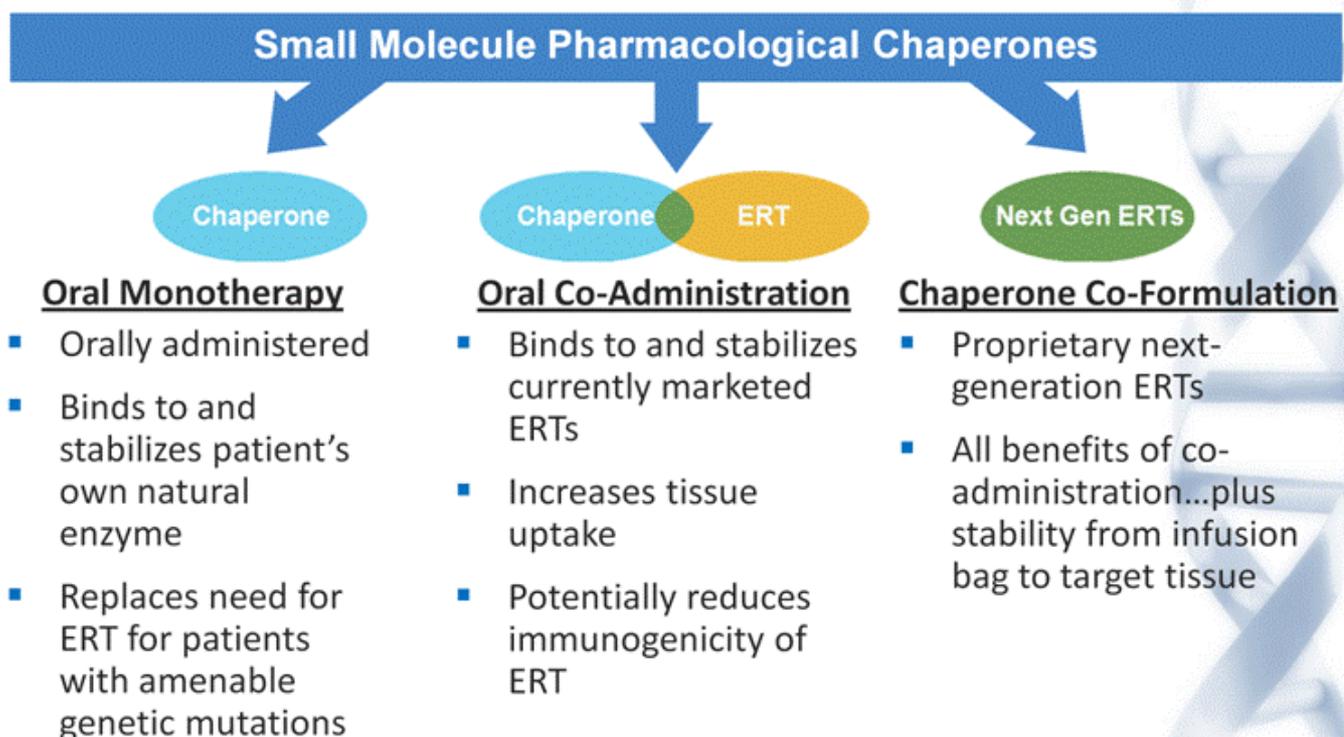
## Key Messages

- Phase 3 Fabry monotherapy study (Study 011) ongoing:
  - Top-line 6-month (Stage 1) results encouraging and consistent with Phase 2 experience
  - FDA to consider “entirety of data” from both 6 and 12 months
- Pompe Chaperone-ERT co-administration repeat-dose clinical study to begin 3Q13
- Fabry Chaperone-ERT co-formulated product advancing towards clinic
- Proprietary Pompe next-generation ERT in preclinical development
- ~\$100M cash at 12/31/12

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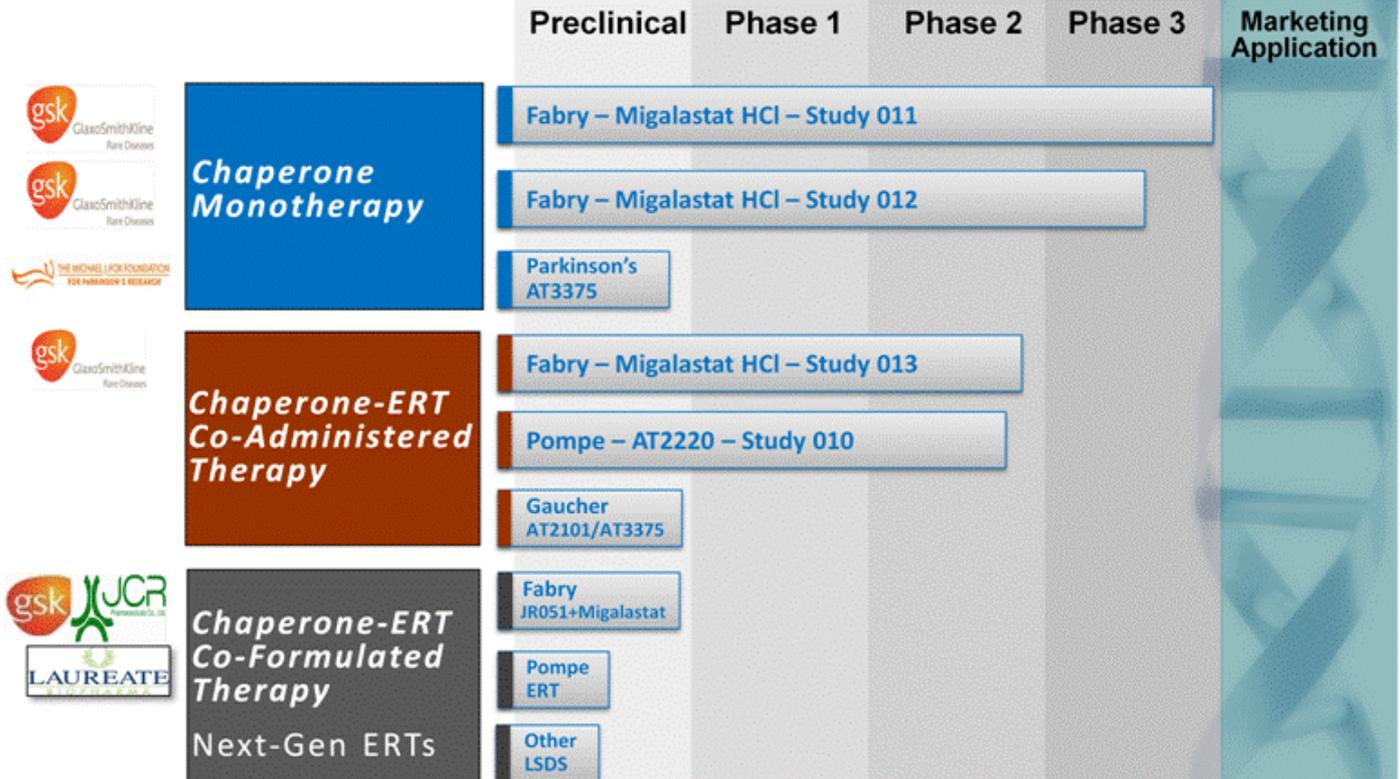
## Core Technology and Focus

