

**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 13, 2014**

**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 13, 2014, 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.

Exhibit Number	Description
99.1	Presentation Materials
99.2	Press Release dated January 13, 2014

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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 13, 2014

By: /s/ William D. Baird III  
William D. Baird III  
Chief Financial Officer

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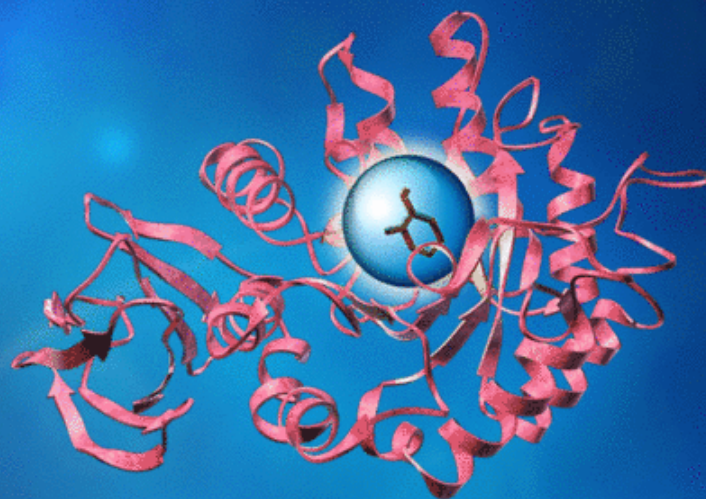
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#### EXHIBIT INDEX

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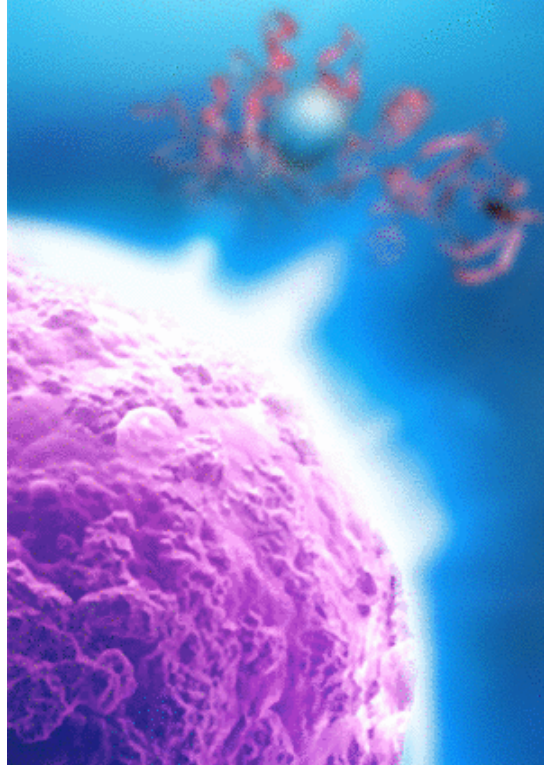
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**32<sup>nd</sup> Annual J.P. Morgan  
Healthcare Conference**

*January 16, 2014*

*at the forefront of therapies  
for rare and orphan diseases*



# 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, ongoing collaborations 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’ 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, 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.*



# 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***

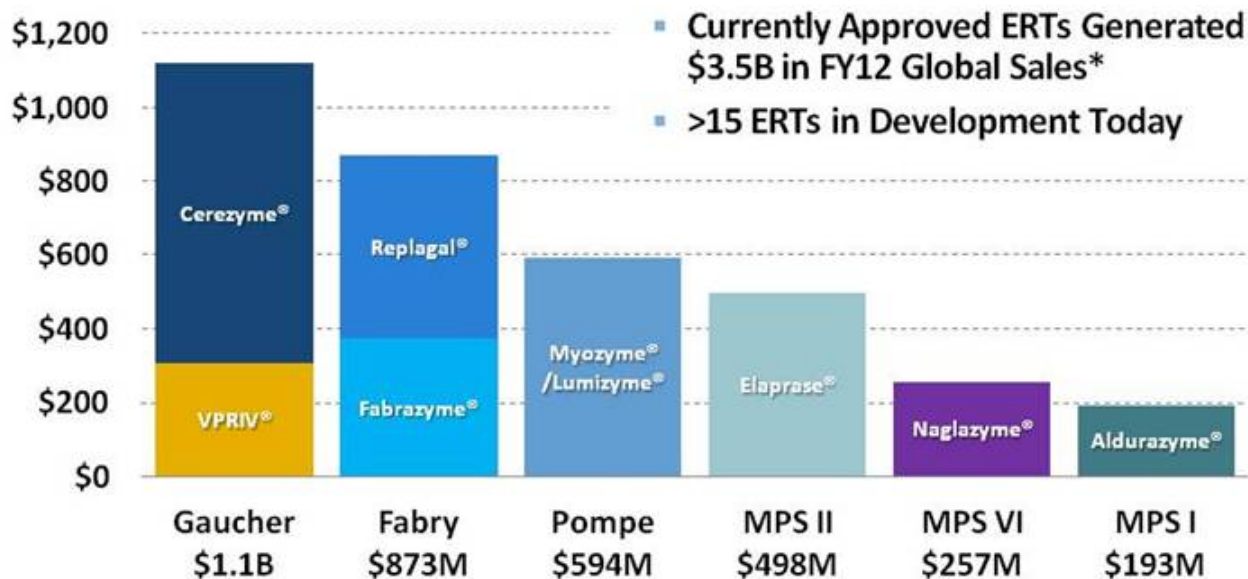


# 2014 Highlights



## \$3.5B Current ERT Market for LSDs

FY12 Global Sales (\$M)

