Amicus Therapeutics

Jefferies 2011 Global Healthcare Conference

June 7, 2011

Matthew R. Patterson
President and Acting CEO

At the Forefront of Therapies for Rare Diseases™

Nasdaq: FOLD
www.amicustherapeutics.com

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 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.

Industry Momentum in Rare Diseases

THE WALL STREET JOURNAL.
WSJ.com

HEALTH INDUSTRY | SEPTEMBER 1, 2010, 10:38 A.M. ET

Pfizer Agrees to Acquire Drug Developer FoldRx

The New York Times

Novartis takes rare road to cures

By Tom Wright
Published: Friday, July 8, 2005

Acceleron, Shire sign pact

Boston Business Journal - by Michelle Lang

Date: Thursday, September 9, 2010, 10:05am EDT - Last Modified: Thursday, September 9, 2010, 10:25am EDT

BIOWORLD®

Rare Disease is the Place to be

Amicus Lands $230M Deal for Fabry Chaperone Amigal

Bloomberg Businessweek
Available on the iPad

THE ASSOCIATED PRESS July 2, 2010, 9:11AM ET

Eli Lilly acquires biotech drug developer Alnara

InPharm

Pfizer forms rare diseases unit

By *Dominic Tyer*
Created *15/06/2010 - 08:46*

BIOWORLD®

Protalix: $115M Gaucher's Deal with Pfizer is Just the Beginning

By Trista Morrison

Amicus Therapeutics

Amicus: Building Shareholder Value in 2011

At the Forefront of Therapies for Rare Diseases™

Novel Pharmacological Chaperone Technology Platform

Advanced Product Pipeline

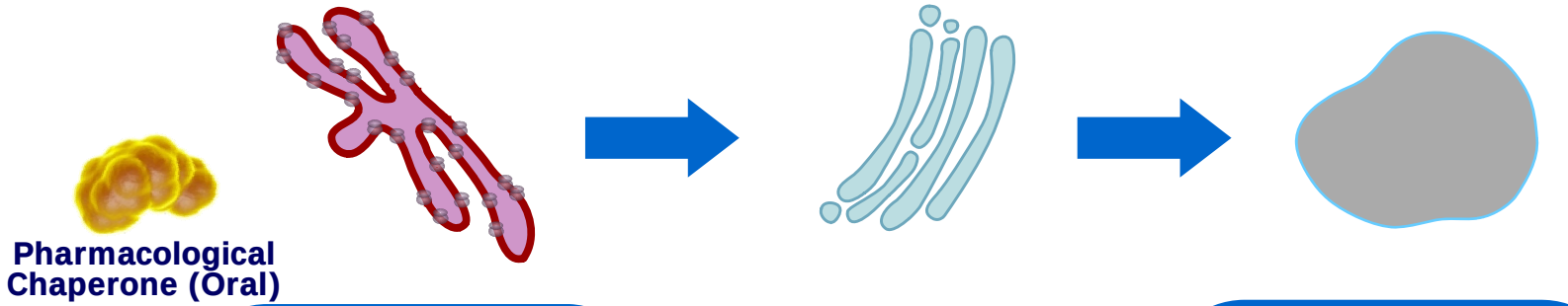
Partnership with GSK Rare Diseases

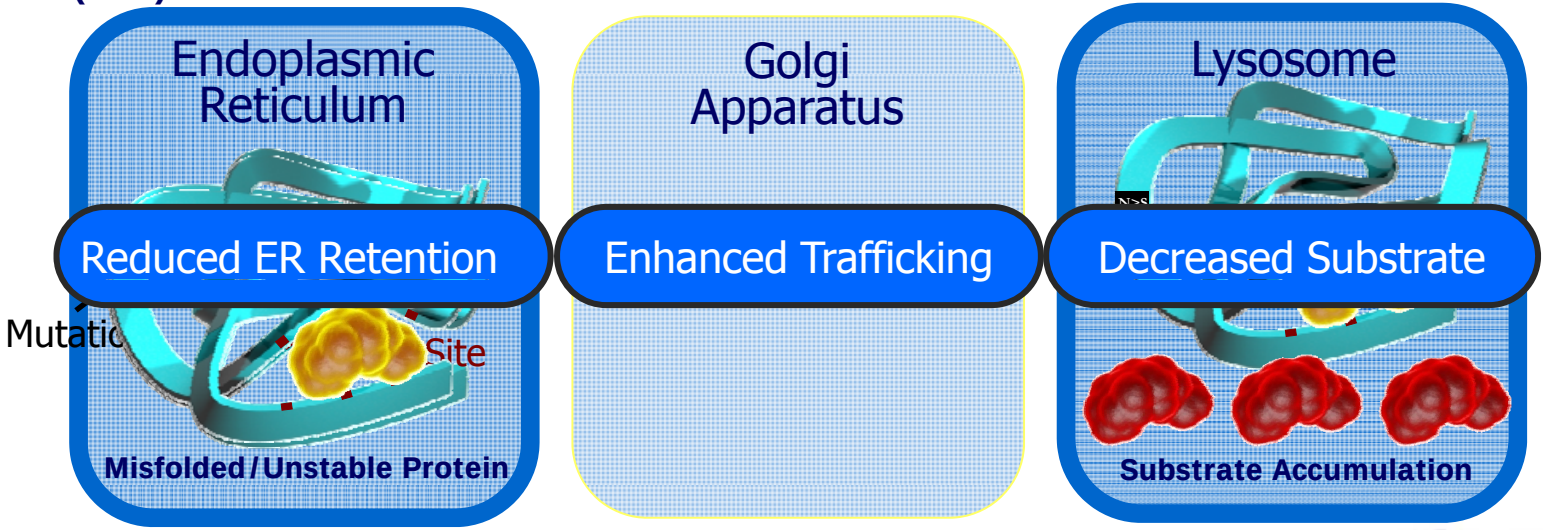
Strong Financial Position

Amicus Therapeutics

Replacing ERTs for Lysosomal Storage Disorders
Pharmacological Chaperone Monotherapy

