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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 9, 2012

**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.)

**6 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.**

On January 12, 2012, John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. (the "Company"), will participate in the 30<sup>th</sup> Annual J.P. Morgan Healthcare Conference (the "Conference"). A copy of the presentation to be given by Mr. Crowley at the Conference is attached to this Current Report as Exhibit 99.1. In addition, on January 9, 2012, 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 9, 2012

By: /s/ Geoffrey P. Gilmore  
Geoffrey P. Gilmore  
Senior Vice President and General Counsel

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## EXHIBIT INDEX

<u>Exhibit No.</u>	<u>Description</u>
99.1	Presentation Materials
99.2	Press Release dated January 9, 2012



# Corporate Overview



*At the Forefront of Therapies for Rare and Orphan Diseases™*  
*January 2012*

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 preclinical studies and clinical trials evaluating Amicus’ candidate drug products, the projected cash position for the Company, and business development and other transactional activities. 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 Annual Report on Form 10-K for the year ended December 31, 2010. 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.

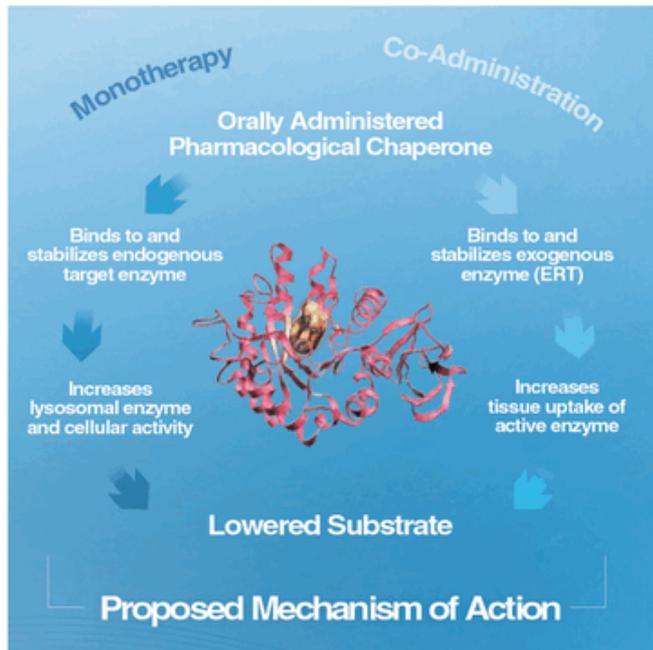
# Amicus is Business Led & Science Driven

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

		
Pharmacological Chaperone Platform Technology	Global Clinical Capabilities & Pipeline	Alliance with GSK Rare Diseases
<ul style="list-style-type: none"><li>▪ Proprietary platform &amp; IP</li><li>▪ Small molecules targeting misfolded and unstable proteins</li><li>▪ Stabilize &amp; enhance patient's own enzyme; <i>or</i></li><li>▪ Potential to stabilize &amp; enhance ERT products for lysosomal storage disorders (LSDs)</li></ul>	<ul style="list-style-type: none"><li>▪ Global expertise in rare disease clinical research</li><li>▪ Clinical sites in over 20 countries</li><li>▪ Multiple Fabry Phase 3 &amp; Phase 2 programs</li><li>▪ Pompe Phase 2 program underway</li></ul>	<ul style="list-style-type: none"><li>▪ 19.9% FOLD ownership stake</li><li>▪ \$60M cash upfront + cost-sharing</li><li>▪ GSK to act as global commercial lead on Fabry</li></ul>

## One Technology, Two Novel Applications

- Based on patient's own mutated enzyme
- Potential alternative to Enzyme Replacement Therapies (ERTs)