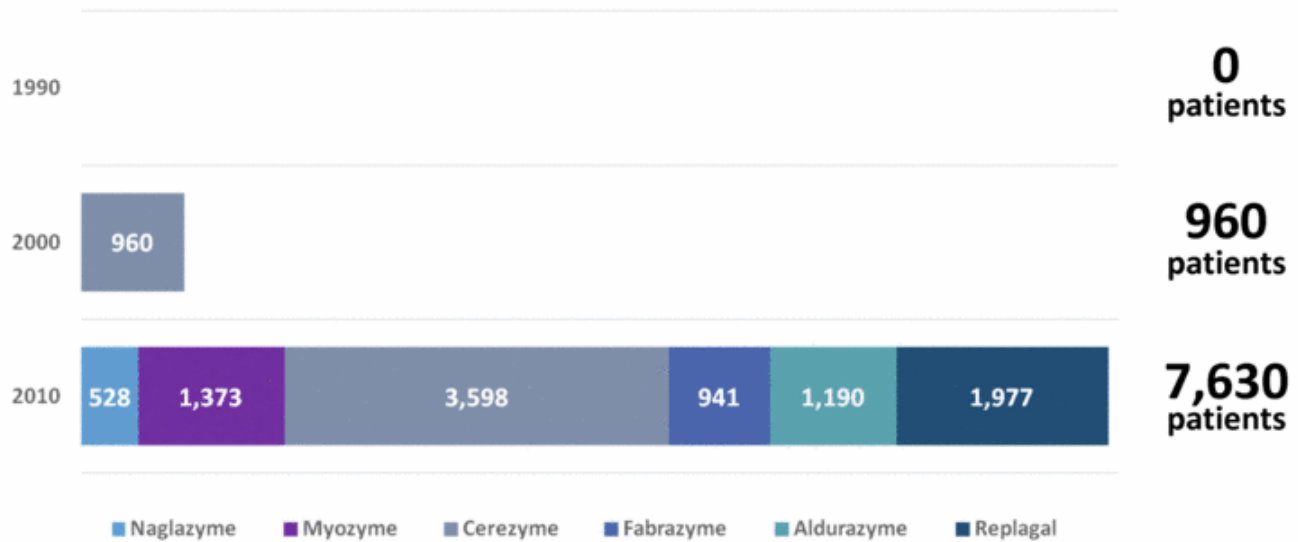


\*Source: 10-Ks from Shire, Sanofi, and BioMarin. Sales of Eleyso for Gaucher disease not shown. Fabrazyme, Cerezyme, Myozyme and Lumizyme are registered trademarks owned by Sanofi-Aventis. VPRIV, Replagal and Elaprase are registered trademarks owned by Shire. Naglazyme and Aldurazyme are registered trademark owned by BioMarin



# Three Decades of ERT for Patients

ERT Has Meaningfully Improved Lives of Patients with LSDs



CBI Conference ppt; <http://www.bizjournals.com/boston/news/2011/10/28/shire-resubmits-rare-disease-drug.html>;  
<http://www.medicalbillingandcoding.org/blog/the-11-most-expensive-medicines-in-america>

# Unmet Needs in LSDs Today

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



*"...one of the major obstacles to enzyme replacement with  $\alpha$ -Gal is the instability of the enzyme."* - Beutler and Mayes, 1977



ORPHANET JOURNAL OF RARE DISEASES

*"Long term ERT does not prevent [Fabry] disease progression, but the risk of developing a first or second complication declines with increasing treatment duration."* - Rombach, et al. 2013

### THE NEW ENGLAND JOURNAL OF MEDICINE

Lumizyme statistically improves overall motor and pulmonary function...14% of pts on treatment have declining 6-minute walk test and 36% have declining forced vital capacity. - van der Ploeg, et al. 2010

Pediatric RESEARCH

*"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



## LSD Products Today: Three Fundamental Challenges



- Enzyme Activity and Stability
- Tissue Uptake and Targeting
- Tolerability and Immunogenicity



# Advanced Product Pipeline

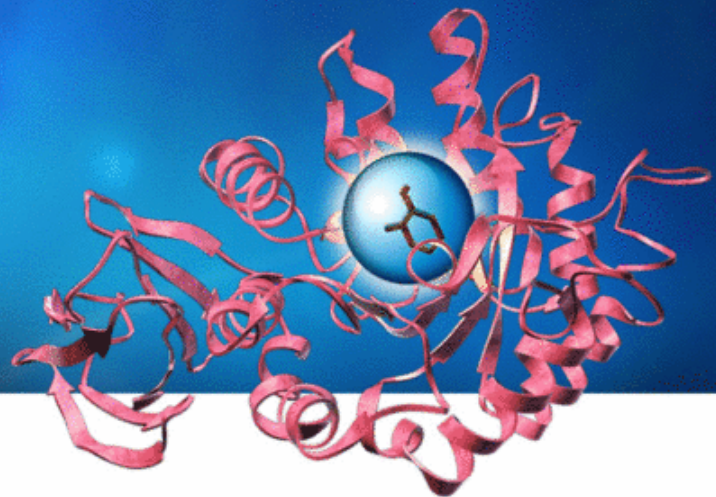


INDICATION	PRE-CLINICAL	PHASE 1	PHASE 2	PHASE 3
<b>Fabry Disease</b>				
AT-B100 (JR-051) + Migalastat HCL				
<b>Pompe Disease</b>				
AT-B200 (rhGAA) + Chaperone				
<b>Mucopolysaccharidosis Type I</b>				
Next-Generation ERT				
<b>Fabry Disease</b>				
Migalastat HCL				
<b>Parkinson's Disease</b>				
Novel Small Molecules				

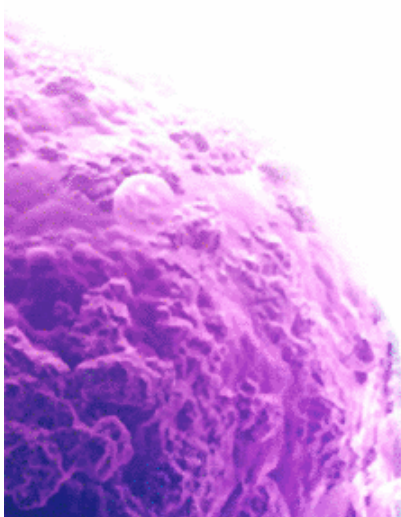
*3 Next-Generation ERTs  
Expected to Enter Clinic  
in Next 3 Years*

NEXT-GENERATION ERTs (co-formulation)

MONOTHERAPY



## Next-Generation ERT for Pompe Disease

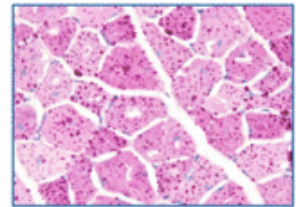




# Pompe Disease Overview

Severe, progressive, fatal neuromuscular disease

- Deficiency of lysosomal enzyme GAA
- Age of onset ranges from infancy to adulthood
- Glycogen accumulation in muscle tissue
- Incidence 1:28,000<sup>1</sup>
- Current ERT sub-optimal



Elevated Glycogen  
in Muscle

 Amicus  
Therapeutics

# Three Challenges with Pompe ERT

## Activity/ Stability

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

## Uptake/ Targeting

Low M6P receptor  
uptake into skeletal  
muscle<sup>2</sup>

Majority of rhGAA  
is not delivered to  
lysosomes<sup>2</sup>

## Tolerability / Immunogenicity

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

High antibody titers  
shown to affect  
treatment  
outcomes<sup>4,5</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.