### Potential to Transform LSD Treatments



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## One Technology, Three Novel Applications



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## 2013 Investment Highlights

### Products

- Migalastat HCl monotherapy: encouraging Phase 3 (Stage 1) and Phase 2 extension study results
- First-in-man Phase 2 results in Fabry and Pompe Chaperone-ERT Co-Administration

### Platform Technology

- Next-Generation ERTs in development
- Multiple potential therapeutic enhancements, including novel routes of delivery

### Partnerships

- GSK  
 "Amicus & GSK are committed to advancing Migalastat HCl monotherapy in Fabry"  
 >\$137M invested capital in Amicus & Migalastat HCl
- JCR  
 Fabry Chaperone-ERT co-formulation (Amicus, GSK and JCR)
- LAUREATE  
 Pompe biologics CMO

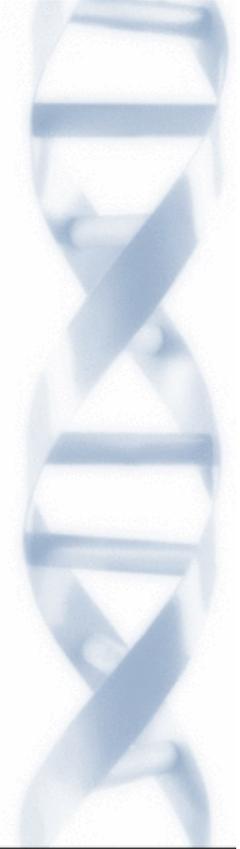
### Financial Strength

- ~\$99.1M cash (12/31/12)
- GSK responsible for 60% of all Fabry development costs
- Projected cash runway 18-24 months at current burn rates
- Multiple catalysts next 12-24 months

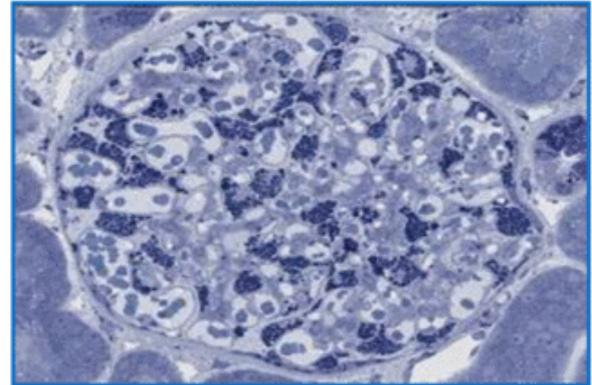
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# PHARMACOLOGICAL CHAPERONES

MONOTHERAPY DEVELOPMENT  
IN FABRY DISEASE



- Progressive, multi-system lysosomal storage disease
- Caused by inherited GLA mutations
- X-linked disease
- Mortality due to renal failure, cardiac failure, stroke
- 5 – 10K patients diagnosed WW (51% female/49% male\*)
- Significantly under-diagnosed



Kidney GL-3

\*Fabry Registry 2011

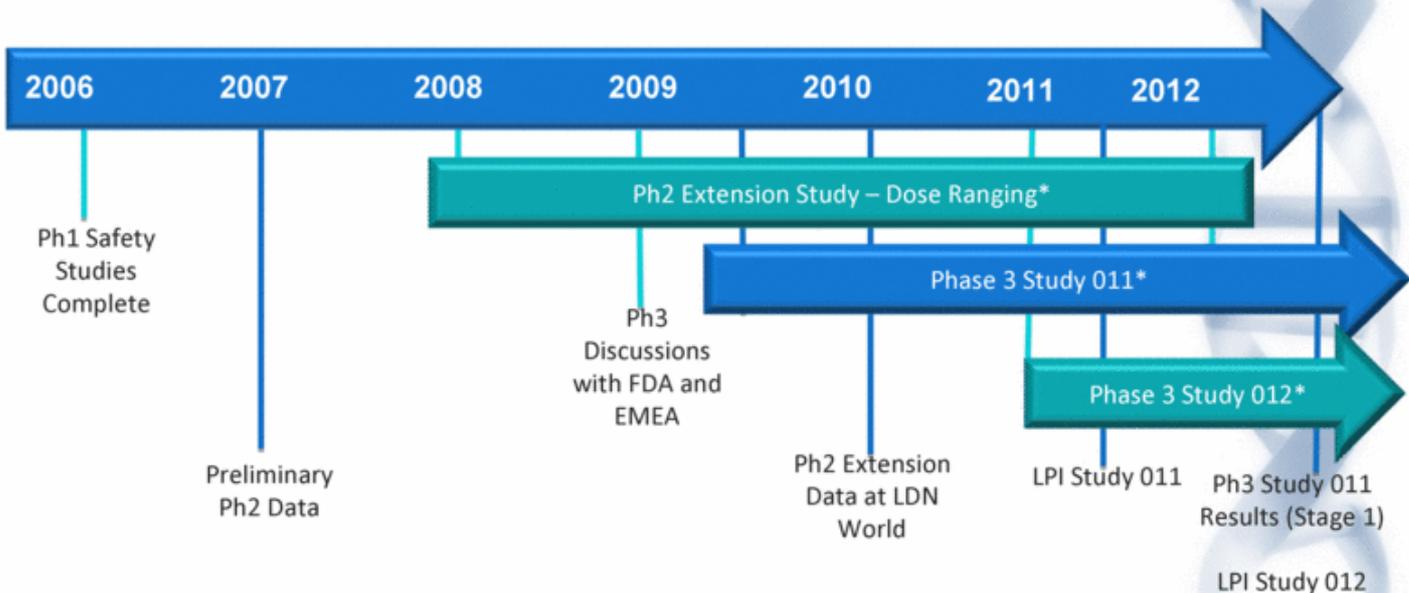
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## Migalastat HCl Monotherapy for Fabry Disease