Next Generation Therapy: replacing ERT
Protein folding & pharmacogenetics

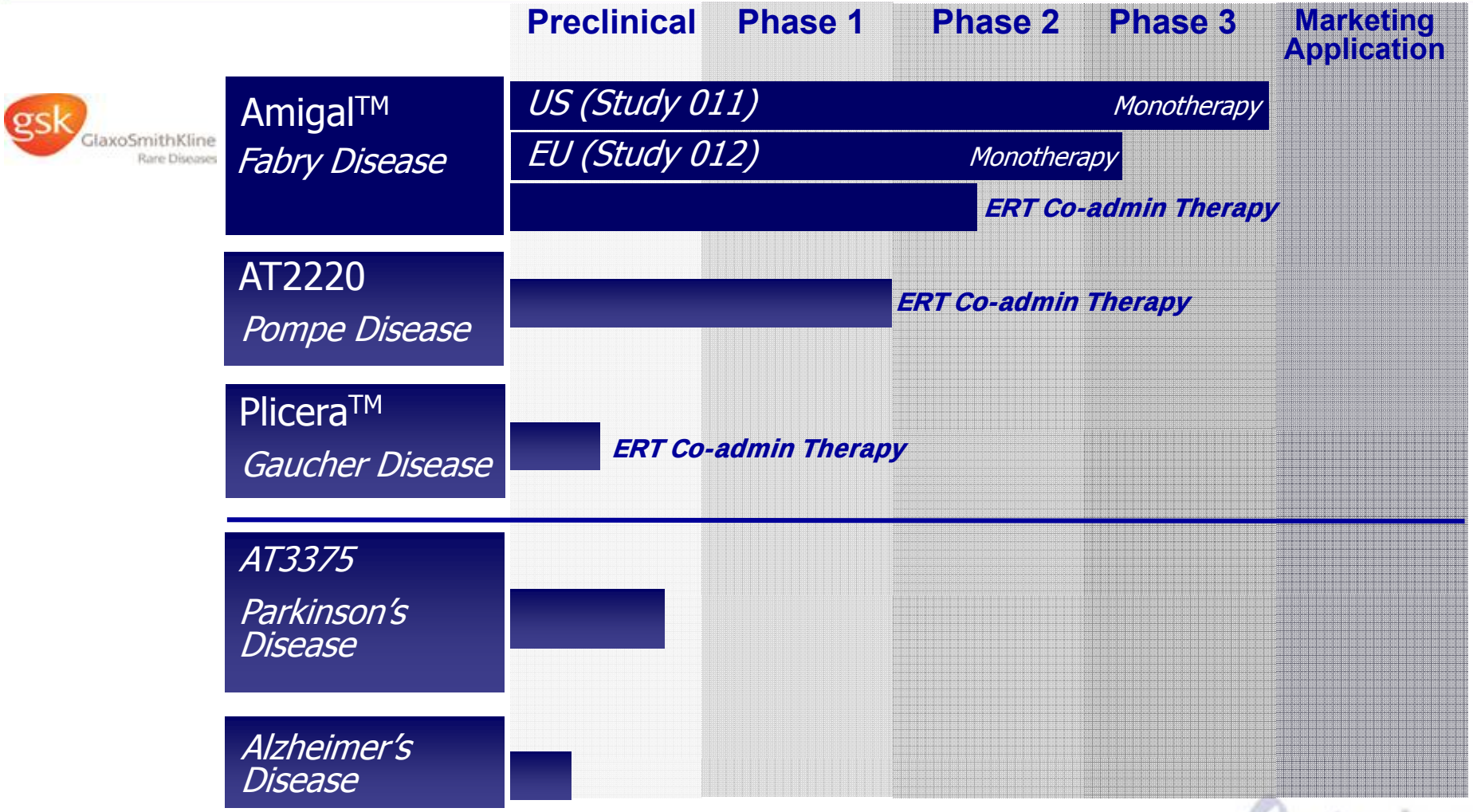




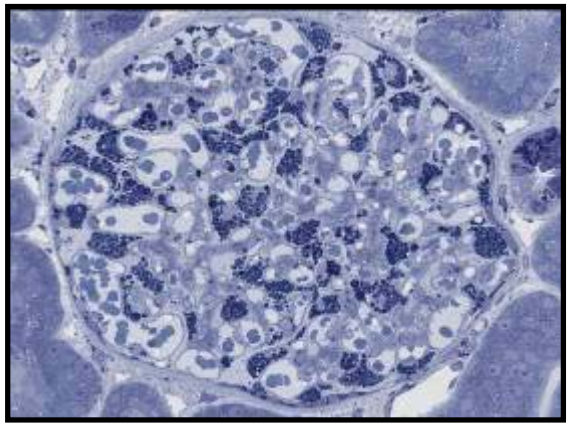
~50% of Fabry patients

Amicus Therapeutics

Advanced Product Pipeline
Building Significant Rare Disease Franchise



Amigal for Fabry Disease
Disease Overview

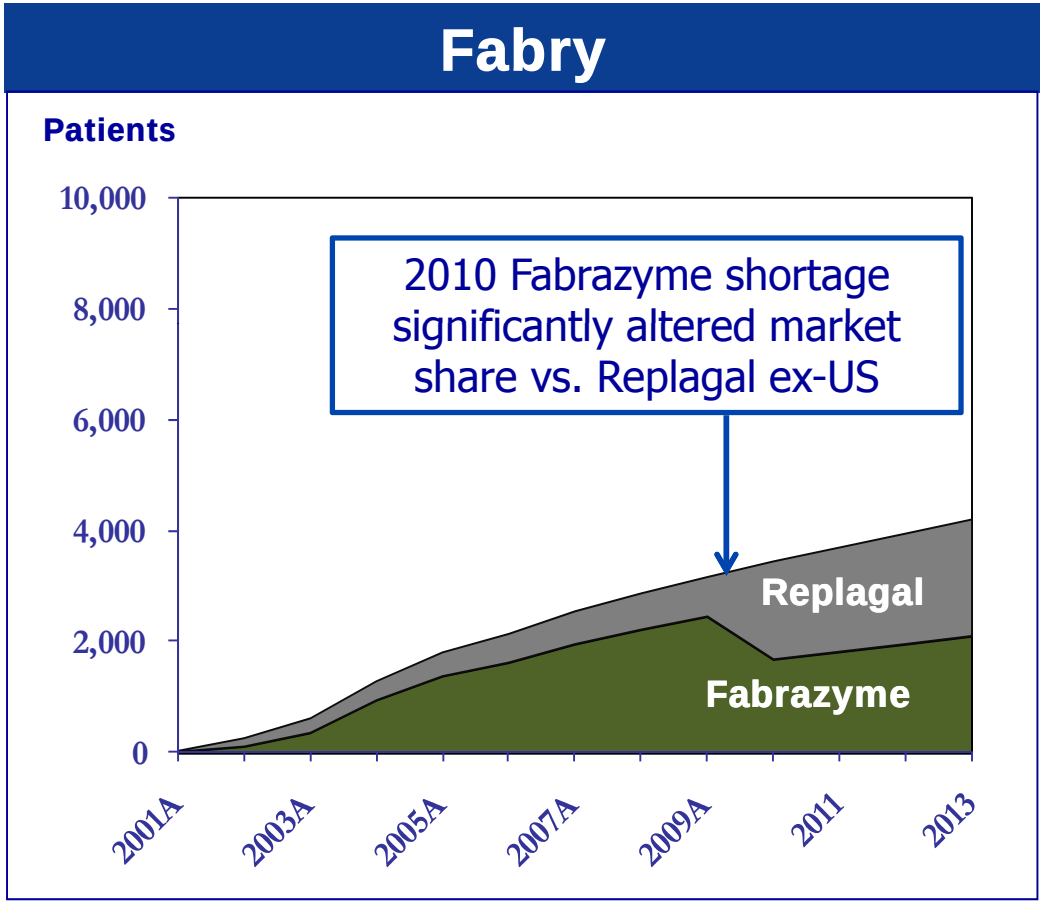


- Lysosomal Storage Disease
- 5,000 – 10,000 patients worldwide
- Fabrazyme® and Replagal® ERTs current standard of care
- Males and Females