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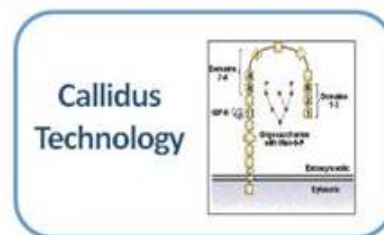


## Complementary Technologies to Address ERT Challenges in Pompe Disease

Callidus Acquisition Provided Pompe ERT (AT-B200) and Platform Technology  
Complementary to Amicus' CHART™ Platform



- ➔ Binds to and stabilizes rhGAA
- ➔ Increases uptake of active enzyme into tissues
- ➔ Improves tolerability and potentially mitigates immunogenicity



- ➔ Enzyme uniquely engineered with high M6P content and optimized carbohydrate structures
- ➔ Peptide tag (variant of IGF-2, or vIGF-2) further enhances drug targeting and uptake

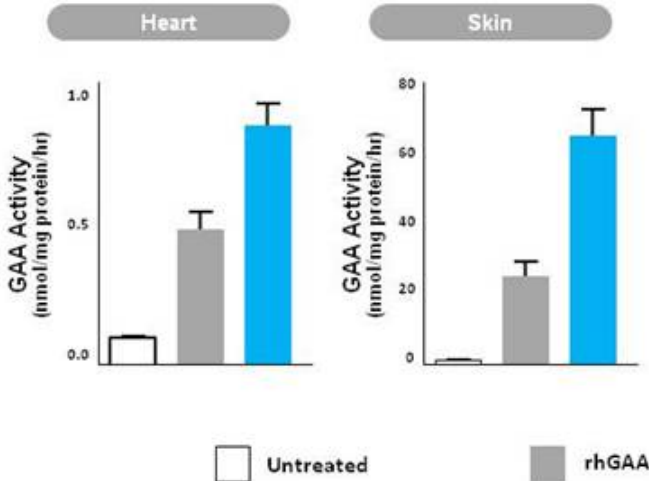
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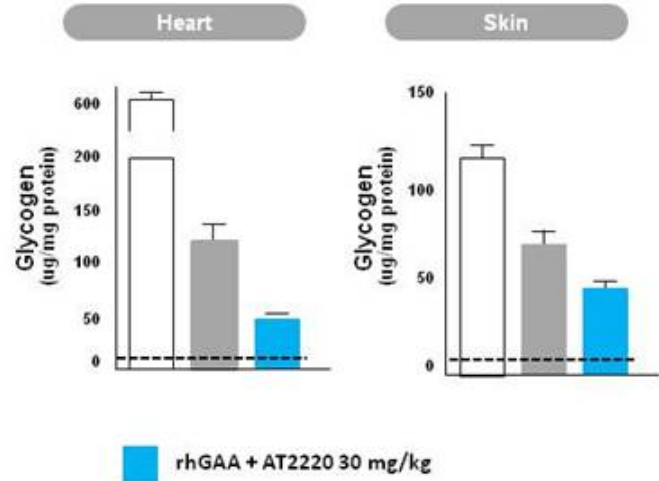
# CHART Preclinical Proof-of-Concept: AT2220 + Myozyme/Lumizyme (rhGAA)<sup>1</sup>

Co-Formulation Results in Significantly Greater Tissue Uptake and Further Substrate Reduction Compared to Myozyme/Lumizyme Alone\*

## rhGAA Tissue Uptake



## rhGAA-Mediated Glycogen Reduction



\* Repeat-dose IV administration in GAA KO Mice

<sup>1</sup>Khanna, et al., Exploring the Use of a Co-formulated Pharmacological Chaperone AT2220 with Recombinant Human Acid Alpha-Glucosidase for Pompe Disease, LDN WORLD 2013

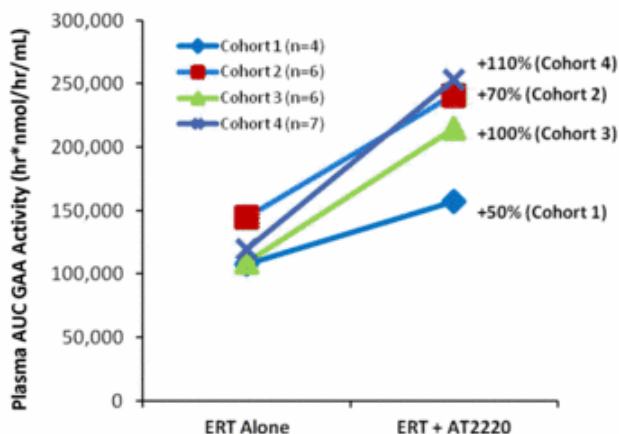


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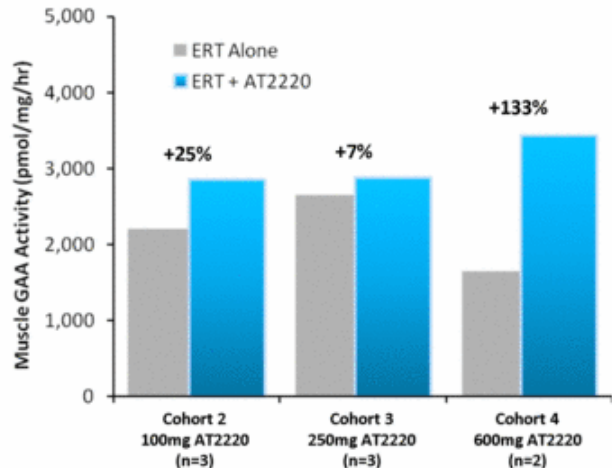
# CHART Human Proof-of-Concept: Phase 2 Pompe Co-Administration Study

Co-Administration Consistently Increases Plasma Enzyme Levels and Tissue Uptake Compared to Myozyme/Lumizyme Alone<sup>1</sup>

## Plasma AUC GAA Activity



## Muscle GAA Activity (Day 3)\*



<sup>1</sup>Kishnani, et al., A Phase 2a Study to Investigate Drug-Drug Interactions between Escalating Doses of AT2220 (Duvoglustat Hydrochloride) and Acid Alfa-Glucosidase in Subjects with Pompe Disease, LDN WORLD 2013  
\*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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# Callidus Biopharma Acquisition