### Development History

#### Jan 2013 Highlights

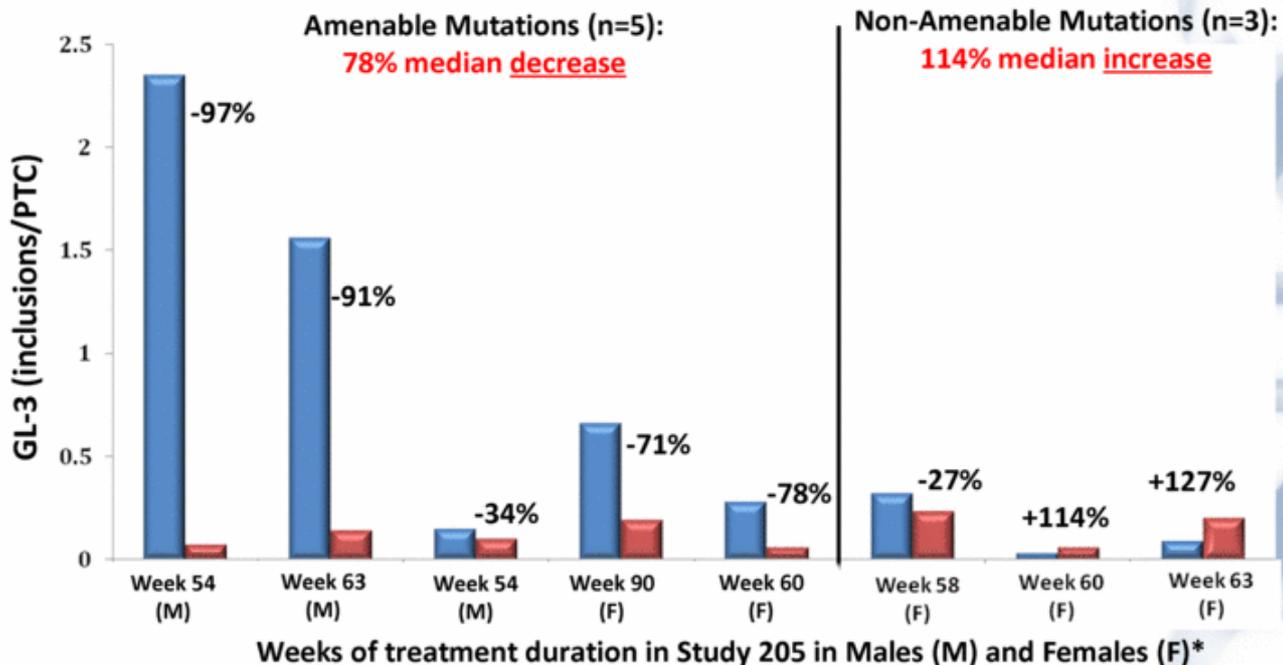
- 102 Fabry patients WW on Migalastat HCl monotherapy as only therapy for Fabry
- >220 patient-years of data
- 57 of 59 pts completed 12 months of Study 011 and elected to continue in long-term extension studies



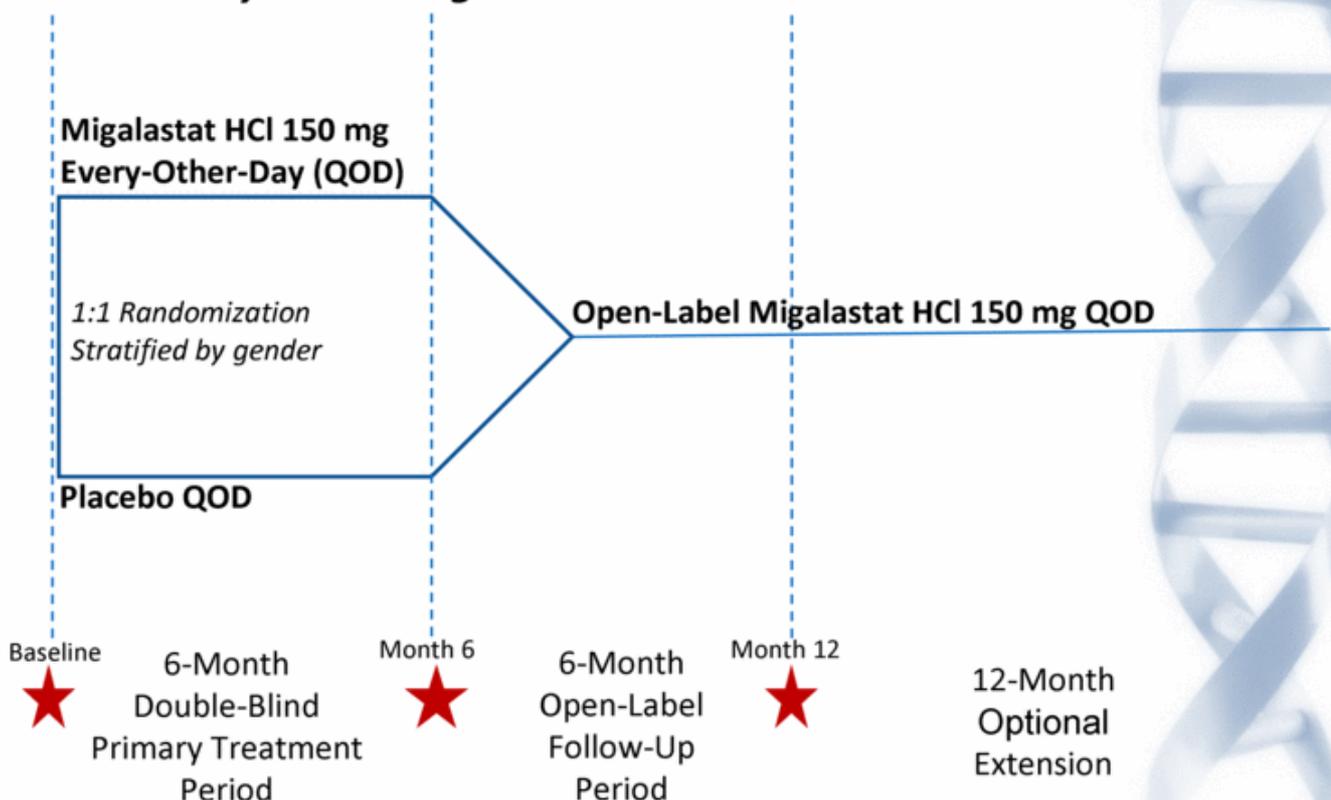
\*Open-label extension studies ongoing in patients who completed Ph2 extension study and Ph3 studies