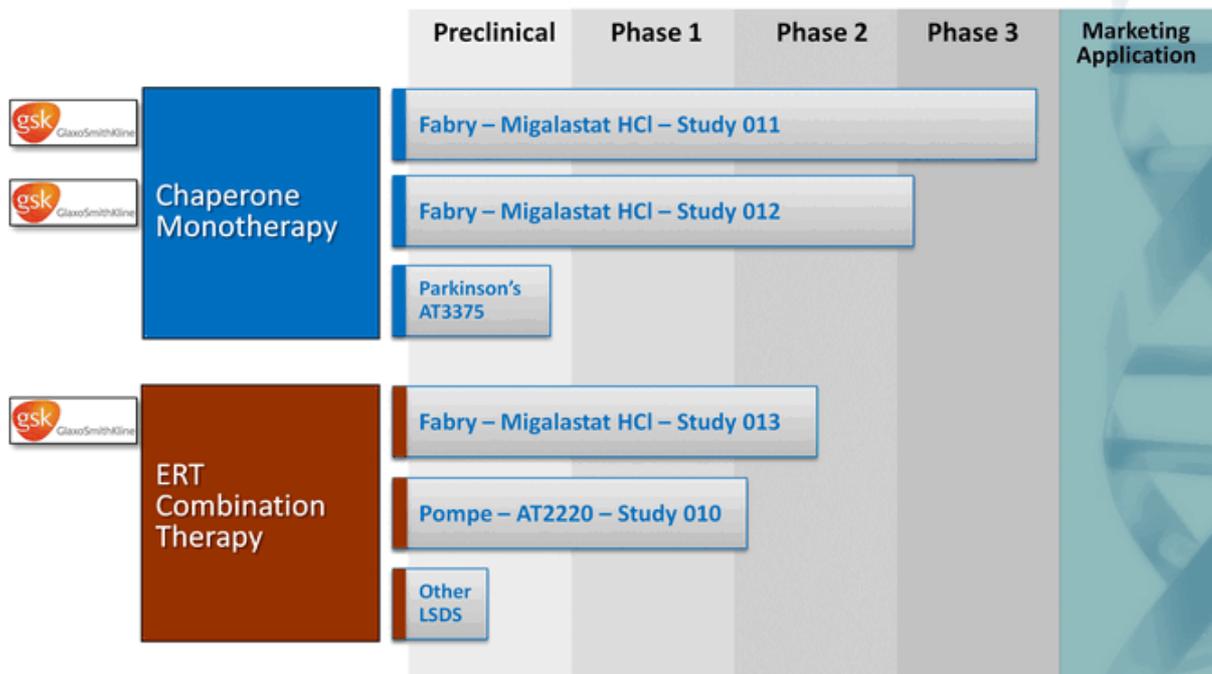


- Potential to improve ERT stability, uptake and activity & lower immunogenicity



# Pharmacological Chaperones:

## Advanced Product Pipeline





### Product and Strategic Alliance



#### Migalastat HCl for Fabry Disease

- Monotherapy & ERT Combination Therapy
- 75% global development costs paid by GSK (as of 2012)
- GSK has exclusive rights to commercialize Migalastat HCl globally
- Amicus eligible to receive up to \$170M in milestones; tiered double-digit royalties on Migalastat HCl
- Close working relationship in rare diseases
- GSK equity ownership



## Three Key Clinical Studies Underway

### STUDY 011

- Phase 3 Monotherapy
- U.S. Registration Study
- Placebo controlled
- 67 patients
- Surrogate endpoint – kidney GL-3
- Eligible for accelerated approval
- Data expected 3Q12
- Pre-NDA meeting anticipated 2H12

### STUDY 012

- Phase 3 Monotherapy
- Global Registration Study
- Switch from ERT
- 50 patients
- 18-month clinical endpoint – kidney function
- Full enrollment targeted by YE12

### STUDY 013

- Phase 2 Chaperone-ERT combination
- Drug-drug interaction study
- Positive preliminary results in humans
- 2 doses of chaperone with Fabrazyme® and REPLAGAL™
- Plasma PK & PD (skin biopsies)
- Expect to complete 1H12

## Phase 3 Confidence

### Study 011 Design Contributes to Potential for Phase 3 Success

#### Phase 2 Experience

- 90+ years of patient experience
- 17 Phase 2 patients remain on migalastat HCl monotherapy
- Positive results on renal and urine GL-3 clearance (key biomarker)
- Long-term trends toward stabilization of kidney function

#### Strict Entry Criteria

- Naïve to ERT / no ERT in past 6 months
- Amenable mutations
- Urine GL-3  $\geq$  4x normal

#### Improved Histological Methodology

- BLISS-VM Methodology more advanced, sensitive & objective\* vs. Thurberg-LM

#### Phase 3 Observations Study 011

- Low dropout rate
- High conversion to extension study

## *Patient Disposition to Date*

### Low Drop Out Rate & High Conversion from Study 011 to Extension Study

67 Patients enrolled

44 to date completed 6 month TX period (19 ongoing)

44 continued in 6-month treatment extension (41 ongoing)

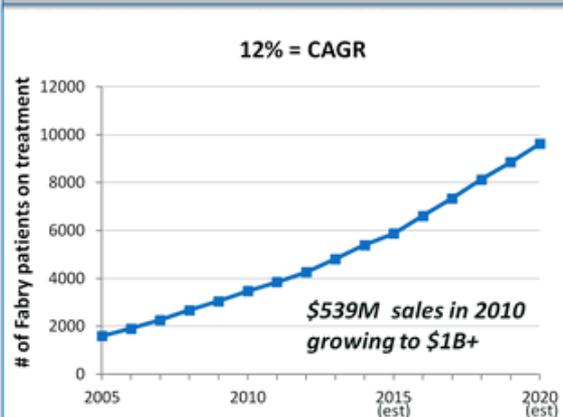
23 to date completed Study 011 (6-mo. TX + 6-mo. Extension)

23 enrolled in open-label extension studies (21 ongoing)

## Continuing to Grow

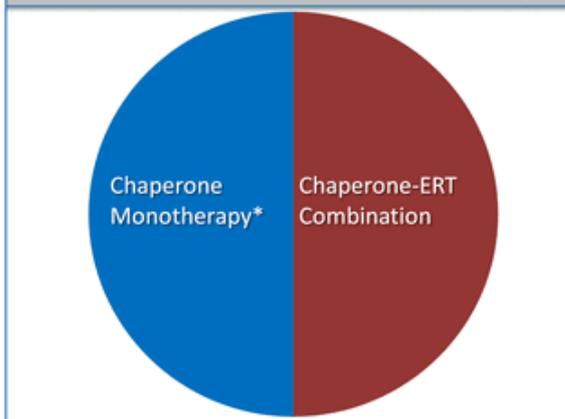
All Fabry Patients Potentially Eligible to Receive Migalastat HCl Upon Approval as Monotherapy or in Combination with ERT