Strengthens Amicus' Pompe Program and Advances Time to Clinic

**Pompe rhGAA ERT Product**

**Novel vIGF-2 Technology Platform**

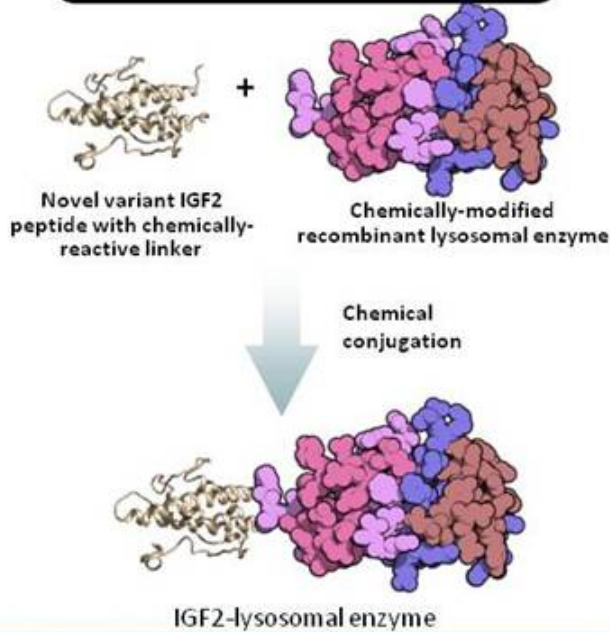
**Synergies with CHART**

**Biologics Experience and Leadership**

**Manufacturing Relationships and Contracts**

## Callidus Technology Offers Flexible Approach to Add Variant of Insulin Growth Factor 2 (vIGF-2) Peptide Tag to Any Lysosomal Enzyme

### Conjugation Strategy



### Substantial Differentiation

- vIGF-2 more selective than IGF-2
  - Does not bind to insulin receptor
  - Does not bind to IGF-1 receptor
- vIGF2 peptide can be conjugated to any ERT without decreasing circulating half-life to ensure good drug exposure.
- Conjugation of vIGF2 peptide can significantly improve receptor binding, even for those that contain high M6P
- Tagging approach applicable to any ERT and other proteins
- Potential conjugation of peptides that cross blood-brain-barrier

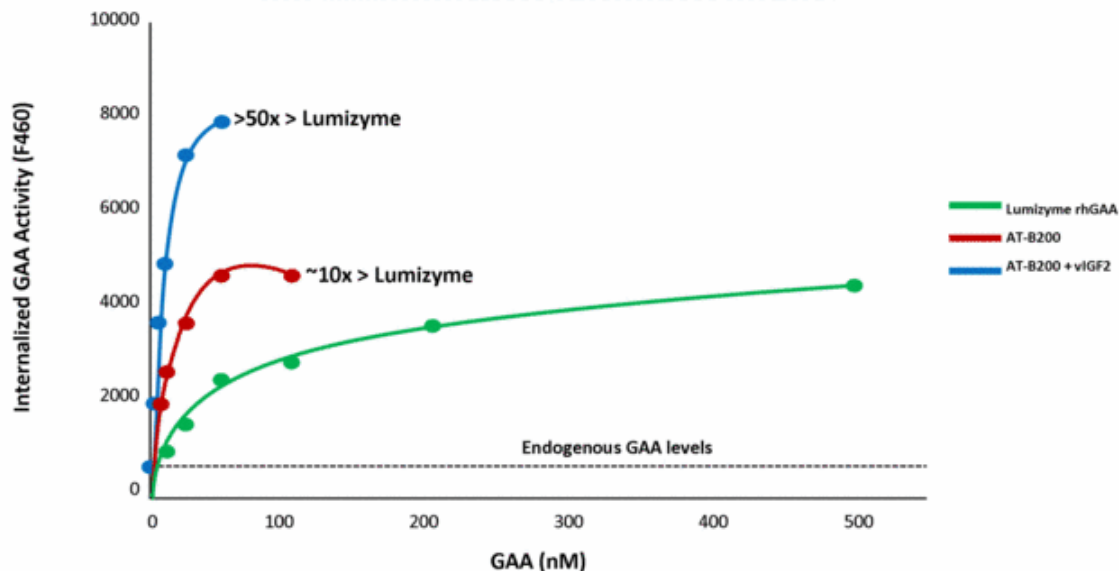
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(Preliminary Results)

## AT-B200: Next-Generation Pompe ERT (rhGAA)

AT-B200 Has Demonstrated Significant Advantages in Preclinical Studies that May Be Further Improved by Co-Formulating with a Chaperone

### L6 Myoblast Uptake

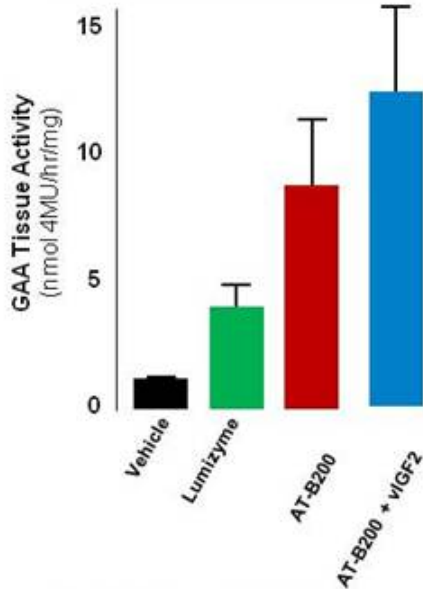


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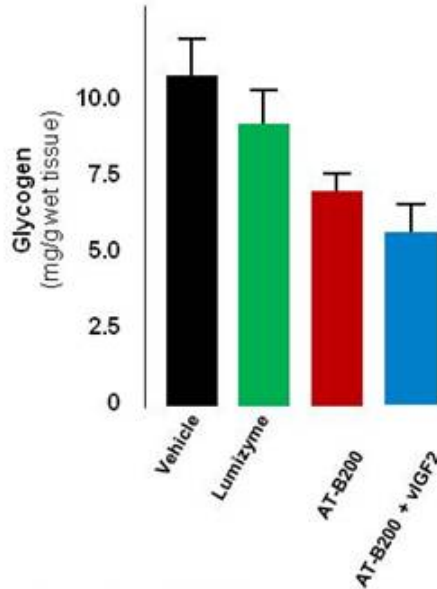
# AT-B200: Next-Generation Pompe ERT (rhGAA)

AT-B200 Led to Better Uptake into Muscle and Further Glycogen Reduction Compared to Lumizyme in Preclinical Studies in GAA Knock-Out Mice\*

GAA Activity in Quadriceps



Glycogen in Quadriceps



\* 4 weekly doses of each GAA preparation. Tissues harvested 1 week after last dose.