- GL-3 substrate accumulation
- Kidney, Heart and Brain
- Fatal

Amicus Therapeutics

Worldwide Fabry Market
Current Landscape



- $800MM in 2011 revenue projected (after shortage resolved)
  - 2010 revenue $529M
  - 2009 revenue $625M
- Shortage lowered sales but growth in treated patients continued
- Equal populations of males and females in patient registries
- Ratio of treated males:females was ~50:50 prior to shortage and is 65:35[4] today
- Significant undiagnosed late onset population[4]

Sources:

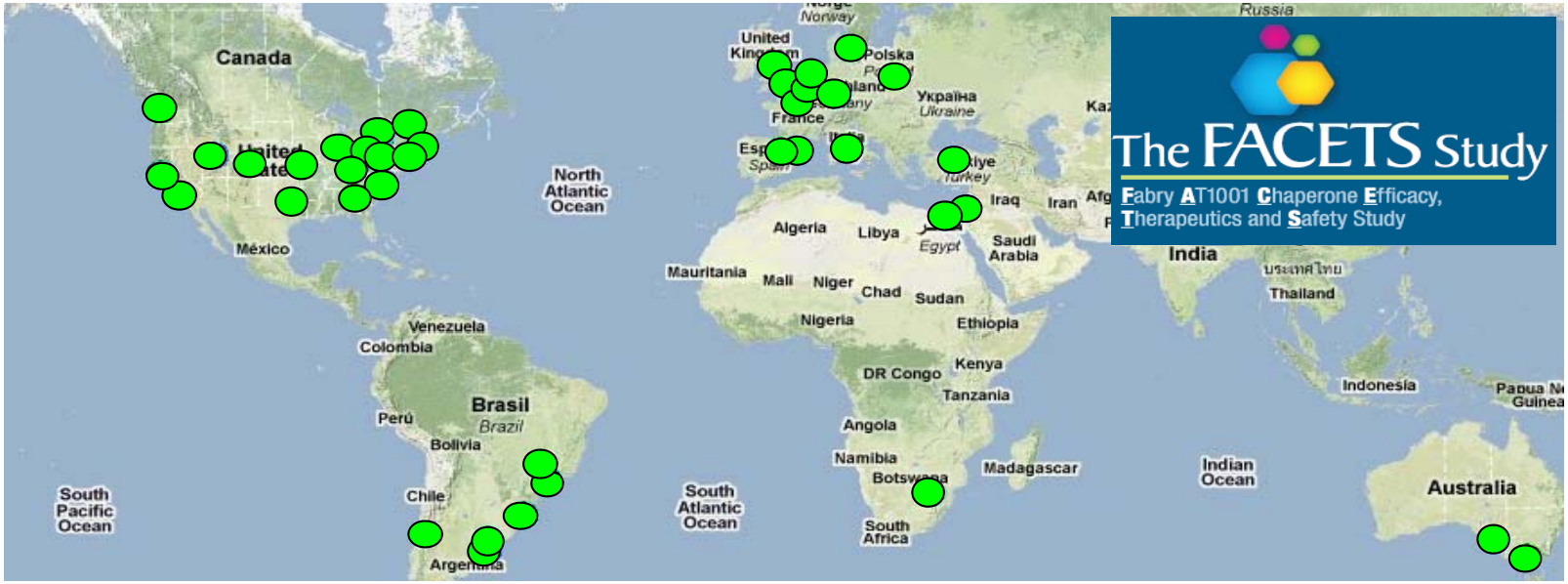
1. GENZ presentation at JP Morgan Conference Jan '10 plus extrapolation of Replagal 2009 revenues; forecast doesn't include US approval of Replagal
2. Estimated change in market share driven by global supply shortage
3. Analyst projected CAGR extrapolated based on JP Morgan, AG Edwards, Collins Stewart, SG Cowen and Credit Suisse projections
4. Fabrazyme Registry 2010, FOS Registry 2009, Canadian Registry 2010; Spada et al

Amigal for Fabry Disease
Program Overview

Lead development program
Global collaboration with GSK Rare Diseases

- Small molecule for oral administration
- First in man: 2005
- Cumulative 85+ patient-years of data
- No drug-related serious adverse events and no adverse event trends
- 17 patients remain in Phase 2 extension study
  - 5 patients > 4 years, 12 patients > 3 years
  - Encouraging safety and renal function data
- Phase 3 registration studies ongoing
- First-in-man Phase 2 study of Amigal co-administered with ERT underway

Amigal for Fabry Disease
Phase 3 U.S. Registration (Study 011)



Study Overview

- Randomized, double-blind, placebo-controlled
- 60 patients (males and females)
- 6-month treatment period
- Endpoints:
  - **Primary**: ≥50% reduction in interstitial capillary GL-3 in kidney biopsy
  - **Secondary**: ≥50% reduction in urine GL-3
- Enriched patient population

Status Update

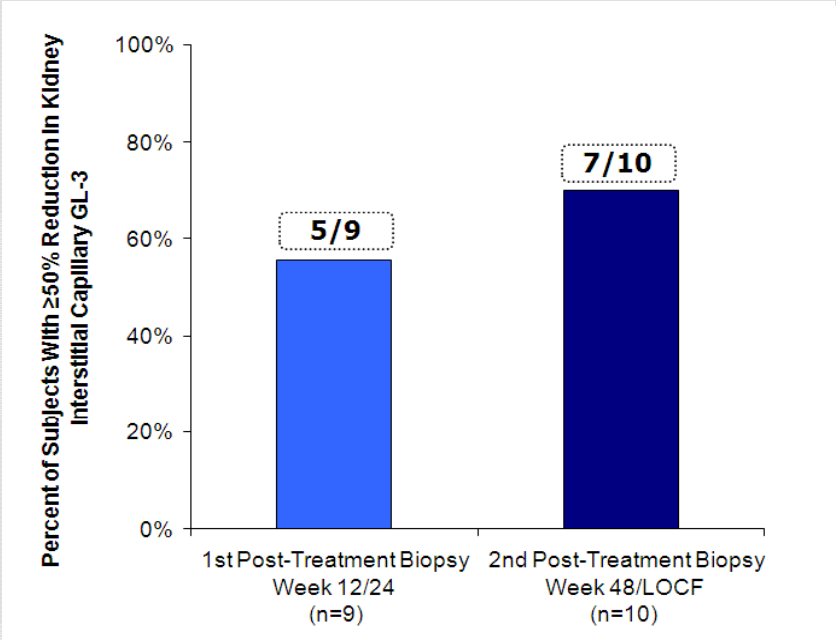
- 37 sites initiated globally
- Nearing completion of enrollment
- Final enrollment expected 3Q11