### Market Opportunity



Sources: Leerink 2011, Cowen 2011, Genzyme/Shire 10Ks

### Projected Future Market Distribution



\*Includes estimated size of undiagnosed population with amenable mutations; Spada 2006, Hwu 2009, Mechtler 2011

# CHAPERONE-ERT COMBINATION TECHNOLOGY



## Potential Limitations of 1st Generation Technology

LSD market currently dominated by ERTs... ...with multiple ERTs in development



However, significant unmet needs remain due to potential limitations of 1<sup>st</sup> generation approach (ERT)

*"...on the basis of our studies, one of the major obstacles to enzyme replacement with alpha-Gal is the instability of the enzyme."*

Beutler and Mayes, 1977  
Biochimica et Biophysica Acta

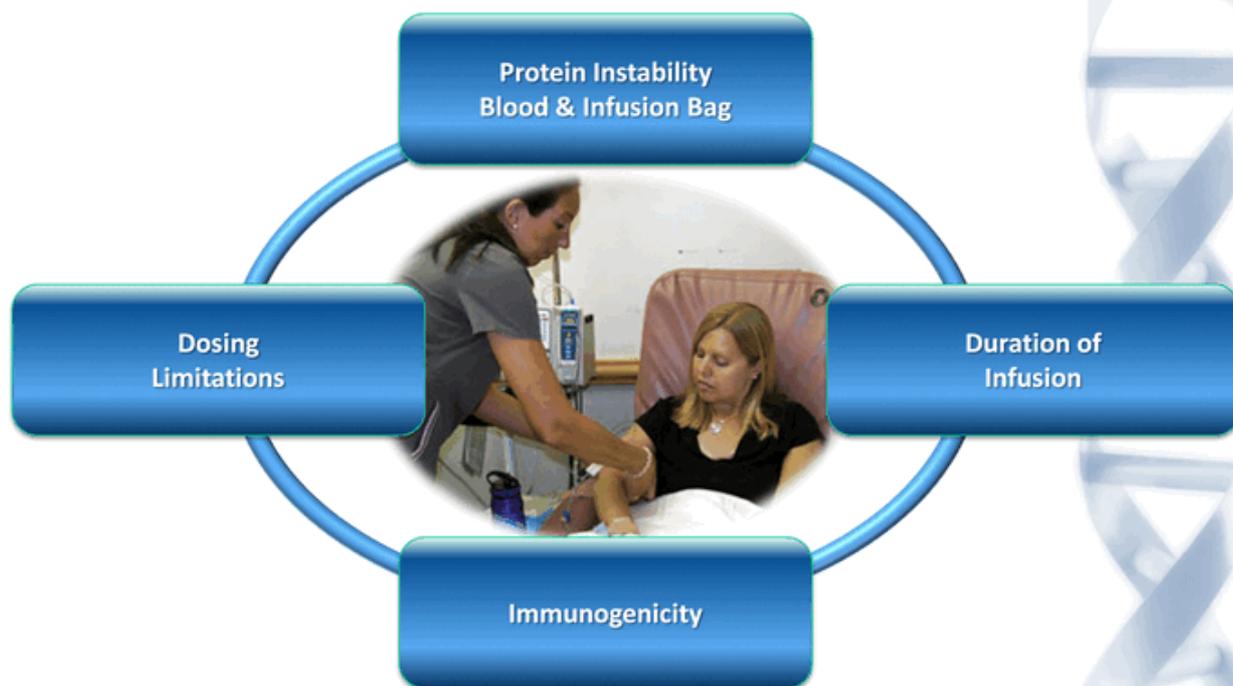
*"Unfortunately, none of the currently prescribed ERTs is able to treat all aspects of the (LSD) disorders equally. This is not surprising and was predicted following the embryonic attempts at ERT in the 1970s..."*

Wraith, 2006  
J Inherit Metab Dis  
(Presented SSIEM 2005)

*"...(Physicians) note that Myozyme is clearly not a cure. They believe response can be variable from patient to patient, with some having excellent response, and others a minimal one."*