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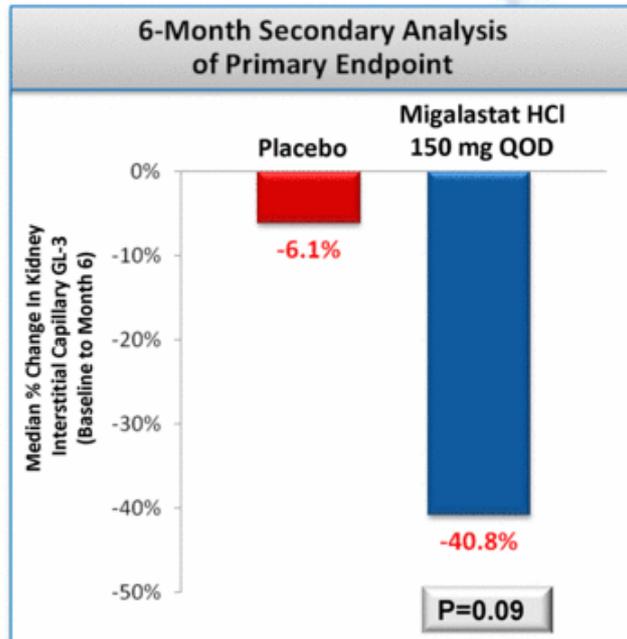
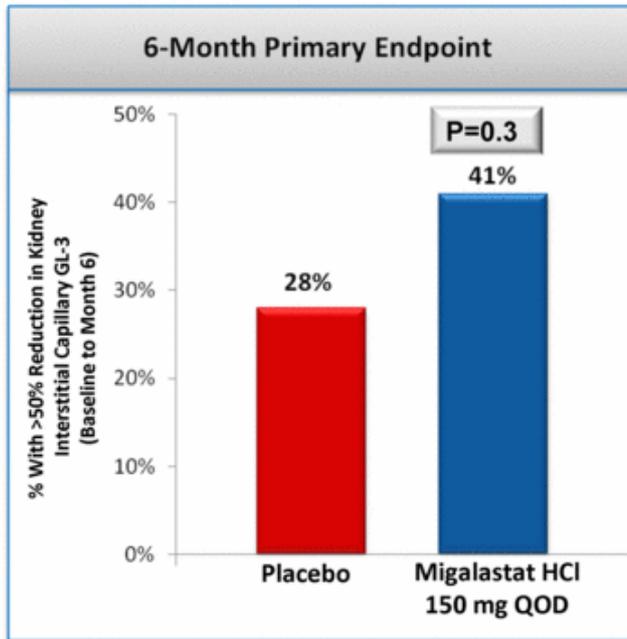
**Median 78% Decrease in Kidney GL-3  
Observed in Study Patients with Amenable Mutations**



\*All patients in Study 205 completed initial Phase 2 studies of migalastat HCl monotherapy



**Kidney Interstitial Capillary GL-3 – Surrogate Biomarker  
Considered Likely to Predict Clinical Benefit in Fabry Patients**



## PDUFA V Impact

### Statement on FDA User Fee Agreements: Strengthening FDA and the Medical Products Industry for the Benefit of Patients (March 29, 2012)



*“FDA assesses the benefit-risk of new drugs on a case-by-case basis, considering the degree of unmet need and the severity and morbidity of the condition the drug is intended to treat.*

*This approach has been critical to increasing patient access to new drugs for cancer and rare and other serious diseases, where existing therapies have been few and limited in their effectiveness.”*

**Janet Woodcock, M.D.**  
Director, CDER U.S. FDA

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## Migalastat HCl Monotherapy for Fabry Disease

### Regulatory Guidance and Path Forward

#### Study 011 is an Ongoing 12-Month Pivotal Study of Migalastat HCl in Patients with Fabry Disease with Amenable Mutations

#### U.S. FDA Feedback

- 6-month analysis is Stage 1
- FDA to consider Stage 1 and Stage 2 (12-month) data for NDA submission
- FDA will evaluate efficacy and safety based on entirety of Study 011 data (no single endpoint will be determinative)
- FDA meeting anticipated in mid-2013 to discuss US approval pathway



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## Study 011 12-Month Analysis Plans

### 12-Month Descriptive Comparisons – Results Anticipated 1H13

#### Study 011 Design (1:1 Randomization)

Study Arm	Stage 1: Month 0-6	Stage 2: Month 6-12*
Placebo	Placebo	<span style="border: 1px solid red; padding: 2px;">switch</span> Migalastat HCl
Treatment	Migalastat HCl	Migalastat HCl

- Placebo arm Stage 1 vs. Stage 2 (migalastat HCl 6 months vs. placebo 6 months)
- Treatment arm Stage 1 vs. Stage 2 (migalastat HCl 12 months vs. 6 months)
- Treatment arm Stage 1 + placebo arm Stage 2 (pooled migalastat HCl 6 months) vs. placebo arm Stage 1
- Additional safety data

\*Additional 12-24 month open-label extension following Stage 2

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## Phase 3 Study 012: Overview and Status



- Comparing open-label oral migalastat HCl (150 mg QOD) to ERT (Replagal and Fabrazyme)
- Switch from ERT to migalastat HCl or remain on ERT (1.5:1 randomization)
- Fabry patients with amenable mutations, no kidney biopsies
- Fully enrolled with 60 patients (26 males and 34 females)
- Clinical Outcome is renal function (Iohexol GFR) at 18 months
- 18-month treatment period expected to complete in 2Q14

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## *Key Anticipated Phase 3 Inflection Points*

Detailed Study 011 6-month data at LDN WORLD	Feb. 2013
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Study 011 12-month (Stage 2) top-line data	2Q13
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FDA meeting to discuss U.S. approval pathway	Mid-2013
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Completion of Study 012 18-month treatment period	2Q14
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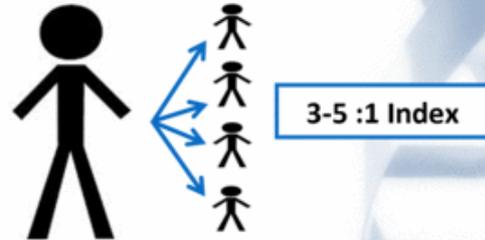
Study 012 top-line data anticipated	2H14
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## Evolving Epidemiology

Although ~30% of Currently Diagnosed Fabry Patients Estimated to Have Amenable Mutations, 75-100% of Patients Identified in Recent Newborn Screening Studies Have Mutations Potentially Amenable to Migalastat HCl

Recent Newborn Screening Studies	# Newborns Screened	# Confirmed Fabry Mutations	% Potentially 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%

Index Patient



*Due to X-linked nature of Fabry, patients identified by screening typically yield 3-5 affected family members (Weidemann 2010)*

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

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# PHARMACOLOGICAL CHAPERONES

## CO-ADMINISTERED WITH MARKETED ERTS

*IMPROVING CURRENT ERTS FOR LYSOSOMAL STORAGE DISORDERS*

### Potential Issues

Protein Instability  
Blood & Infusion Bag

Dosing  
Limitations

Duration of  
Infusion

Immunogenicity



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## Chaperone-ERT Combination Therapy Goals