Amigal for Fabry Disease
Phase 2 Data – Surrogate Endpoints

GL-3 substrate reduced

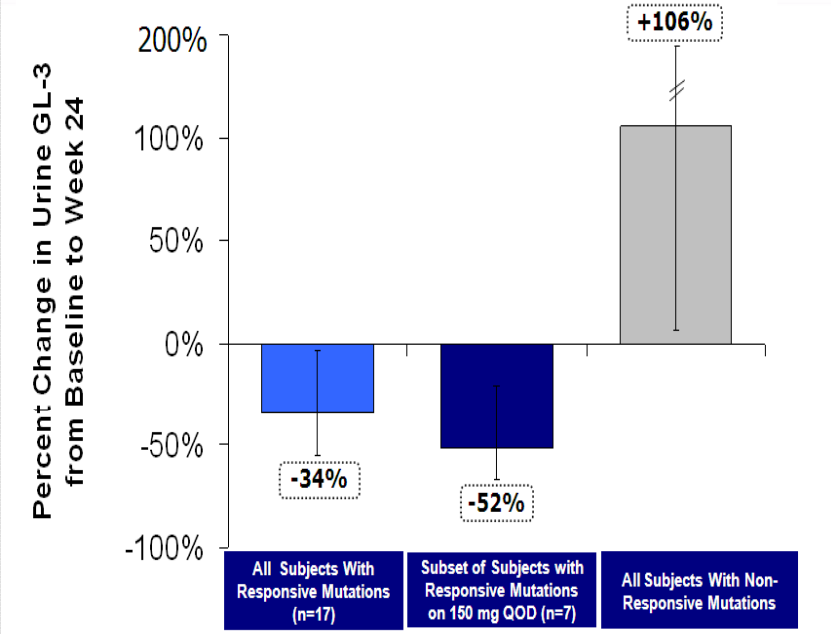
Primary Surrogate

Kidney Interstitial Capillary GL-3



Secondary Surrogate

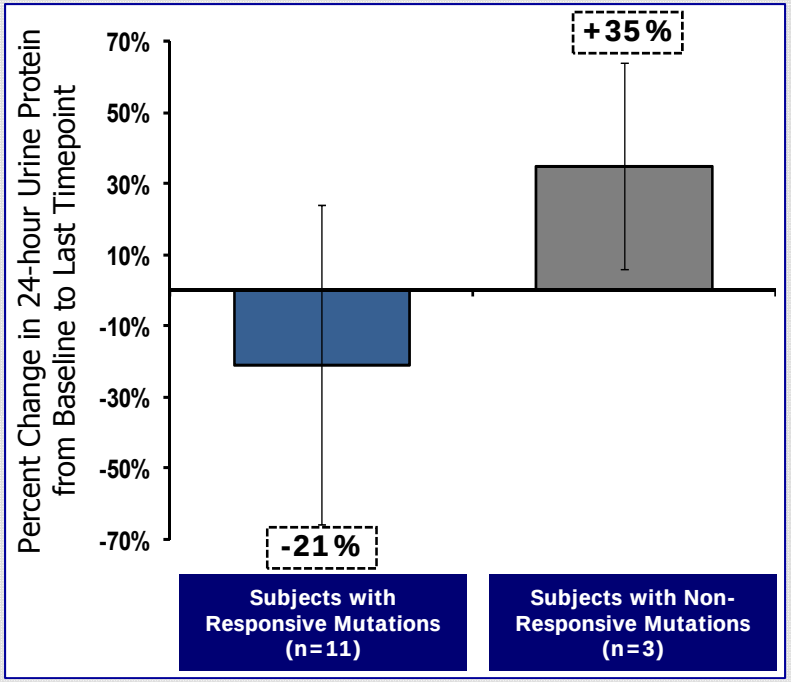
Urine GL-3



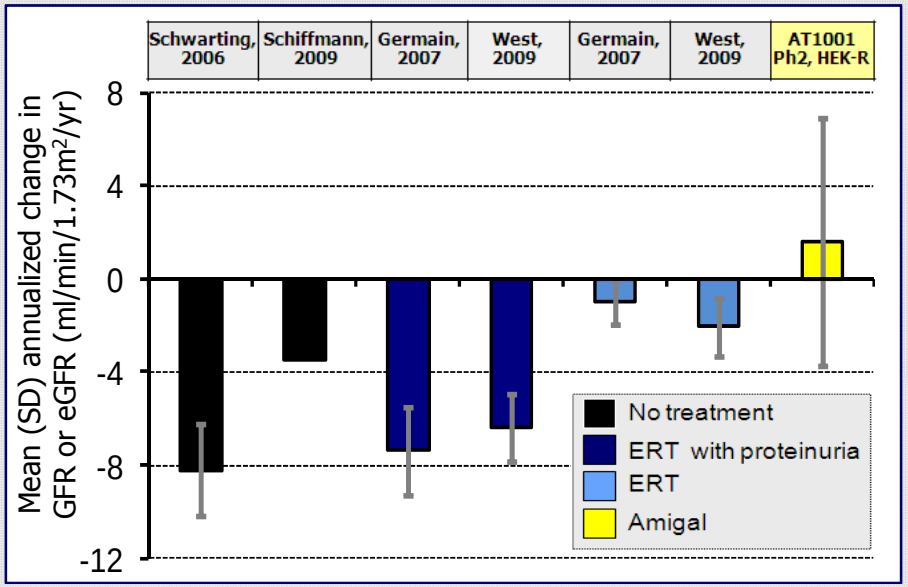
Amigal for Fabry Disease
Phase 2 Data – Clinical Endpoints