Cowen and Company, 2011  
Orphan Disease Research Report

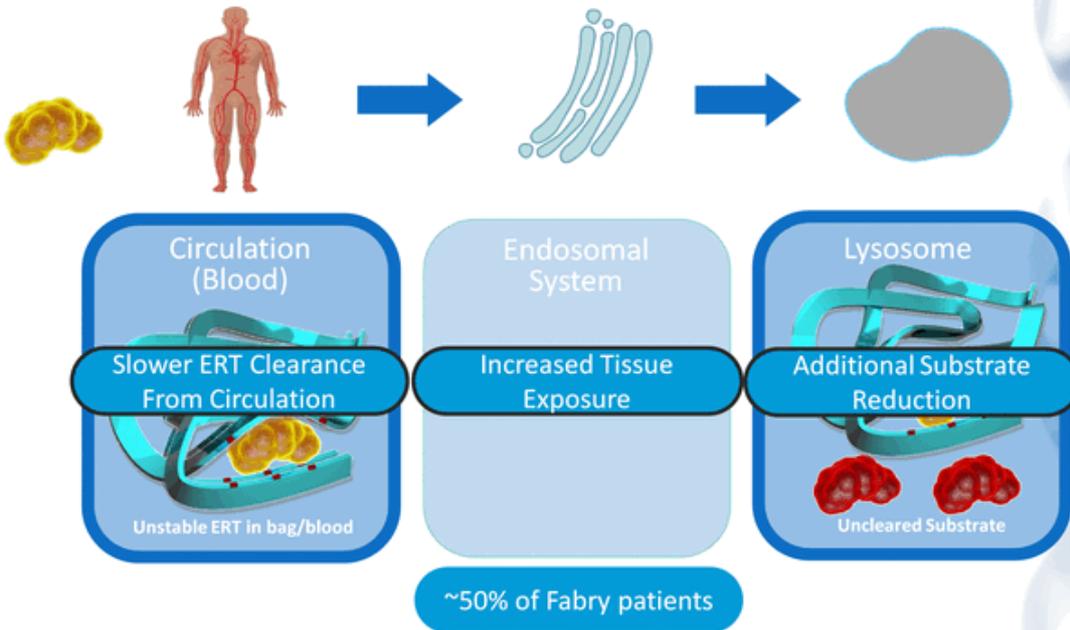
## Potential Issues



# Improving ERTs for Lysosomal Storage Disorders

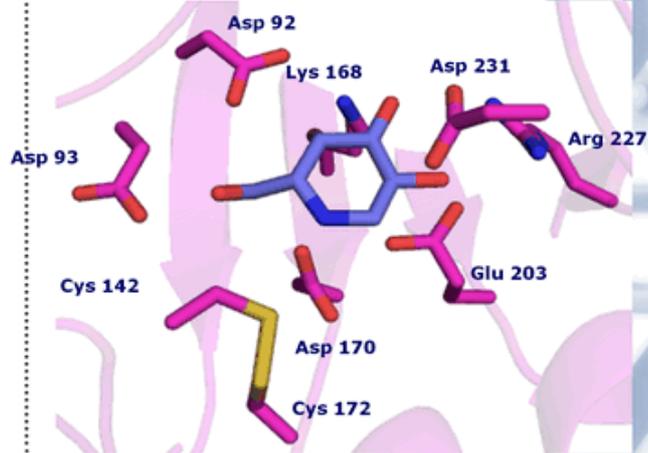
## Pharmacological Chaperone Co-Administration

Next Generation Therapy: potential for improving ERT





Structure of  $\alpha$ -Gal A Active Site Bound to AT1001



Crystal structures by R. Lieberman & G. Petsko

## Overview

### Study Population

- 18-24 male Fabry patients on ERT (all mutation types eligible)

### ERTs Evaluated

- 0.5 mg/kg Fabrazyme (every 2 weeks)
- 1.0 mg/kg Fabrazyme (every 4 weeks)
- 0.2 mg/kg Replagal (every 2 weeks)

### Migalastat HCl Doses Evaluated

- 150 mg }  
450 mg } *Single dose, 2 hours prior to ERT infusion*

### Endpoints Studied

- Safety
- $\alpha$ -Gal A activity in plasma and in skin +/- Migalastat HCl

Day 1

ERT Alone (period 1)  
ERT + Migalastat HCl (pd 2)

- Baseline Skin Biopsy for  $\alpha$ -Gal A Activity (predose)
- Serial Blood Sampling for Plasma  $\alpha$ -Gal A Activity

Day 2 (24 hours)

- Skin Biopsy for  $\alpha$ -Gal A Activity

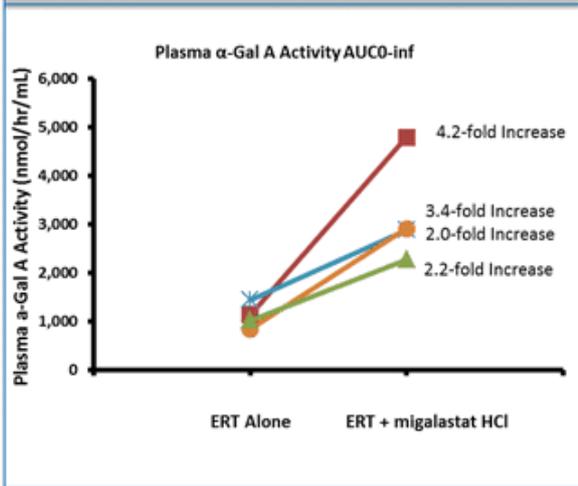
Day 7

- Skin Biopsy for  $\alpha$ -Gal A Activity

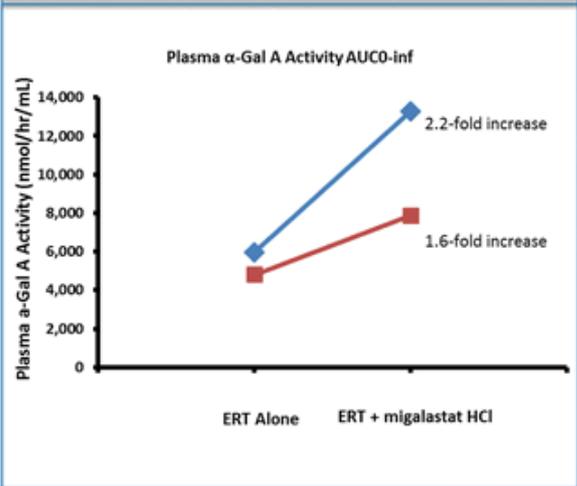
Plasma PK Preliminary Data (n = 6)