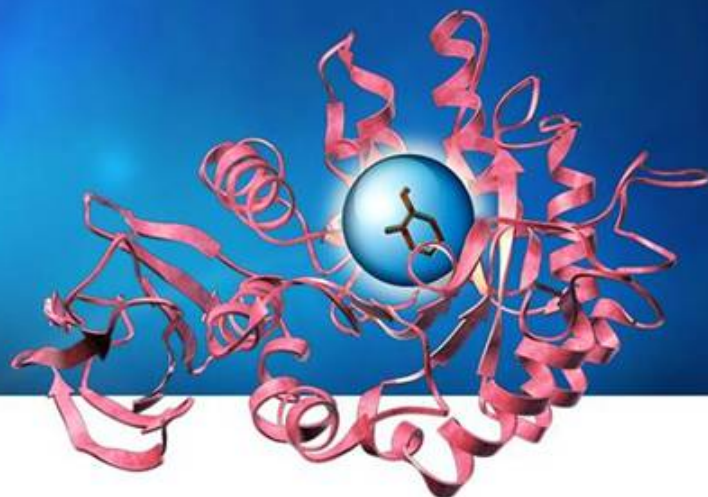


## Approaches in Pompe Drug Development

Challenges with Pompe ERT	IGF2-GAA	Neo-GAA	AT-B200 + Chaperone	vIGF2-AT-B200 + Chaperone
Stability & Activity	X	X	✓	✓
Targeting & Uptake	✓ (IGF2 Tag)	✓ (M6P)	✓ (M6P)	✓✓ (M6P + vIGF2 Tag)
Tolerability & Immunogenicity	X	X	✓	✓
Development Stage	Phase 3	Phase 1	Preclinical	Preclinical

✓ = May address    X = May not address



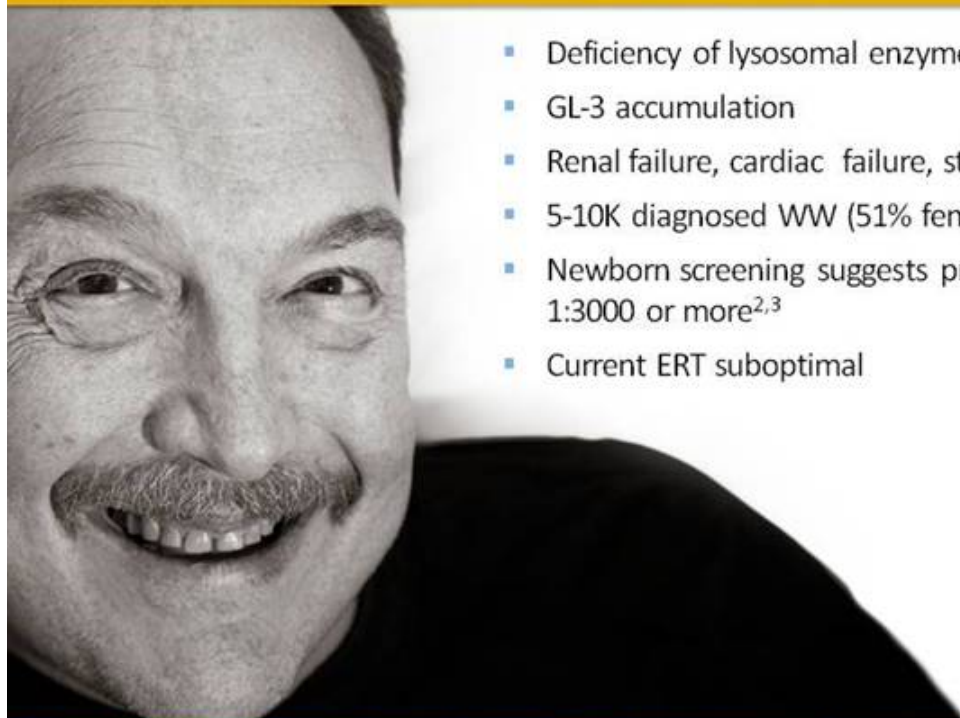


***Next-Generation ERT for Fabry Disease***

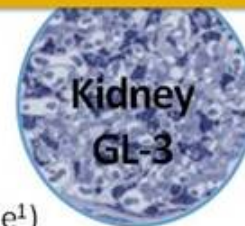


# Fabry Disease Overview

Proprietary ERT (AT-B100) Co-Formulated with Migalastat HCl Expected to Enter Clinic in 2014



- Deficiency of lysosomal enzyme GLA
- GL-3 accumulation
- Renal failure, cardiac failure, stroke
- 5-10K diagnosed WW (51% female/49% male<sup>1</sup>)
- Newborn screening suggests prevalence of 1:3000 or more<sup>2,3</sup>
- Current ERT suboptimal



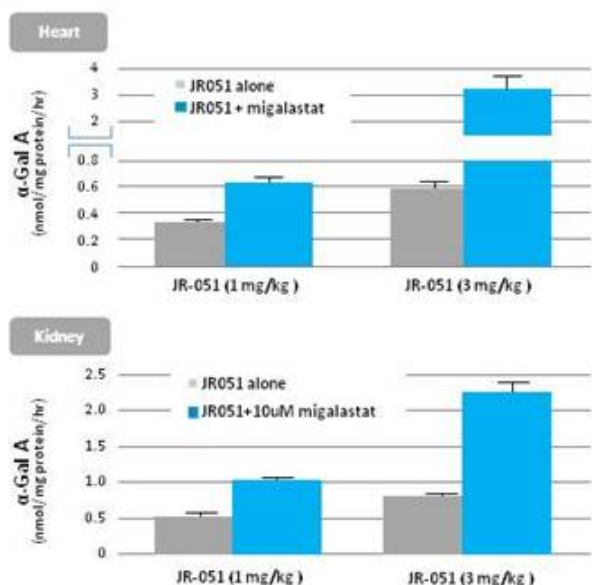
22 <sup>1</sup>Fabry Registry 2011; <sup>2</sup>Spada et al., Am J Human Genetics 2006; <sup>3</sup>Lin, et al., Circulation 2009 <sup>4</sup>Rambacher et al., PLoS ONE, 2012