### Potential to Improve Safety and Efficacy of ERT While Reducing Patient Burden

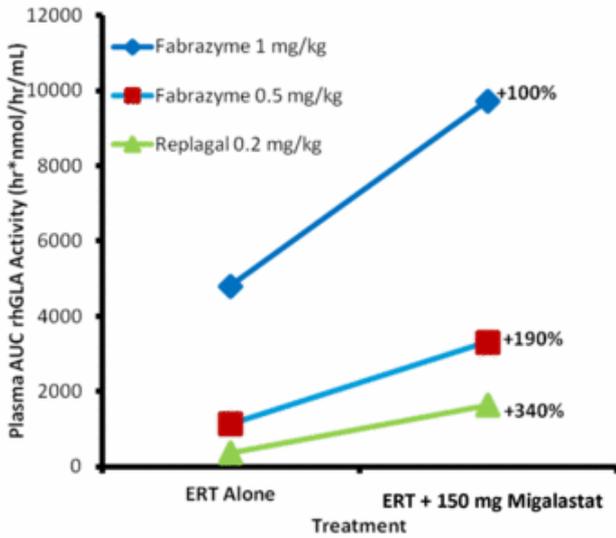
- Enhance ERT pharmacokinetics (greater overall exposure)
- Increase enzyme uptake in tissues, possibly into cell types and tissues not well-served by ERT alone (e.g., podocytes, cardiomyocytes, brain)
- Improve substrate reduction in disease-relevant cell types and tissues
- Mitigate ERT-mediated immunogenicity
- Reduce burden of therapy for patients (e.g., more convenient ERT)

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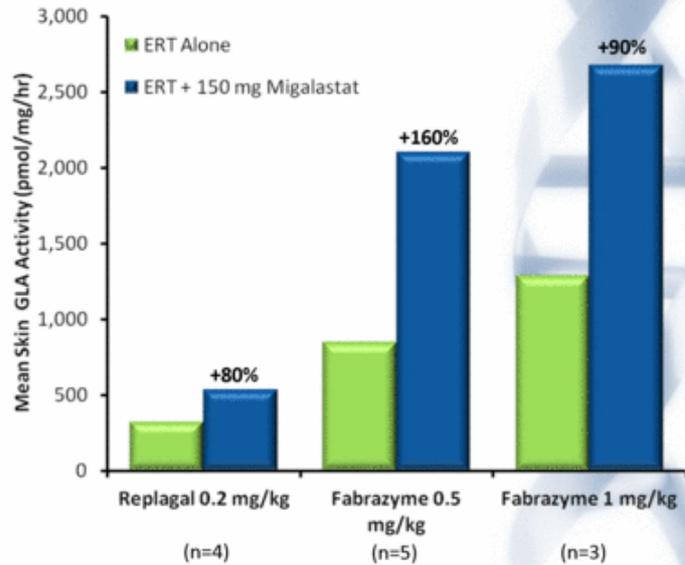
## Phase 2 Study 013: Preliminary Results

Oral Migalastat HCl 150 mg\* Co-Administered with Fabrazyme or Replagal Led to Consistent Increases in Levels of Active Plasma Enzyme and Tissue Uptake (Skin)

**Plasma rhGLA Activity (Area Under Curve)**



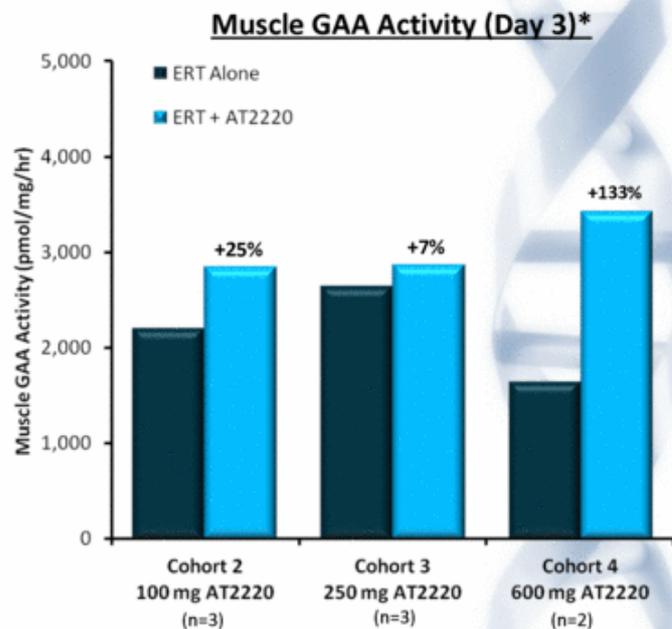
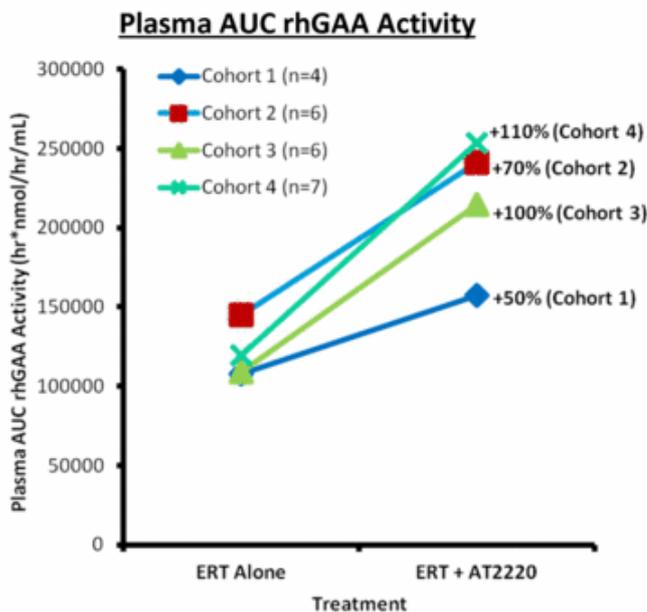
**Mean Skin GLA Activity (Day 2)**



\*Single oral dose 2 hours prior to ERT infusion

## Phase 2 Study 010: Cohorts 1-4

Oral AT2220 Co-Administered with Myozyme/Lumizyme Also Leads to Consistent Increases in Plasma Enzyme Activity and Tissue Uptake (Muscle)



\*Cohort 1 (AT2220 50 mg) muscle GAA activity not shown; 50 mg dose did not demonstrate meaningful change in tissue uptake (muscle)

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# Pompe Chaperone-ERT Co-Administration

## ERT-Related Immunogenicity Problem

### Genetics in Medicine

The impact of antibodies on clinical outcomes in diseases treated with therapeutic protein: Lessons learned from infantile Pompe disease

- “... identification of patients at risk for developing high sustained antibody titer is critical.”<sup>1</sup>

### Molecular Genetics and Metabolism

High antibody titer in an adult with Pompe disease affects treatment with alglucosidase alfa

- “... approximately 40% of the administered alglucosidase alfa was captured by circulating antibodies.”<sup>2</sup>