**Co-Administration Increases Levels of Active Enzyme in Plasma  
~2.0- to 4.0-Fold vs. ERT Alone in First 6 Patients**

Migalastat HCl 150 mg + Fabrazyme 0.5 mg/kg (n=4)

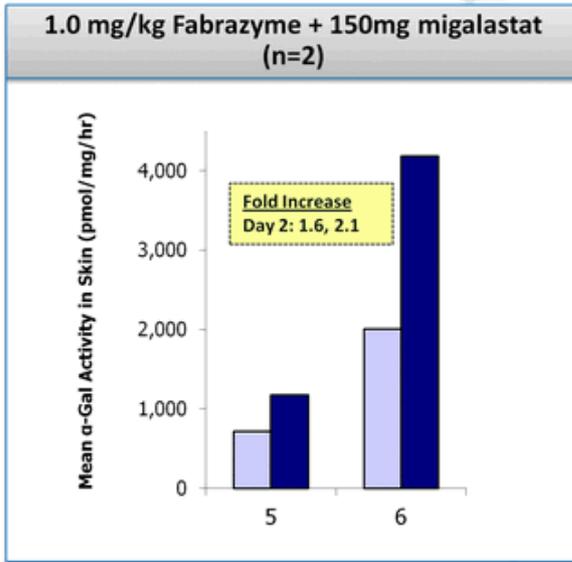
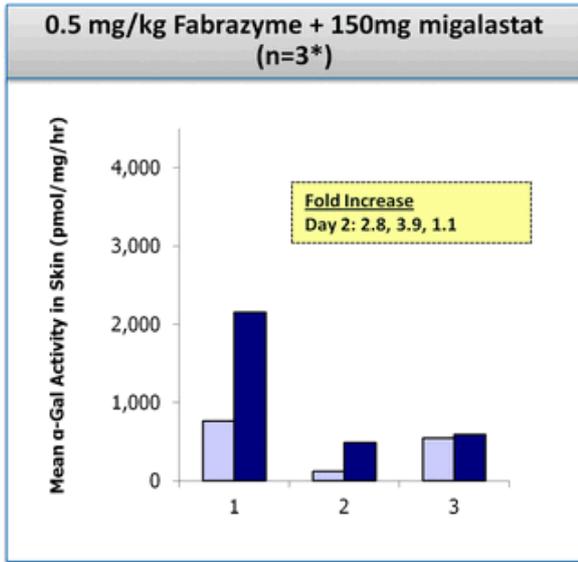


Migalastat HCl 150 mg + Fabrazyme 1 mg/kg (n=2)



**Skin Biopsies – Preliminary Data (n = 6)**

**Co-Administration Increases Levels of Active Enzyme in Skin at Day 2 vs. ERT Alone**

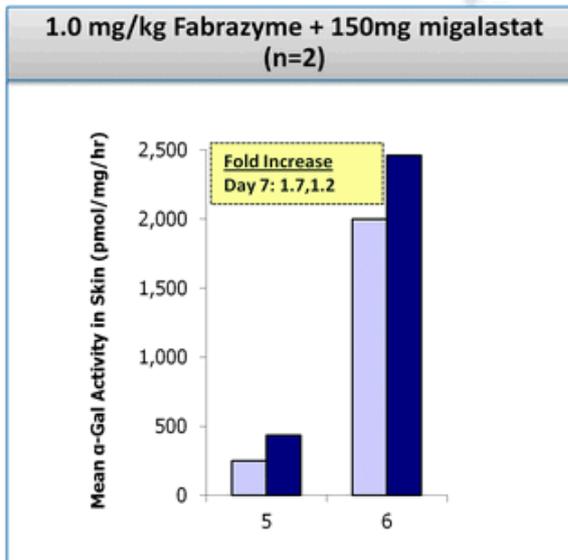
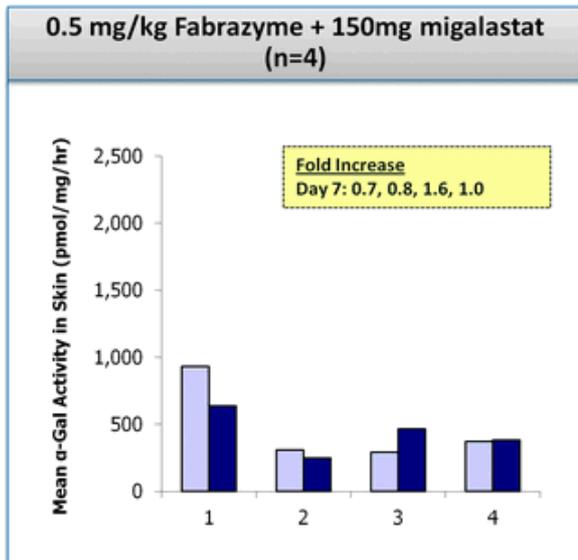