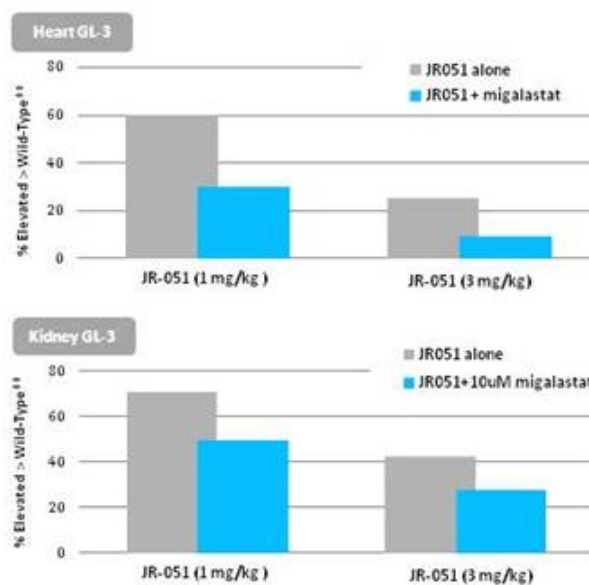
## CHART Preclinical Proof-of-Concept: (Preliminary Results) Next-Generation Fabry ERT

Co-Formulation (JR-051 + Migalastat) Results in Significantly Greater Tissue Uptake and Further Substrate Reduction\*

### A-Gal A Tissue Uptake



### GL-3 Substrate Reduction



\* JR-051 +/- Migalastat HCl in GLA Knock-Out Mice (Repeat-Dose IV Administration) <sup>†</sup> JR-051 designed to be biosimilar to Fabrazyme: \*\*0 = wild-type, 100 = untreated KO mouse

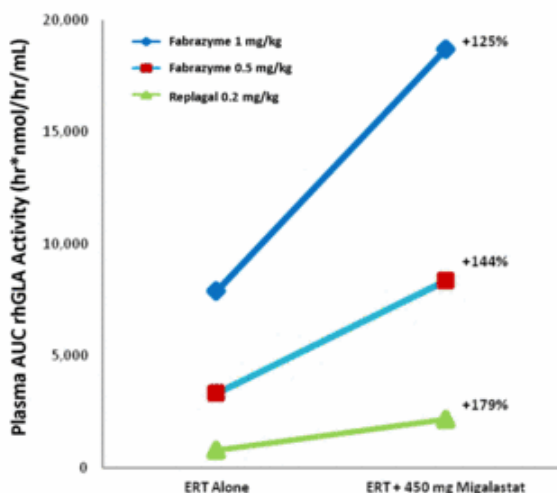




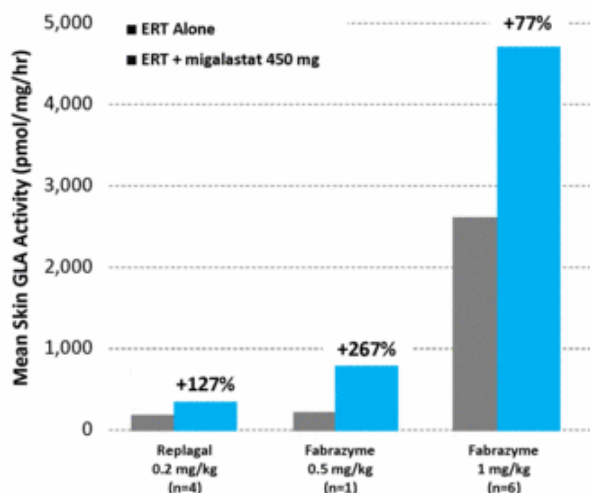
# CHART Human Proof-of-Concept: Phase 2 Fabry Co-Administration Study

Co-Administration with Fabrazyme or Replagal Leads to Consistent Increases in Active Plasma Enzyme Levels and Tissue Uptake<sup>1</sup>

Plasma rhGLA Activity (Area Under Curve)



Mean Skin GLA Activity (Day 2)



<sup>1</sup> Bichet, et al., A Phase 2a Study to Investigate the Effect of a Single Dose of Migalastat HCl, a Pharmacological Chaperone, on Agalsidase Activity in Subjects with Fabry Disease, LDN WORLD 2013.

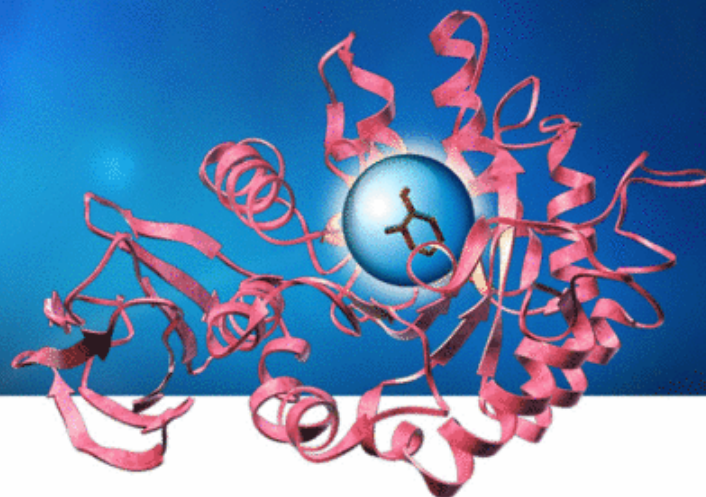


## Pathway to Clinic in 2014

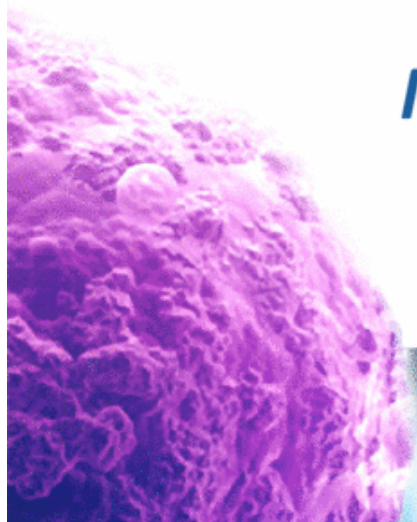
(Preliminary Results)