Renal Function

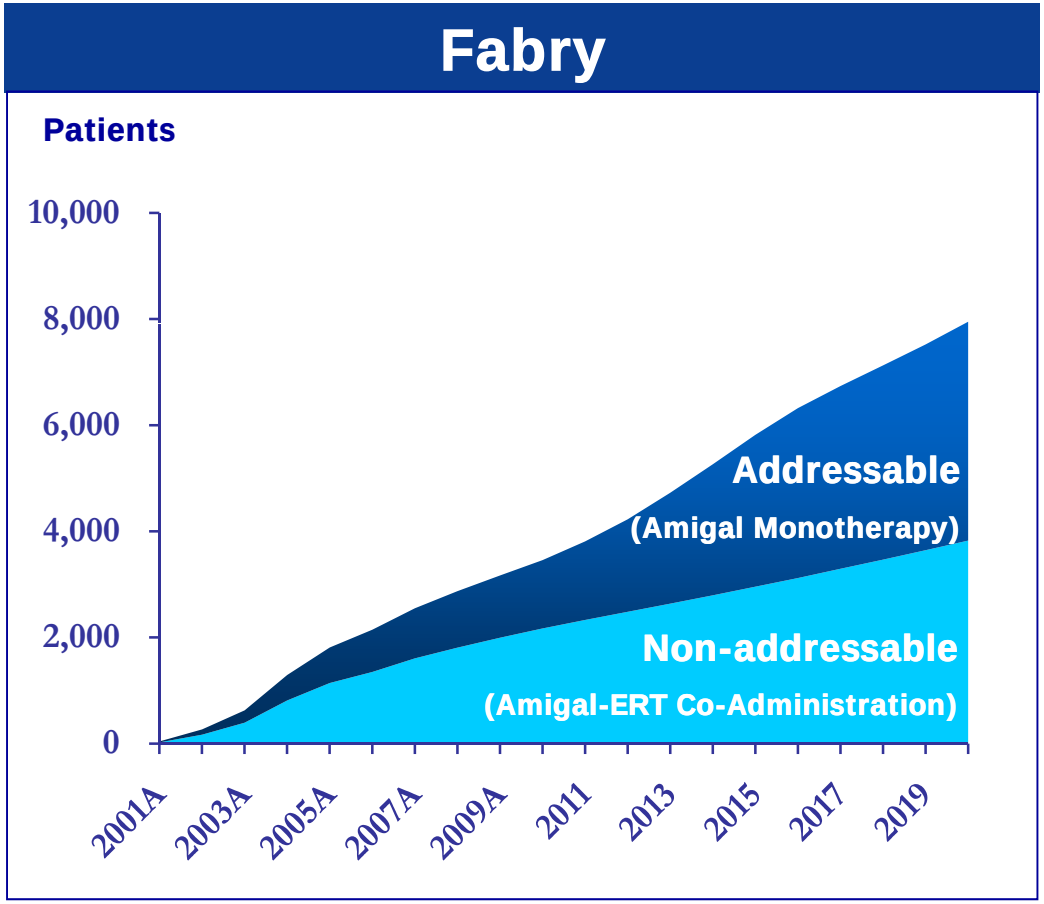
Proteinuria



eGFR



Worldwide Fabry Market
Projected Future Growth



Assumptions

- Significant market growth expected from increased diagnosis and treatment of females and late-onset males
- Higher percentage of mutations addressable by Amigal in females and late-onset males
- Additional market growth driven by availability of an oral agent

Sources:

1. Analyst projected CAGR extrapolated based on JP Morgan, AG Edwards, SG Cowen, Collins Stewart and Credit Suisse projections
2. Addressable mutation percentages are estimates

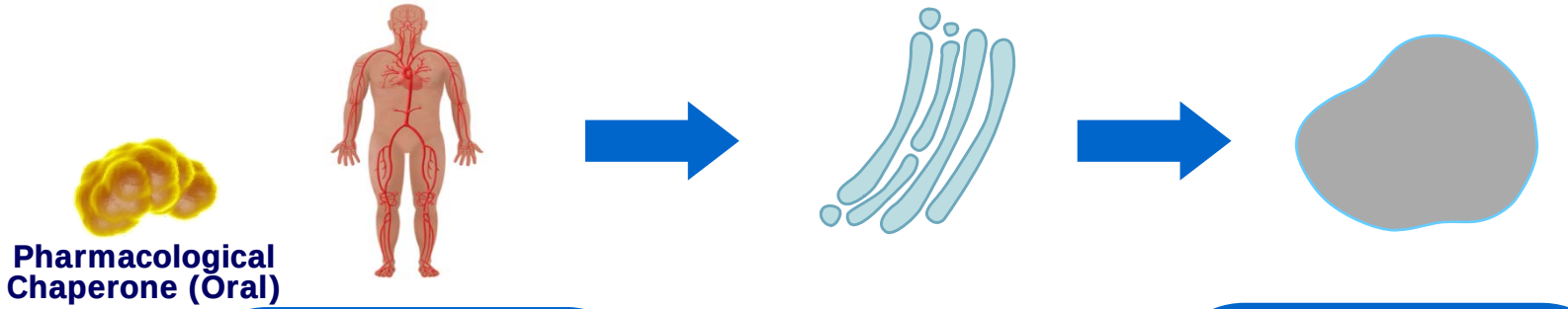
Amicus Therapeutics

Pharmacological Chaperone-ERT Co-administration Therapy

At the Forefront of Therapies for Rare Diseases™

Improving ERTs for Lysosomal Storage Disorders
Pharmacological Chaperone Co-Administration