ERT alone    ERT + migalastat

\*1 Biopsy Sample Lost

## Skin Biopsies – Preliminary Data (n = 6)

### Co-Administration Increases Levels of Active Enzyme in Skin at Day 7 vs. 1 mg/kg Fabrazyme Alone



ERT alone    ERT + migalastat

### *Next Steps*

The following additional data are expected in 1H 2012:

Fabrazyme (0.5 mg/kg) + 450 mg Migalastat

Fabrazyme (1.0 mg/kg) + 450 mg Migalastat

Replagal (0.2 mg/kg) + 150 mg and 450 mg Migalastat

## *Pompe Market Overview*

- **Worldwide population of ~5,000 Pompe patients<sup>1</sup>**
  - Estimated true incidence is ~1 in 40,000 live births
  - Literature suggests incidence of 1 in 20,000-100,000 live births (~2,000-10,000 patients)
  
- **Significant unmet needs exist due to potential limitations of Myozyme/Lumizyme<sup>1,2</sup>**
  - Outcomes vary greatly among treated patients
  - Challenges of addressing muscle manifestations of disease
  - Delivered relatively inefficiently to affected cells and organs
  - Potential immunogenicity issues; antibody neutralization and infusion reactions



<sup>1</sup>Cowen and Company Orphan Disease Research Report, 2011

<sup>2</sup>BioMarin R&D Day, Dec 2011

## Phase 2 PK/PD Study 010

### Study Population

- 16 Pompe Patients on ERT

### ERT Evaluated

- Myozyme/Lumizyme

### AT2220 Doses Evaluated

- 4 increasing doses (single dose, given prior to ERT infusion)

### Endpoints Studied

- Safety
- GAA activity in plasma and in muscle +/- AT2220

Preliminary Data from Study 010 Expected 1H 2012

# PARKINSON'S



## Amicus at the Forefront

2011

- MJFF approves second year to continue preclinical studies of AT3375

2010

- MJFF awards \$500,000 grant to focus on Parkinson's-implicated protein alpha-synuclein
- IP issued for use of chaperone to treat neurological disorders by enhancing Gcase
- IND-enabling work for chaperone AT3375 begins

2008

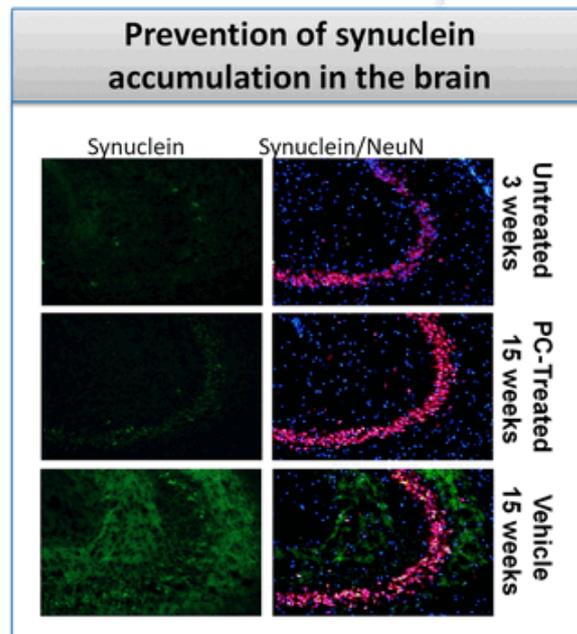
- New series of chaperones identified, improve on AT2101 properties to target CNS

2007

- Chaperone AT2101 increases activity of brain GCase, prevents accumulation of  $\alpha$ -synuclein in brain, and improves motor function in preclinical animal models

2006

- MJFF awards \$196,000 grant to support research into chaperones for Parkinson's disease



## Increasing Focus of Key Publications

J Inherit Metab Dis  
DOI 10.1007/s10545-010-9055-0

ORIGINAL ARTICLE

### The risk of Parkinson's disease in type 1 Gaucher disease

Gilberto Baltrou • Katherine Kacena • Daniel Pearson •  
Michael Boxer • Ruhua Yang • Swati Sathe •  
Gregory Pastores • Pramod K. Mistry

In conclusion, we determined that the risk of a patient with GD1 developing PD was approximately 20-times that of the control population. These results have implications

**ANNALS**  
of Neurology

THE OFFICIAL JOURNAL OF  
THE AMERICAN NEUROLOGICAL  
ASSOCIATION AND THE  
CHILD NEUROLOGY SOCIETY

### Acid $\beta$ -glucosidase mutants linked to Gaucher disease, Parkinson's and Lewy body dementia alter $\alpha$ -synuclein processing

Valerie Cullen, S. Paolo Sardi, Juliana Ng, Xu-Yu Hui, Ying Sun, Julianna I. Tomlinson, Piotr Kolodziej, Ilana Kahn, Paul Saftig, John Woulfe, Jean C. Rochet, Marie A. Gluckman, Seng H. Cheng, Gregory L. Grabowski, Lanya S. Shihabuddin, Michael G. Schlossmacher

**jbc** THE JOURNAL OF  
BIOLOGICAL CHEMISTRY

### $\alpha$ -SYNUCLEIN INTERACTS WITH GLUCOCEREBROSIDASE PROVIDING A MOLECULAR LINK BETWEEN PARKINSON AND GAUCHER DISEASES\*