Timing	Milestone	✓
1Q12	Initial preclinical studies for next-generation Fabry ERT	✓
3Q12	Preclinical proof-of-concept	✓
1H13	Scale up to 2,000 L	✓
Ongoing	IND-enabling studies	✓
2H13	Preclinical data presented at SSIEM and ASHG	✓
<b>1H14</b>	<b>Phase 1 study initiation of IV migalastat in healthy volunteers</b>	
<b>2H14</b>	<b>Pre-IND meeting</b>	
<b>2H14</b>	<b>Next-generation ERT Phase 2 study initiation</b>	



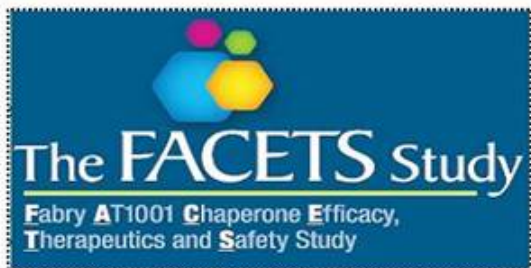


***Migalastat HCl Monotherapy  
for Fabry Disease***



# Global Registration Studies

Assembling Robust Dataset to Maximize Chances for Global Approvals of Migalastat HCl Monotherapy for Fabry Patients with Amenable Mutations



- Placebo-controlled (6 months)
- 67 patients naïve to ERT
- 6-month surrogate primary endpoint: kidney GL-3 (reported 4Q12)
- 12-month biopsy and 24-month clinical data (expected 2Q14)

## THE ATTRACT STUDY

AT1001 Therapy Compared to Enzyme Replacement in Fabry Patients with AT1001-responsive Mutations: a Global Clinical Trial

- ERT switch study
- 60 patients (1.5:1 randomization)
- 18 Month clinical endpoint: kidney function (GFR)
- Data expected 3Q14

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# Global Regulatory Strategy

- Totality of clinical data
- 7+ years of data in extension studies
- Complete data from Phase 3 Studies (011 and 012)

- Clear regulatory pathway
- Non-inferiority to ERT (Study 012)

99 patients on migalastat today

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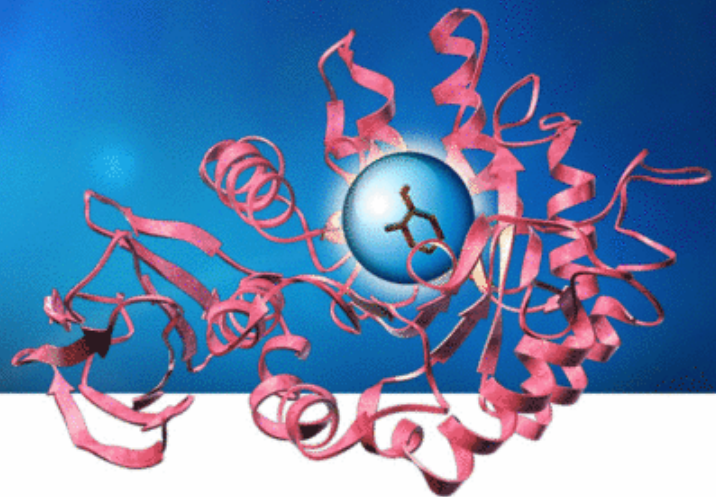


# Migalastat HCl Monotherapy: Key Milestones

(Preliminary Results)

Timing	Milestone	
3Q09	Phase 3 Study 011 initiation	✓
3Q11	Phase 3 Study 012 initiation	✓
4Q12	Interim 6-month data from first Phase 3 Study (011)	✓
2Q13	FDA meeting (Type C)	✓
<b>2Q14</b>	<b>12-month Study 011 data (kidney biopsies)</b>	
<b>2Q14</b>	<b>24-month Study 011 data (clinical outcomes)</b>	
<b>2H14</b>	<b>18-month Study 012 data (kidney function)</b>	

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***Financial Overview and  
Upcoming Milestones***

# Current Financial Picture

With Successful Completion of \$40M in Equity and Debt Financing in 4Q13, Amicus Has Cash into 2H15

## Financial Position

December 31 cash: \$82.0M

2014 net cash spend: \$54-\$59M

Cash runway: 2H15

## Capitalization

Shares outstanding: 64,360,571

# 2014 Highlights



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# Anticipated 2014 Milestones

1H14

2H14

Additional preclinical data for Fabry and Pompe Next-Gen ERTs

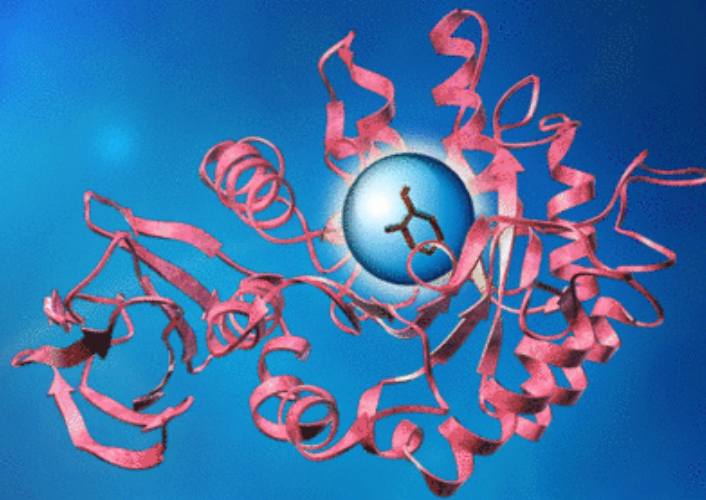
Complete 12- **and** 24-Month Data from Phase 3 Fabry Monotherapy Study 011

Next-Gen Fabry ERT Entry into Clinic

Top-Line Data from Phase 3 Monotherapy Study 012

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**32<sup>nd</sup> Annual J.P. Morgan  
Healthcare Conference**

*January 16, 2014*

*at the forefront of therapies  
for rare and orphan diseases*



## Amicus Therapeutics Provides Full-Year 2014 Strategic Outlook and Financial Guidance

### 3 Next-Generation Enzyme Replacement Therapies (ERTs) Expected to Enter Clinic in Next 3 Years, Beginning in 2014

#### FY14 Cash Spend Guidance of \$54-\$59 Million — Current Cash Expected to Fund Operations into 2H15

CRANBURY, NJ, January 13, 2014 — Amicus Therapeutics (Nasdaq: FOLD), a biopharmaceutical company at the forefront of therapies for rare and orphan diseases, today provided its full-year 2014 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 32nd Annual J.P. Morgan Healthcare Conference on Thursday, January 16, 2014 at 11:30 a.m. PT (2:30 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.