Next Generation Therapy: improving ERT



~50% of Fabry patients

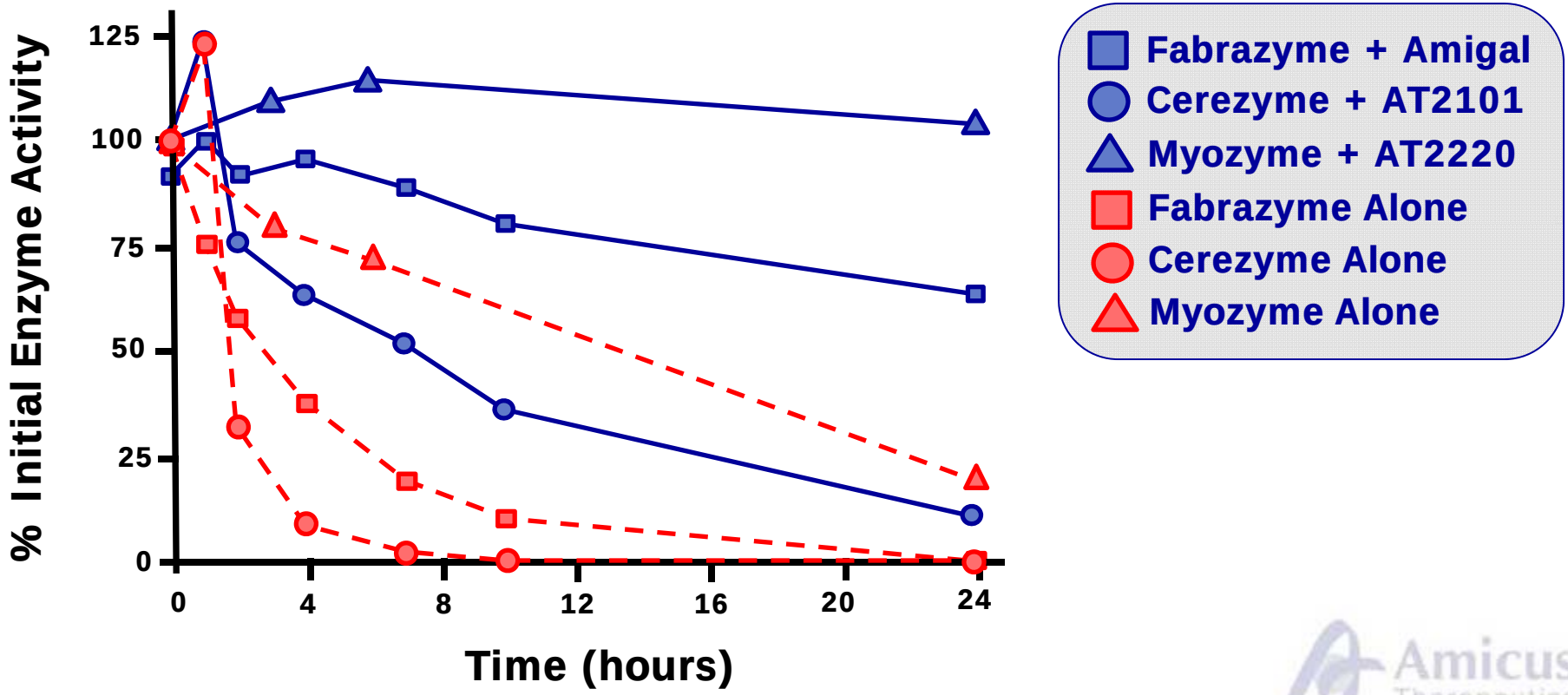
Amicus Therapeutics

Improving ERTs for Lysosomal Storage Disorders

ERTs Denature Rapidly in Blood

Co-Administration: preclinical proof-of-concept

Loss of Activity of ERTs at pH=7.4



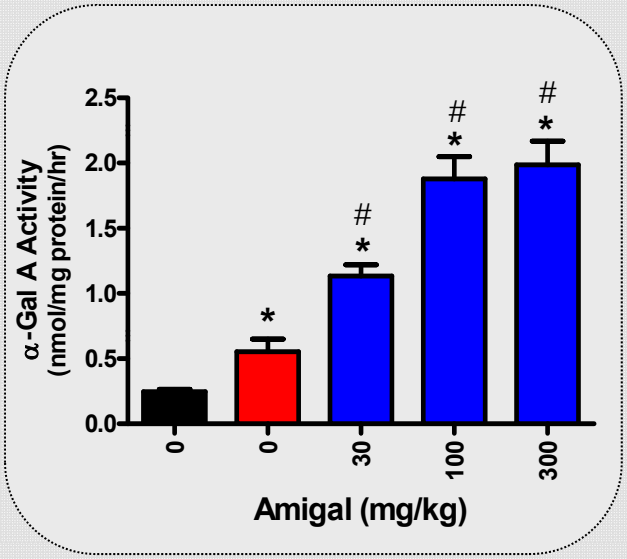
Amicus Therapeutics

Improving ERT for Fabry Disease

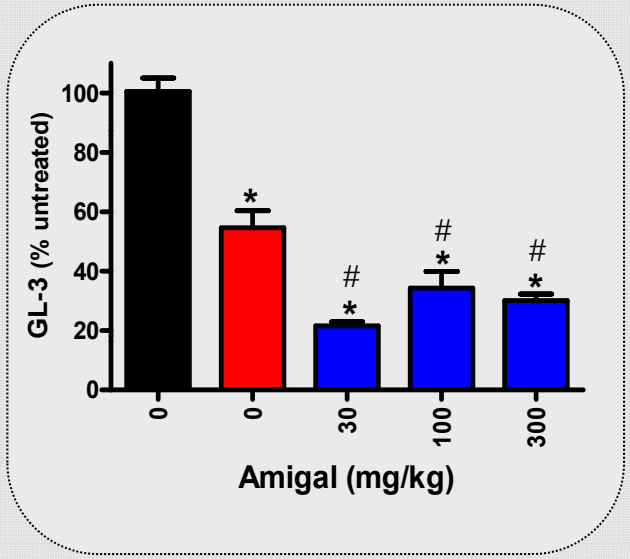
Preclinical Data: Amigal Co-Administered with Fabrazyme

Amigal significantly increases Fabrazyme tissue uptake and markedly reduces GL-3 levels in kidney

Fabrazyme Kidney Tissue Uptake



GL-3 Levels in Kidney



*p value < 0.05 vs. untreated controls
#p value < 0.05 vs. Fabrazyme

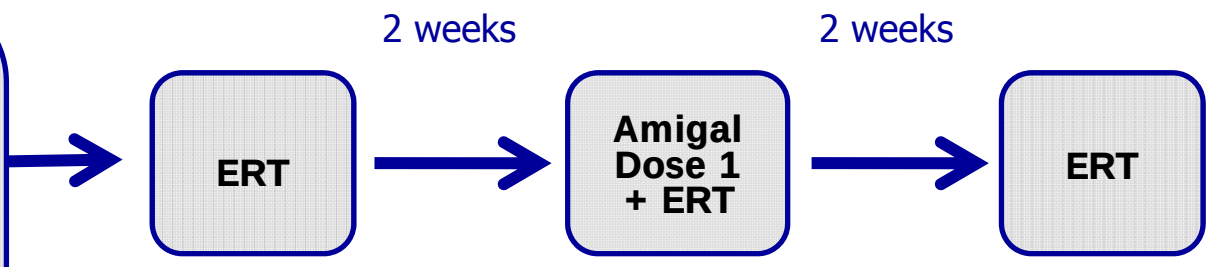
Improving ERT for Fabry Disease

Phase 2 Study: Amigal Co-Administered with Fabrazyme

First-in-man, open-label, single-dose study to evaluate safety and PK/PD

Enrollment

- Up to 18 male patients
- Ages 18-65
- ≥ 1 mo. stable ERT dose, regimen
- Responsive genetic mutation not required
- 2 Amigal dose groups



Plasma PK assessment (over 24 hours)
Skin Biopsy (ERT in tissue)

Amicus Therapeutics

Amigal for Fabry Disease
Complete Market Opportunity

Opportunity to Address ***ALL* Fabry Patients Regardless of Mutation**

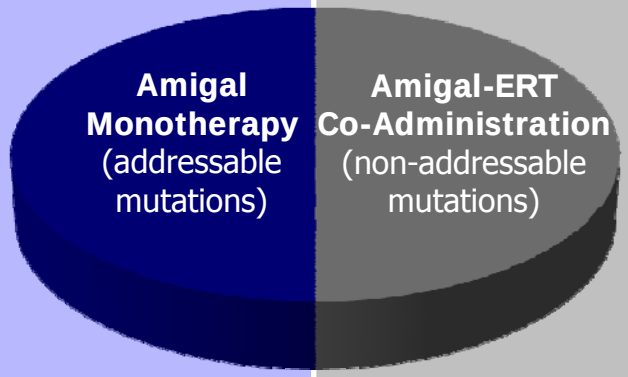
Monotherapy

Oral Amigal

Chaperone endogenous enzyme from ER to lysosome

Potential Advantages

- Oral administration
- Broad tissue distribution
- Chemical synthesis



Co-Administration

Oral Amigal, IV ERT

Chaperone ERT from circulation to lysosome

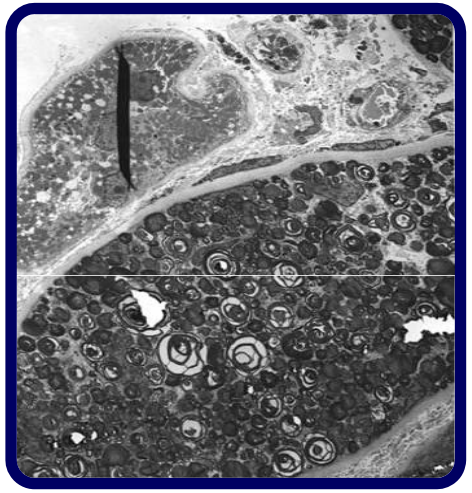
Potential advantages

- Increased tissue uptake
- Reduced immunogenicity
- Reduced dose/frequency

**Extrapolations based on HEK responsiveness and FOS registry data*

Amicus Therapeutics

AT2220 for Pompe Disease
Disease Overview



- 5,000 – 10,000 patients worldwide
- Leads to heart and respiratory failure, muscle degeneration
- >90% of patients have later onset disease
- 2010 revenue of ~$400MM (Myozyme/Lumizyme)