Thai Leong Yap<sup>1</sup>, James M. Gruschus<sup>1</sup>, Arash Velayati<sup>1</sup>, Wendy Westbrook<sup>2</sup>, Ehud Goldin<sup>2</sup>, Nima Moaven<sup>2</sup>, Ellen Sidransky<sup>2</sup>, and Jennifer C. Lee<sup>1</sup>

**Cell**

### Gaucher Disease Glucocerebrosidase and $\alpha$ -Synuclein Form a Bidirectional Pathogenic Loop in Synucleinopathies

Joseph R. Mazzulli<sup>1</sup>, You-Hai Xu<sup>2,3</sup>, Ying Sun<sup>2,3</sup>, Adam L. Knight<sup>4</sup>, Pamela J. McLean<sup>4</sup>, Guy A. Caldwell<sup>4,5</sup>, Ellen Sidransky<sup>6</sup>, Gregory A. Grabowski<sup>4,7</sup> and Dimitri Krainovich<sup>1,7</sup>

**The Journal of Neuroscience**  
The Official Journal of the Society for Neuroscience

### $\alpha$ -Syn Suppression Reverses Synaptic and Memory Defects in a Mouse Model of Dementia with Lewy Bodies

Younghin Lim, Victoria M. Kehm, Edward B. Lee, James H. Soper, Chi Li, John Q. Trojanowski, and Virginia M.-Y. Lee

# **STRATEGIC VISION, 2012 MILESTONES AND GUIDANCE**



## *Multiple Paths Forward for ERT-Chaperone Combinations*

**We Envision Series of Incremental, Important Product Advances Unique to Each LSD Over This Decade**

Currently Marketed ERTs

Currently Marketed ERTs Co-Administered with Chaperones

Currently Marketed ERTs Co-Formulated with Chaperones

Currently Marketed ERTs Co-Formulated with Chaperones and with Improved Delivery/Regimen

Next Generation ERT Co-Formulated with Chaperones

Next Generation ERT Co-Formulated with Chaperones and with Improved Delivery/Regimen

# 2012 Anticipated Milestones

## Building Shareholder Value

### Fabry

- Study 011 Phase 3 Data Q3
- Study 012 Complete Enrollment Q4
- Study 013 Complete 1H

### Pompe

- Study 010 Phase 2 Preliminary Data 1H

### Genetic Parkinsons

- '3375 IND Enabling Studies Complete Q4



Estimated Beginning Cash: ~\$60 M

2012 Cash Spend Guidance: \$37 M to \$43 M

Expected Current Cash Runway: Mid Q3 2013

*“We begin 2012 as a company with late stage clinical programs, a broad platform technology, a strong balance sheet and a key strategic partnership with GSK. We believe that these pillars of strength uniquely position Amicus to grow into a fully integrated biopharmaceutical company at the forefront of developing therapies for rare and orphan diseases,”*

-John F. Crowley, Amicus Chairman & CEO (January 9, 2012)



**Amicus Therapeutics Provides Full-Year 2012  
Strategic Outlook and Financial Guidance**

***Phase 3 Results of Migalastat HCl Monotherapy for Fabry Disease on Track for 3Q12***

***Positive Preliminary Chaperone-Enzyme Replacement Therapy Data Obtained in Humans***

***Plans to Advance Multiple Chaperone-ERT Combination Programs***

***FY12 Operating Expense Guidance Range of \$37-\$43 Million —  
Expects Current Cash to Fund Operations Into Mid-3Q13***

**CRANBURY, NJ, US, January 9, 2012** — Amicus Therapeutics (Nasdaq: FOLD) today provided its business outlook and financial guidance for fiscal year 2012, including details of its strategic vision and plan. John F. Crowley, Chairman and CEO of Amicus will discuss Amicus' corporate objectives and key milestones in a presentation at the 30<sup>th</sup> Annual J.P. Morgan Healthcare Conference on Thursday, January 12, 2012 at 8 a.m. PT (11 a.m. ET).

Mr. Crowley stated, "We expect that 2012 will be a transformational year for Amicus as we look forward to the Phase 3 data and path toward a U.S. marketing application for migalastat monotherapy for Fabry disease. We are also greatly encouraged by the positive preliminary Chaperone-ERT data in Fabry patients in our ongoing Phase 2 study. We believe that this combination platform technology has the potential to improve the therapeutic efficacy and safety profile of multiple enzyme replacement therapies for lysosomal storage diseases".

"We begin 2012 as a company with late-stage clinical programs, a broad platform technology, a strong balance sheet and a key strategic partnership with GSK. We believe that these pillars of strength uniquely position Amicus to grow into a fully integrated biopharmaceutical company at the forefront of developing therapies for rare and orphan diseases," concluded Mr. Crowley.