#### Key Highlights:

- Amicus announces “3-in-3” strategy to bring three next-generation Enzyme Replacement Therapies (ERTs) expected to enter the clinic in next three years with lead programs in Fabry, Pompe and Mucopolysaccharidosis I (MPS I)
- Preclinical proof-of-concept data for next-generation ERTs for Fabry and Pompe to be featured at Lysosomal Disease Network WORLD Symposium and American College of Medical Genetics (ACMG) meetings in 1H14
- Data from global registration studies of migalastat HCl monotherapy for Fabry disease expected to include complete 12- and 24-month data from Study 011 in 2Q14 and 18-month clinical data from Study 012 in 2H14
- Current cash projected to fund operating plan into 2H15

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc., stated, “As we enter 2014, Amicus is strongly positioned to continue our focus on advancing a pipeline of next-generation enzyme replacement therapies for patients with lysosomal storage diseases. There continues to be significant unmet medical needs in how we treat these diseases today. We believe that our CHART platform together with our Callidus technology provide a unique tool set to enhance enzyme activity, increase enzyme uptake into tissues, and potentially address the tolerability and immunogenicity associated with

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current ERTs. With the strengthening of our biologics business strategy, we are introducing the Amicus 3-in-3 strategy: advancing three next-generation ERTs into the clinic in the next three years.”

Mr. Crowley continued, “We are well-capitalized to move these programs forward and expect our current cash position to fund our operating plan through many value-creating inflection points, including our Fabry next-generation ERT into the clinic this year and our Pompe next-generation ERT into the clinic in 2015. Both of these programs have the potential to markedly improve outcomes for patients.”

#### 2014 Financial Guidance

Cash, cash equivalents, and marketable securities totaled \$82.0 million at December 31, 2013 compared to \$99.1 million at December 31, 2012. The Company's balance sheet was strengthened in the fourth quarter of 2013 with a \$15.0 million equity financing and a \$25.0 million debt financing under which \$15.0 million was drawn and \$10.0 million remains available. Amicus expects full-year 2014 net cash spend between \$54 million and \$59 million. The current cash position is projected to fund operations into the second half of 2015.

#### Program Updates

Amicus owns exclusive global rights to its next-generation ERTs, as well as all applications of its CHART and Callidus technology platforms. In each CHART program, a unique pharmacological chaperone is designed to bind to and stabilize a specific therapeutic enzyme in its properly folded and active form. Through its purchase of Callidus Biopharma, Amicus has also acquired a differentiated peptide tagging technology that can be used to uniquely engineer bio-better ERTs. These platform technologies provide a complementary tool set to design next-generation therapies for enhanced tissue uptake of active enzyme, greater lysosomal activity, more reduction of substrate, and lower immunogenicity compared to current standard of care ERTs.

#### **Next-Generation ERT for Pompe Disease**

Amicus is advancing a recombinant human acid-alpha glucosidase (rhGAA) for Pompe disease into late preclinical development. The Company's acquisition of Callidus Biopharma, brings a differentiated Pompe ERT, designated AT-B200, with a unique carbohydrate structure. In preclinical studies AT-B200 has shown superior uptake and activity when compared to current standard of care. This ERT may be further improved through co-formulation with Amicus' pharmacological chaperone AT2220 and through the application of the Callidus enzyme targeting technology.

#### **Next-Generation ERT for Fabry Disease**

Amicus' next-generation ERT for Fabry disease consists of a proprietary recombinant human alpha-Gal A enzyme co-formulated with the novel small molecule pharmacological chaperone migalastat HCl. In combination with ERT, migalastat HCl is designed to bind to and stabilize infused alpha-Gal A enzyme, independent of a patient's genetic mutation. Amicus believes this approach has the potential to benefit all patients with Fabry disease. This chaperone-ERT co-formulated product is expected to enter the clinic in the second half of 2014.



## **Next-Generation ERT for MPS I**

Amicus is leveraging its CHART platform to develop a proprietary human recombinant alpha-L-iduronidase (rhIDUA) enzyme for MPS I. In support of its development of this next-generation ERT, Amicus has received funding of up to \$250,000 from a private U.S.-based donor that provides medical research grants to find better treatments and cures for rare genetic disorders, including lysosomal storage diseases.

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

Migalastat HCl monotherapy is being investigated in two ongoing Phase 3 studies for Fabry patients with amenable mutations. Interim 6-month data from the first ongoing Phase 3 study (Study 011) have been reported, and 12-month data from this study are now anticipated in the second quarter of 2014. Top-line, 18-month clinical data from the second ongoing Phase 3 study (Study 012) continue to be anticipated in the second half of 2014.

## **Novel Small Molecules for Parkinson's Disease**

In September 2013 Amicus and Biogen Idec entered a multi-year collaboration to discover of a new class of small molecules that target the GCase enzyme, for further development and commercialization by Biogen Idec. Biogen Idec is responsible for funding all discovery, development, and commercialization activities. In addition Amicus will be reimbursed for all full-time employees working on the project. Amicus is also eligible to receive development and regulatory milestones, as well as modest royalties on global net sales.

## **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 include the small molecule pharmacological chaperones migalastat HCl as a monotherapy and in combination with enzyme replacement therapy (ERT) for Fabry disease; and AT2220 (duvoglustat HCl) in combination with ERT for Pompe disease.

## **About Chaperone-Advanced Replacement Therapy (CHART)**

The Chaperone-Advanced Replacement Therapy (CHART™) platform combines unique pharmacological chaperones with enzyme replacement therapies (ERTs) for lysosomal storage diseases (LSDs). Amicus is leveraging the CHART platform to develop proprietary next-generation therapies that consist of lysosomal enzymes co-formulated with pharmacological chaperones.

In a chaperone-advanced replacement therapy, a unique pharmacological chaperone is designed to bind to and stabilize a specific therapeutic enzyme in its properly folded and active form. This proposed CHART mechanism may allow for enhanced tissue uptake of active enzyme, greater lysosomal activity, more reduction of substrate, and lower immunogenicity compared to ERT alone. Improvements in enzyme stability may also enable more convenient delivery of next-generation therapies.

## **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, cash runway, 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

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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 Annual Report on Form 10-K for the year ended December 31, 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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