### MUSCLE & NERVE

ENZYME REPLACEMENT THERAPY INDUCES T-CELL RESPONSES IN LATE-ONSET POMPE DISEASE

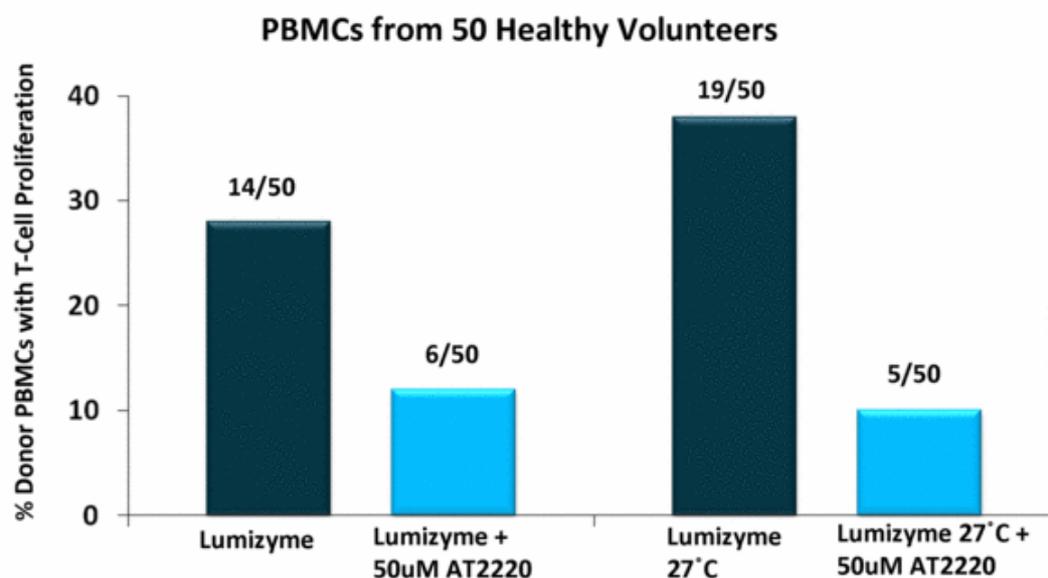
- “... infusion-associated reactions (IARs) [occur] in ~50% of patients receiving alglucosidase alfa infusions.”<sup>3</sup>

1. Banugaria *et al.*, *Gen. Med.*, 2011 Aug.  
 2. de Vries *et al.*, *Mol Genet Metab.*, 2010 Dec.  
 3. Banati *et al.*, *Muscle Nerve*, 2011 Dec.

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## Potential to Mitigate ERT Immunogenicity

AT2220 Mitigates Human T-Cell Response Induced by Lumizyme *ex vivo* and May Significantly Reduce Immunogenicity of Lumizyme



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# Pompe Chaperone-ERT Co-Administration

## Repeat-Dose Clinical Study Anticipated to Begin 3Q13

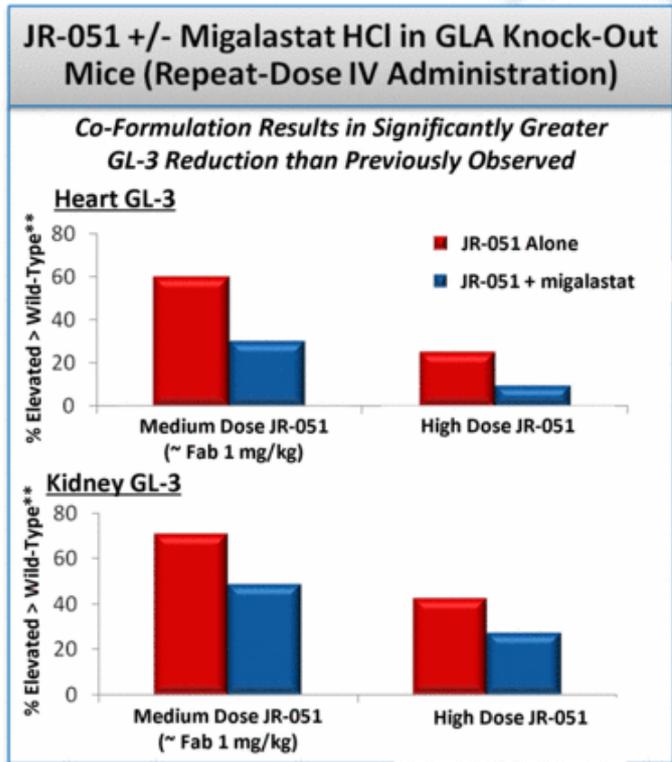
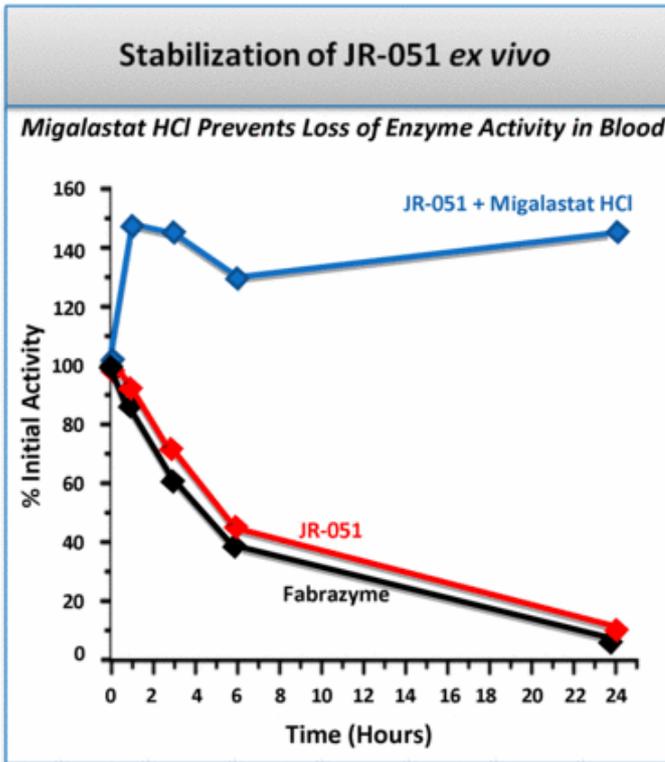
- Target enrollment
  - Adolescents/adults with Pompe disease
  - ERT naïve and ERT experienced
- Endpoints
  - PK
  - Safety
  - Efficacy
  - Immunogenicity
- Treatment duration
  - 12-24 week primary treatment period with potential extension

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# PHARMACOLOGICAL CHAPERONES