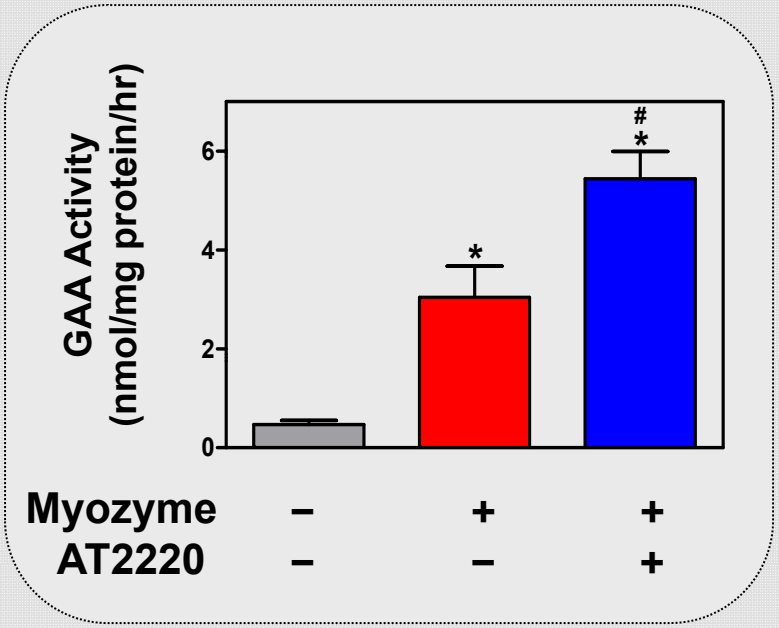
- Glycogen accumulation
  - Heart, skeletal muscles, liver, and nervous system
- Current standard of care: ERTs
  - Moderate clinical benefit
  - Immunogenicity
  - Black box warning for anaphylaxis

Improving ERT for Pompe Disease

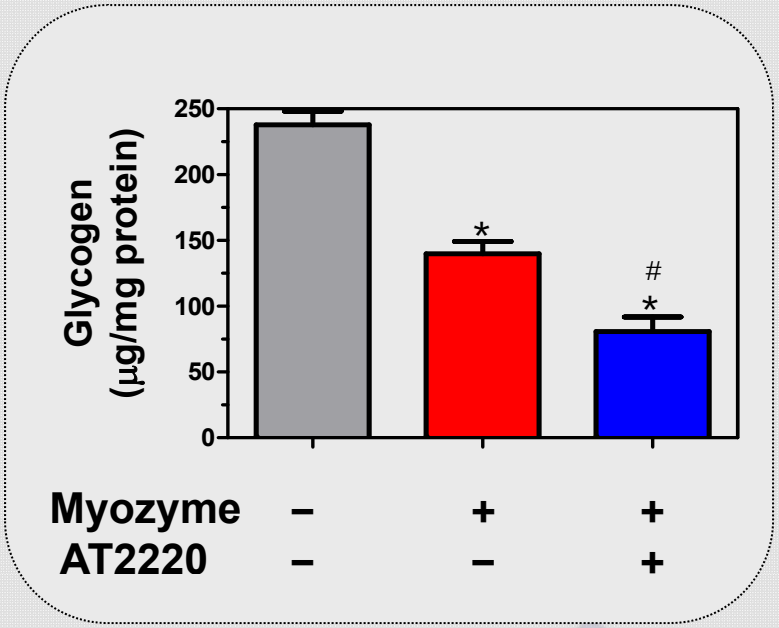
Preclinical Data: AT2220 Co-Administered with Myozyme

Amicus plans to perform a Phase 2 trial of AT2220 co-administered with ERT based on encouraging preclinical data

Myozyme Diaphragm Uptake



Glycogen Levels in Diaphragm



*p value < 0.05 vs. untreated controls
#p value < 0.05 vs. Myozyme

Amicus Therapeutics

Pharmacological Chaperone Technology for Diseases of Neurodegeneration

At the Forefront of Therapies for Rare Diseases™

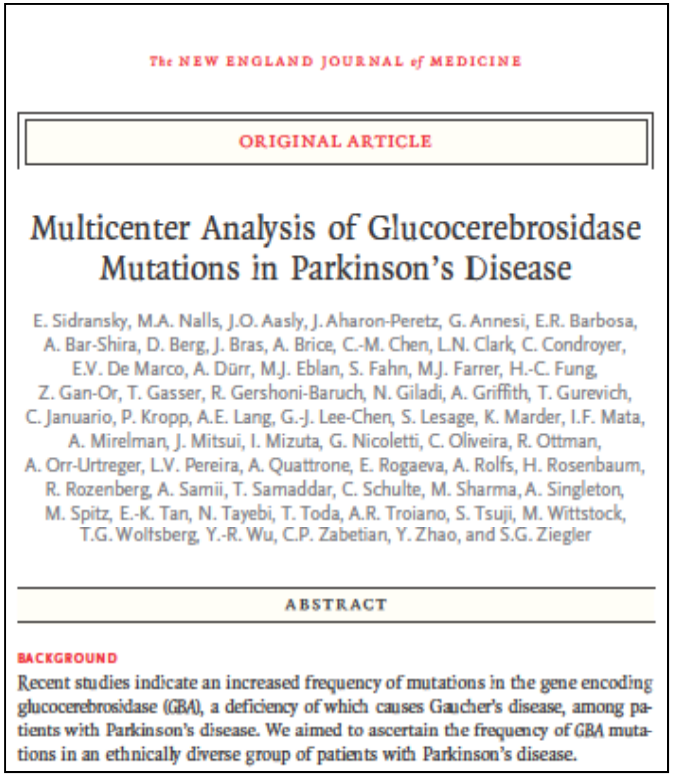
Expansion into Diseases of Neurodegeneration:
Link to Lysosomal Storage Disorders

Pharmacological chaperones for genetically defined sub-populations

- Parkinson's disease
  - Link to GCase enzyme deficient in Gaucher disease
  - Funded in part by grant from Michael J. Fox Foundation
- Alzheimer's disease
  - Link between lysosomal dysfunction and neurodegeneration
  - Funded in part by grant from Alzheimer's Drug Discovery Foundation

Pharmacological Chaperones for Parkinson's
Established Link to Gaucher Disease

Mutations in GCase gene (*GBA*) considered most common genetic risk factor for Parkinson's Disease