**Strategic Vision and Plan**

The company plans to advance in 2012 two pharmacological chaperone monotherapy programs for genetic diseases:

- Migalastat HCl for patients with Fabry disease identified as having alpha-galactosidase A mutations amenable to chaperone therapy
- AT3375 for Parkinson's disease in Gaucher disease carriers and potentially the broader Parkinson's population

Amicus also plans to advance multiple pharmacological chaperone-ERT combination programs for genetic diseases, including:

- Migalastat HCl co-administered with ERT for patients with Fabry disease receiving ERT treatment, with any genetic mutation
  - AT2220 (duvoglustat HCl) co-administered with ERT for Pompe disease
  - Several new, undisclosed pharmacological chaperone programs focused on the combination of chaperones with ERTs for additional lysosomal storage diseases (LSDs).
-

## Fabry Disease Programs

Migalastat HCl is an investigational oral pharmacological chaperone for the treatment of Fabry disease being developed in collaboration with GlaxoSmithKline (GSK). Under the terms of the collaboration, GSK has an exclusive worldwide license to develop, manufacture and commercialize migalastat HCl.

Amicus and GSK are conducting two Phase 3 global registration studies (Study 011 and Study 012) of migalastat HCl monotherapy, along with a Phase 2 study (Study 013) evaluating migalastat co-administered with enzyme replacement therapy (ERT) for the treatment of Fabry disease.

Positive preliminary results from the ongoing Phase 2 Study 013 were announced in a press release last week and will be presented as a "late breaking" abstract at the 8th Annual Lysosomal Disease Network WORLD Symposium (LDN WORLD) in San Diego, February 8-10, 2012.

Key objectives in 2012 for these Fabry programs include:

- Presentation of Phase 3 (Study 011) patient demographics, preliminary Phase 2 (Study 013) co-administration results, and additional abstracts at LDN WORLD
- Complete Phase 2 (Study 013) co-administration study in 1H12
- Phase 3 (Study 011) results of migalastat HCl monotherapy in 3Q12
- Completion of Phase 3 (Study 012) enrollment by year-end 2012 to support additional global marketing applications

## Chaperone-ERT Combination Programs

The Company owns exclusive rights to the pharmacological chaperone AT2220 (duvoglustat HCl) for Pompe disease. During the first half of 2012, Amicus expects to announce preliminary results from an ongoing, open-label Phase 2 drug-drug interaction study (Study 010) of AT2220 co-administered with the ERT alglucosidase alfa in approximately 16 individuals with Pompe disease. Study 010 is designed to evaluate the safety of co-administered AT2220 and ERT. The study will also evaluate the plasma pharmacokinetics of the infused enzyme with and without co-administration of AT2220, and will assess uptake of active enzyme into skeletal muscle (quadriceps) via needle biopsy.

Data from preclinical studies in Pompe knock-out mice presented in 2011 at several scientific symposia demonstrated that AT2220 co-administered with ERT significantly enhanced the uptake of the active enzyme into key organs involved in Pompe disease, including heart, diaphragm, and skeletal muscles. These preclinical data also showed a greater reduction of glycogen in key organs with the co-administration of AT2220 versus ERT alone.

In 2012, Amicus intends to increase its commitment to the broader application of the chaperone-ERT combination technology as a potential next-generation treatment approach for multiple LSDs. The Company has initiated new undisclosed pharmacological chaperone research and development programs to investigate the use of chaperones in combination with ERTs potentially to improve treatment outcomes.

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## **Parkinson's Disease Program**

Amicus has been a pioneer in investigating the link between Gaucher and Parkinson's disease (PD), and has been exploring the possibility of using pharmacological chaperones that target glucocerebrosidase (GCase), the enzyme deficient in Gaucher disease, for more than five years.

In 2011, numerous peer-reviewed publications in leading scientific journals shed additional light on the underlying mechanisms that link Gaucher and PD, and further validated GCase as a target for the treatment of Parkinson's disease. In particular, these new papers demonstrated a direct connection between GCase and alpha-synuclein, whose accumulation in the brain is a hallmark of PD, and showed that increased GCase activity in the brain of mouse models could correct alpha-synuclein pathology and other deficits.

Amicus will continue preclinical and IND-enabling studies for the pharmacological chaperone AT3375, which targets the same GCase enzyme that is deficient in Gaucher disease. These preclinical studies are anticipated to be complete by year-end 2012 and are funded in part by a grant awarded by the Michael J. Fox Foundation.

## **FY12 Financial Guidance**

The Company expects to begin 2012 with a cash balance of approximately \$60 million and full-year 2012 operating expenses to total between \$37 million and \$43 million, net of cost sharing and milestones related to the GSK collaboration. The Company anticipates that its current cash position, including anticipated Fabry program reimbursements from GSK will be sufficient to fund operations through the middle of third quarter 2013. This does not include any regulatory milestones that the Company may be eligible to receive in this time period under its agreement with GSK.

Amicus and GSK equally shared development costs for migalastat HCl (monotherapy and co-administration) in 2011, and GSK is responsible for 75% of these costs in 2012 and beyond. The Company is also eligible to receive up to \$170 million in development, regulatory and commercial milestones under the Fabry collaboration.

## **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 diseases including lysosomal storage disorders and diseases of neurodegeneration. Amicus' lead program migalastat HCl is in Phase 3 for the treatment of Fabry disease.

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### **About Fabry Disease**

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 Pompe Disease**

Pompe disease is a lysosomal storage disease characterized by progressive skeletal muscle weakness and respiratory insufficiency. It is caused by a deficiency in lysosomal alpha-glucosidase (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 “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995 relating to clinical development of Amicus’ candidate drug products 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,” “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 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. 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, 2010. 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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