CO-FORMULATED WITH RECOMBINANT ERTS

*TOWARD THE NEXT-GENERATION OF  
PROPRIETARY ERTS FOR LYSOSOMAL  
STORAGE DISORDERS*



\*JR-051 designed to be biosimilar to Fabrazyme  
 \*\*0 = wild-type, 100 = untreated KO mouse

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Advancing JR-051 + Migalastat HCl Toward Clinic



- Now manufacturing at 2,000 L scale
- IND-enabling studies underway
- Potential to enter clinic 4Q13/1Q14

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## Next-Generation ERT for Pompe

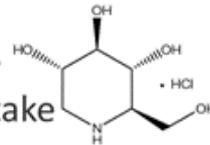
Combining Core Pharmacological Chaperone Technology with Advanced Biologics Capabilities to Create a Next-Generation Pompe ERT

### Next-Generation Pompe ERT



### AT2220 Small Molecule Stabilizer

- Increased exposure & tissue uptake
- Reduced immunogenicity
- Formulation for SQ route of administration



### Potential Improvements

- Optimized glycosylation (e.g., M6-P)
- De-immunization

# 2013 KEY MILESTONES AND CATALYSTS

## Building Shareholder Value

### Migalastat HCl Monotherapy for Fabry Disease

- Study 011 6-Month data (Stage 1) at LDN WORLD Feb. 2013
- Top-line Study 011 12-month data (Stage 2) 2Q13
- FDA meeting to discuss U.S. approval pathway Mid-2013

### Pompe Chaperone-ERT Co-Administration

- Phase 2 Study 010 data at LDN WORLD (all 4 cohorts) Feb. 2013
- Initiation of repeat-dose clinical study 3Q13

### Fabry Chaperone-ERT Co-Administration

- Phase 2 Study 013 data at LDN WORLD (oral migalastat HCl 450 mg + ERT) Feb. 2013

### Fabry Chaperone-ERT Co-Formulation (Migalastat HCl + JR-051)

- IND-enabling studies and clinical supply manufacturing Ongoing
- Potential entry into clinic 4Q13/1Q14



**Amicus Therapeutics Provides Full-Year 2013  
Financial Guidance and Strategic Outlook**

*6-Month (Stage 1) Results from Ongoing Phase 3 Fabry Disease Monotherapy Study at Lysosomal Storage Disease Network WORLD Symposium (LDN WORLD) in February 2013; 12-Month Results Expected in 2Q13*

*Pompe, Fabry and Other New Chaperone-Enzyme Replacement Therapy (ERT)  
Programs Advancing in 2013*

*FY13 Cash Spend Guidance Range of \$52-\$58 Million — Current Cash Expected to Fund Operations into at Least 2H14*

**CRANBURY, NJ, US, January 7, 2013** — Amicus Therapeutics (Nasdaq: FOLD), a biopharmaceutical company at the forefront of developing therapies for rare and orphan diseases, today provided its full-year 2013 strategic outlook and financial guidance. John F. Crowley, Chairman and CEO of Amicus, will discuss Amicus' corporate objectives and key milestones in a presentation at the 31st Annual J.P. Morgan Healthcare Conference on Wednesday, January 9, 2013 at 3 p.m. PT (6 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.

Mr. Crowley stated, "We have a great 2013 ahead of us. The Stage 1 data from our Fabry monotherapy Study 011 are certainly encouraging. We look forward to presenting additional detail about this 6-month data from Study 011 at the WORLD Symposium in February. We also look forward to receiving and analyzing the 12-month efficacy and safety data from Stage 2 of Study 011 in the second quarter of this year. With these data, we expect to engage in constructive discussions with the FDA regarding a U.S. approval pathway for migalastat HCl as the first orally available pharmacological chaperone monotherapy for Fabry disease. Additionally, our Pompe program continues to provide excellent data demonstrating positive effects of AT2220-ERT co-administration, especially regarding the potential to mitigate the immune response to ERT as shown in preclinical studies. Finally, we continue to advance our chaperone-ERT co-formulated products. We believe that these next-generation ERTs have the potential to transform the treatment paradigm for many lysosomal storage diseases."

### **2013 Financial Guidance**

Cash, cash equivalents, and marketable securities totaled \$99.1 million at December 31, 2012 compared to \$55.7 million at December 31, 2011. Amicus expects full-year 2013 net cash spend between \$52 million and \$58 million. The current cash position and anticipated Fabry program reimbursements from GSK are projected to fund operations into the second half of 2014.

Amicus and GSK are co-developing all formulations of migalastat HCl under a global Fabry collaboration. Amicus has commercial rights to all Fabry products in the United States and GSK has commercial rights to all of these products in the rest of world. Amicus and GSK are responsible for 40% and 60% of global development costs, respectively, in 2013 and beyond. Outside the GSK collaboration, Amicus owns exclusive rights to the rest of its pipeline and applications of its platform technology.

### **Strategic Outlook**

Amicus is leveraging its pharmacological chaperone technology platform to develop next-generation medicines for a range of rare and orphan diseases, with a focus on improved therapies for lysosomal storage disorders. During 2013, Amicus and GSK are committed to advancing migalastat HCl for Fabry

disease. Amicus will also continue to advance its pharmacologic chaperone technology platform to develop next-generation therapies (ERTs) for Pompe and additional lysosomal storage disorders. These programs include novel small molecules co-administered with existing enzyme replacement products, as well as proprietary next-generation enzyme replacement therapies that are co-formulated with pharmacologic chaperones.

### **Migalastat HCl Monotherapy for Fabry Disease**

Migalastat HCl monotherapy (150 mg, every-other-day (QOD)) is in two randomized ongoing Phase 3 studies for Fabry Disease (Study 011 and Study 012) in patients with genetic mutations identified as amenable to this pharmacologic chaperone in a cell-based assay.

- Study 011 is comparing migalastat HCl to placebo to potentially support a U.S. marketing application. In December 2012, Amicus and GSK announced encouraging top-line Stage 1 results from the 6-month double-blind treatment period in Study 011 (Stage 1). Additional 6-month data will be presented at the Lysosomal Disease Network WORLD Symposium (LDN WORLD), to be held February 12-15, 2013, in Orlando, Florida. Data from the 6-month open-label follow up period in Study 011 (Stage 2) in which all patients received migalastat HCl are anticipated in the first half of 2013. These results will include 12 months of data for the migalastat HCl group and 6 months of data for the group that crossed over from placebo to migalastat HCl. The FDA has indicated that it will consider the entirety of the efficacy and safety data from Stage 1 and Stage 2 of Study 011. Amicus and GSK expect to meet with the FDA in 2013 to discuss a U.S. approval pathway for migalastat HCl monotherapy.
- Study 012 is comparing open-label migalastat HCl to current standard of care ERTs (Fabrazyme and Replagal) to support global registration. In December 2012, this study achieved full enrollment of 60 patients, who were randomized 1.5:1 to switch from ERT to migalastat HCl or remain on ERT. Data is anticipated in the second half of 2014 on the primary outcome measure, which is renal function assessed by iohexol Glomerular Filtration Rate (GFR) at 18 months.