The NEW ENGLAND JOURNAL of MEDICINE

ORIGINAL ARTICLE

Multicenter Analysis of Glucocerebrosidase Mutations in Parkinson's Disease

E. Sidransky, M.A. Nalls, J.O. Aasly, J. Aharon-Peretz, G. Annesi, E.R. Barbosa, A. Bar-Shira, D. Berg, J. Bras, A. Brice, C.-M. Chen, L.N. Clark, C. Condroyer, E.V. De Marco, A. Dürr, M.J. Eblan, S. Fahn, M.J. Farrer, H.-C. Fung, Z. Gan-Or, T. Gasser, R. Gershoni-Baruch, N. Giladi, A. Griffith, T. Gurevich, C. Januario, P. Kropp, A.E. Lang, G.-J. Lee-Chen, S. Lesage, K. Marder, I.F. Mata, A. Mirelman, J. Mitsui, I. Mizuta, G. Nicoletti, C. Oliveira, R. Ottman, A. Orr-Urtreger, L.V. Pereira, A. Quattrone, E. Rogaeva, A. Rolfs, H. Rosenbaum, R. Rozenberg, A. Samii, T. Samaddar, C. Schulte, M. Sharma, A. Singleton, M. Spitz, E.-K. Tan, N. Tayebi, T. Toda, A.R. Troiano, S. Tsuji, M. Wittstock, T.G. Wolfsberg, Y.-R. Wu, C.P. Zabetian, Y. Zhao, and S.G. Ziegler

ABSTRACT

BACKGROUND

Recent studies indicate an increased frequency of mutations in the gene encoding glucocerebrosidase (*GBA*), a deficiency of which causes Gaucher's disease, among patients with Parkinson's disease. We aimed to ascertain the frequency of *GBA* mutations in an ethnically diverse group of patients with Parkinson's disease.

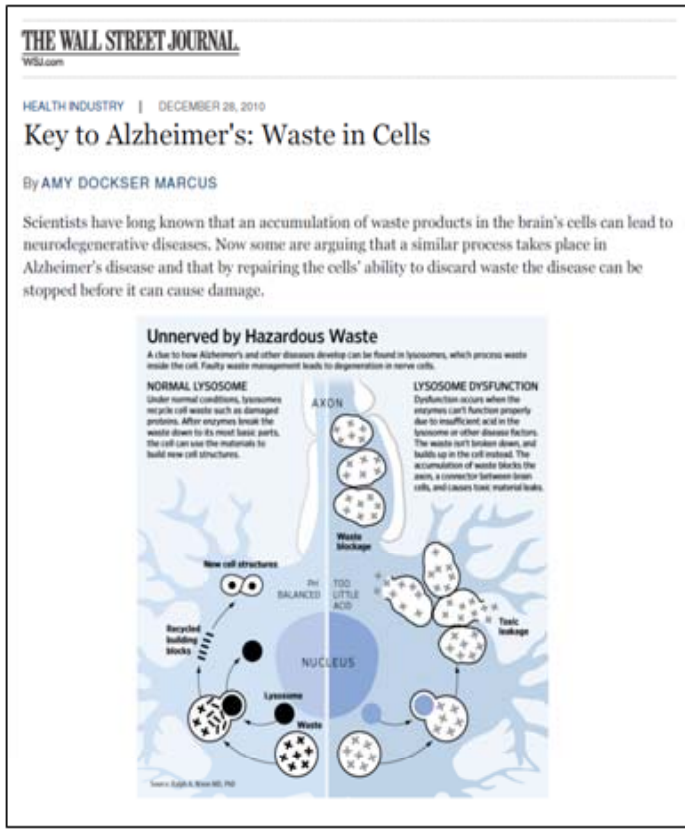
- Gaucher carriers[1]
  - 5x more prevalent in Parkinson's disease population
- Gaucher patients[2]
  - 20-fold risk for developing Parkinson's disease
- Lead pharmacological chaperone: AT3375
  - Targeting GCase for Parkinson's disease
  - Completing preclinical studies, including IND-enabling studies, in 2H11
  - Potential to modify course of disease

[1]Sidransky, New Engl J Med, 2009 Oct 22; 361(17): 1651-61
[2]Bultron, Journal of Inherited Metabolic Disease, 2010, 33(2):167-173

Amicus Therapeutics

Pharmacological Chaperones for Alzheimer's
Link to Lysosomal Storage Disorders

Researching novel approaches for
2 distinct targets and patient populations



THE WALL STREET JOURNAL.
WSJ.com

HEALTH INDUSTRY | DECEMBER 28, 2010

Key to Alzheimer's: Waste in Cells

By AMY DOCKSER MARCUS

Scientists have long known that an accumulation of waste products in the brain's cells can lead to neurodegenerative diseases. Now some are arguing that a similar process takes place in Alzheimer's disease and that by repairing the cells' ability to discard waste the disease can be stopped before it can cause damage.

- Genetic (familial) Alzheimer's disease
  - Presenilin 1 target
  - Missense mutations
  - 50,000-150,000 patients in U.S.
  - Early pre-clinical POC established
- Sporadic Alzheimer's disease
  - Lysosomal enzyme target
  - ~4.5MM patients (U.S.)

Amicus Therapeutics

Amicus Therapeutics

Partnership with GSK Rare Diseases and Financial Outlook

At the Forefront of Therapies for Rare Diseases™

Strong Partnership with GSK Rare Diseases
Exclusive Worldwide Rights for Amigal

Value for Amicus

- Validation for pharmacological chaperone technology and Fabry program
- GSK clinical, regulatory, commercial and manufacturing expertise
- Financial strength and flexibility

Deal Terms

- $30MM upfront license
- $31MM equity investment
- $170MM development + sales milestones
- Global development cost-sharing
  - 50/50 in 2011
  - 75 GSK/25 Amicus in 2012+
- Tiered double-digit royalties

"*Amicus' scientific and clinical expertise in human genetic diseases is among the best in the industry, and we are pleased to be collaborators and investors in this exceptional company.*"

- Marc Dunoyer, Global Head, GSK Rare Diseases

gsk GlaxoSmithKline Rare Diseases

Amicus Therapeutics

Amicus Financial Strength

GSK partnership allows Amicus to fully invest its pipeline while maintaining cash reserves

- Cash balance (3/31/11): $93.8MM
- 2011 Projected Net Spend: $45-55MM
- Projected cash runway: through anticipated Amigal U.S. commercial launch (net of anticipated GSK collaboration payments)

Amicus: Building Shareholder Value in 2011

Recent and Expected Milestones

Recent Milestones

- ✓ Ph 2 Amigal extension data in Fabry Patients out 3-4 years
- ✓ 1st patient in Ph 2 Amigal-ERT co-administration study in Fabry disease
- ✓ Sites opened for Amigal Ph 3 EU study in Fabry disease
- ✓ Ph 2 AT2220-ERT co-administration moving forward in Pompe disease

Upcoming Milestones

- Complete enrollment in Amigal Ph 3 US Study in 3Q11
- 1st patient in Amigal Ph 3 EU study in 2Q-3Q11
- Phase 2 Amigal-ERT co-administration data in 2H11
- 1st patient in Ph 2 AT2220-ERT co-administration study in 3Q11
- Late-stage preclinical POC, including IND-enabling activities, for AT3375 in Parkinson's in 2H11

Amicus: Building Shareholder Value in 2011

Value Proposition

- Leader in rare diseases – validated by strong commercial partner GSK
- Robust development pipeline and technology platform
- ~ $250MM market capitalization
- ~ $93.8MM cash
- Multiple near-term milestones

Amicus Therapeutics

Jefferies 2011 Global Healthcare Conference

June 7, 2011

Matthew R. Patterson
President and Acting CEO

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