### **Chaperone-ERT Co-Administration**

### ***AT2220 Co-Administered with Marketed ERTs for Pompe Disease***

In January 2013, Amicus announced positive results from all four patient cohorts in a Phase 2 study (Study 010) to evaluate the safety and pharmacokinetic (PK) effects of the pharmacological chaperone AT2220 co-administered with the standard of care ERTs for Pompe disease (Myozyme®/Lumizyme®, or recombinant GAA enzymes). Results from this study established human proof-of-concept that AT2220-ERT co-administration increases GAA enzyme activity in muscle, particularly at the fourth and highest dose cohort of AT2220 (600 mg). Based on these results, Amicus plans to conduct a repeat-dose clinical study to investigate the effect of AT2220-ERT co-administration on ERT stability and activity, ERT-related immunogenicity, and other clinical measures. This study is expected to begin in the third quarter of 2013.

By stabilizing the folded and active form of the infused GAA enzyme, AT2220 may also mitigate ERT-induced immunogenicity since unfolded and aggregated proteins are generally more antigenic than properly folded proteins. Initial *ex vivo* studies using T cells derived from blood from 50 healthy donors demonstrated that the addition of AT2220 may significantly reduce the immunogenicity of Myozyme and Lumizyme. The studies utilized Antitope Ltd.'s EpiScreen™ assay and are being repeated in samples from the Pompe patients in Study 010. Results from Study 010 will be presented during LDN WORLD. Additional details regarding the Amicus Pompe program will be provided during the presentation and live webcast at the JPMorgan conference.

### ***Migalastat HCl Co-Administered with Marketed ERTs for Fabry Disease***

When co-administered with ERT, migalastat HCl is designed to bind to and stabilize infused enzyme in the circulation in any patient receiving ERT for Fabry disease. Amicus and GSK have completed an open-label Phase 2 study (Study 013) to investigate the effects of a single oral dose of migalastat HCl (150 mg or 450 mg) co-administered prior to ERT (Fabrazyme or Replagal) in 23 males with Fabry disease. Positive preliminary results were announced during 2012 in patients who received migalastat HCl 150 mg

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co-administered with ERT. Results for migalastat HCl 450 mg co-administered with ERT will be presented at LDN WORLD.

### **Chaperone-ERT Co-Formulation**

#### ***Migalastat HCl Co-Formulated with Proprietary ERT for Fabry Disease***

Migalastat HCl co-formulated with JCR Pharmaceutical Co. Ltd.'s proprietary investigational ERT (JR-051, recombinant human alpha-Gal A enzyme) is in preclinical development. Amicus and GSK, in collaboration with JCR, are currently conducting preclinical formulation and IND-enabling studies of this chaperone-ERT co-formulated product, which has the potential to enter the clinic in late-2013 or early 2014.

#### ***AT2220 Co-Formulated with Amicus Proprietary Next-Generation ERT***

Amicus is combining its core pharmacological chaperone technology with advanced biologics capabilities to create a next-generation Pompe ERT. The Company is designing this co-formulated chaperone-ERT product with the goal of increasing exposure and tissue uptake and reducing immunogenicity of current ERTs. The co-formulation with AT2220 may also allow the ERT to be administered through novel routes. Amicus has entered into a contract with Laureate Pharmaceuticals for the contract manufacture of this next-generation ERT. Additional details regarding this aspect of the Amicus Pompe program will also be provided during the presentation and live webcast at the JPMorgan conference.

### **About Amicus Therapeutics**

Amicus Therapeutics (Nasdaq:FOLD) is a biopharmaceutical company at the forefront of developing therapies for rare and orphan diseases. The Company is developing orally-administered, small molecule drugs called pharmacological chaperones, a novel, first-in-class approach to treating a broad range of human genetic diseases. Amicus' late-stage programs for lysosomal storage disorders include migalastat HCl monotherapy in Phase 3 for Fabry disease; migalastat HCl co-administered with enzyme replacement therapy (ERT) in Phase 2 for Fabry disease; and AT2220 co-administered with ERT in Phase 2 for Pompe disease.

### **About Migalastat HCl for Fabry Disease**

Amicus in collaboration with GlaxoSmithKline (GSK) is developing the investigational pharmacological chaperone migalastat HCl for the treatment of Fabry disease. Amicus has commercial rights to all Fabry products in the United States and GSK has commercial rights to all of these products in the rest of world. As a monotherapy, migalastat HCl is designed to bind to and stabilize, or "chaperone" alpha-galactosidase A (alpha-Gal A) enzyme in patients with genetic mutations that are amenable to this chaperone in a cell-based assay. For patients currently receiving ERT for Fabry disease, migalastat HCl in combination with ERT may improve ERT outcomes by keeping the infused alpha-Gal A enzyme in its properly folded and active form.

Fabry disease is an inherited lysosomal storage disease that is currently estimated to affect approximately 5,000 to 10,000 people worldwide. Fabry Disease is caused by deficiency of an enzyme called alpha-galactosidase A (alpha-Gal A). The role of alpha-Gal A within the body is to break down a complex lipid called globotriaosylceramide (GL-3). Reduced or absent levels of alpha-Gal A activity leads to the accumulation of GL-3 in the affected tissues, including the central nervous system, heart, kidneys, and skin. This accumulation of GL-3 is believed to cause the various symptoms of Fabry disease, including pain, kidney failure, and increased risk of heart disorders and stroke.

### **About AT2220 for Pompe Disease**

AT2220 is an investigational, orally-administered pharmacological chaperone owned exclusively by Amicus. In published preclinical studies, AT2220-ERT co-administration resulted in significant increases in muscle rhGAA levels and decreases in glycogen levels in a mouse model of Pompe disease. Preclinical results to date also suggest that AT2220-ERT co-administration may mitigate ERT-induced immunogenicity by stabilizing the enzyme in its properly folded and active form.

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Pompe disease is a lysosomal storage disease characterized by progressive skeletal muscle weakness and respiratory insufficiency. It is caused by a deficiency in GAA activity, which leads to accumulation of glycogen in tissues affected by the disease (primarily muscle). Pompe disease affects an estimated 5,000 to 10,000 individuals worldwide and is clinically heterogeneous in the age of onset, the extent of organ involvement, and the rate of progression.

## **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, and the projected cash position for the Company. Words such as, but not limited to, “look forward to,” “believe,” “expect,” “anticipate,” “estimate,” “intend,” “plan,” “targets,” “likely,” “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 effects or other safety issues; the potential that we will need additional funding to complete 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 Quarterly Report on Form 10-Q for the quarter ended June 30, 2012. 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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