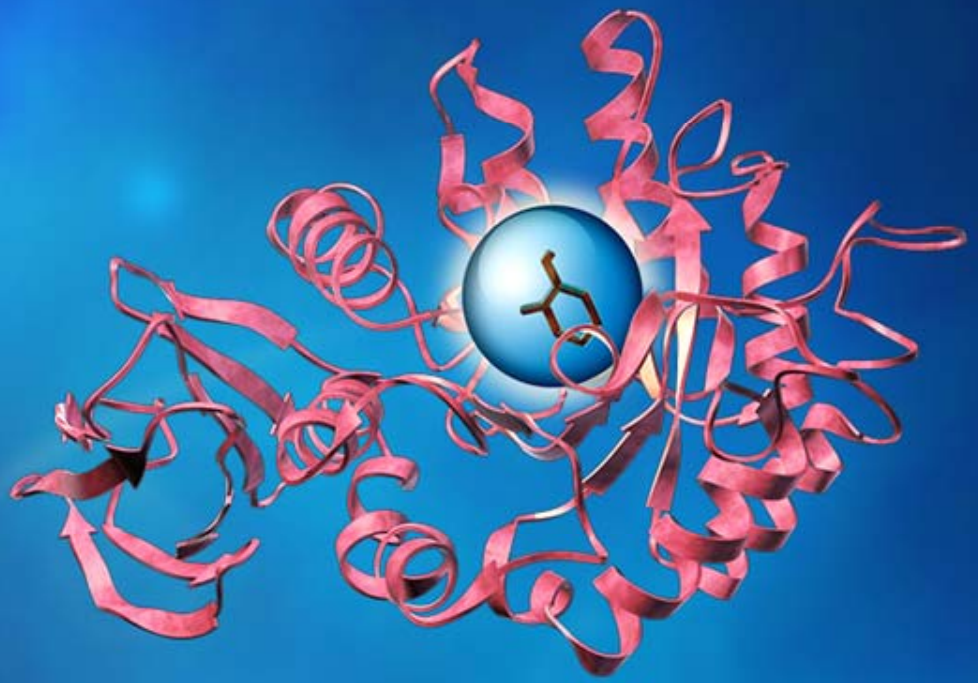
Amicus Therapeutics



UBS Global Healthcare Conference

May 21, 2013

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, 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.

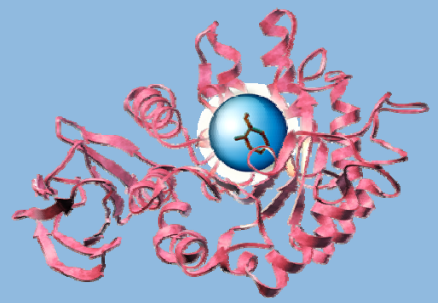
Company Mission

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

2013 Investment Highlights

Amicus Therapeutics

PRODUCTS



PLATFORM

CHART™

CHAPERONE-ADVANCED REPLACEMENT THERAPY

PARTNERSHIPS

gsk GlaxoSmithKline

LAUREATE BIOPHARMA

JCR Pharmaceuticals Co., Ltd.

FINANCIAL STRENGTH

Core Technology and Focus: Potential to Transform LSD Treatments

Monotherapy

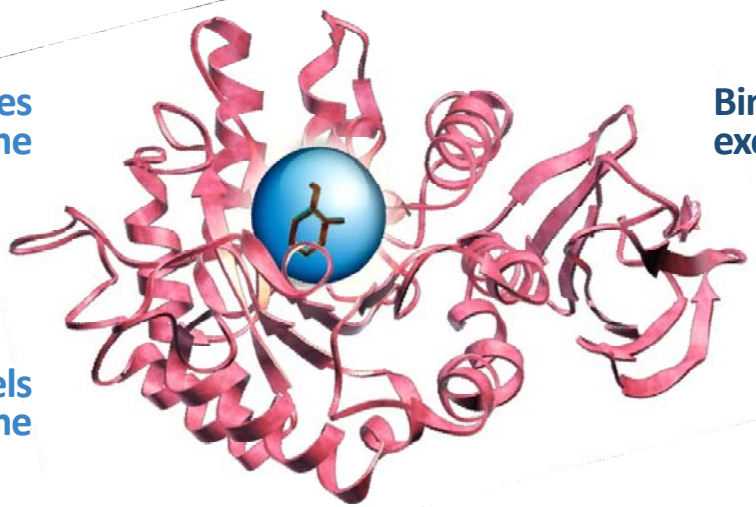
Binds to and stabilizes endogenous target enzyme

Increases lysosomal levels of active enzyme

Combination

Binds to and stabilizes exogenous enzyme (ERT)

Increases tissue uptake of active enzyme



LOWERED SUBSTRATE

Amicus Therapeutics

CHART™: Chaperone-Advanced Replacement Therapy

CHAPERONE-ADVANCED REPLACEMENT THERAPY

CO-ADMINISTRATION
(Chaperones + Marketed ERTs)

NEXT-GENERATION ERTs
(IV Co-formulated Chaperones + Proprietary Enzymes)

NEXT-GENERATION ERTs WITH IMPROVED DELIVERY REGIMEN

Amicus Therapeutics

Advanced Product Pipeline

INDICATION	PRE-CLINICAL	PHASE 1	PHASE 2	PHASE 3	MARKETING APPLICATION	PARTNER
Fabry Disease						
Migalastat HCL						GSK
Parkinson's						
AT3375						Michael J. Fox Foundation

Fabry Disease						
Migalastat HCL + Fabrazyme/Replagal						GSK, JCR Pharmaceuticals
Migalastat HCL + JR-051						
Pompe Disease						
AT2220 + Myozyme/Lumizyme						
AT2220 + Proprietary ERT						
Other LSDs						
Next-Generation ERTs						

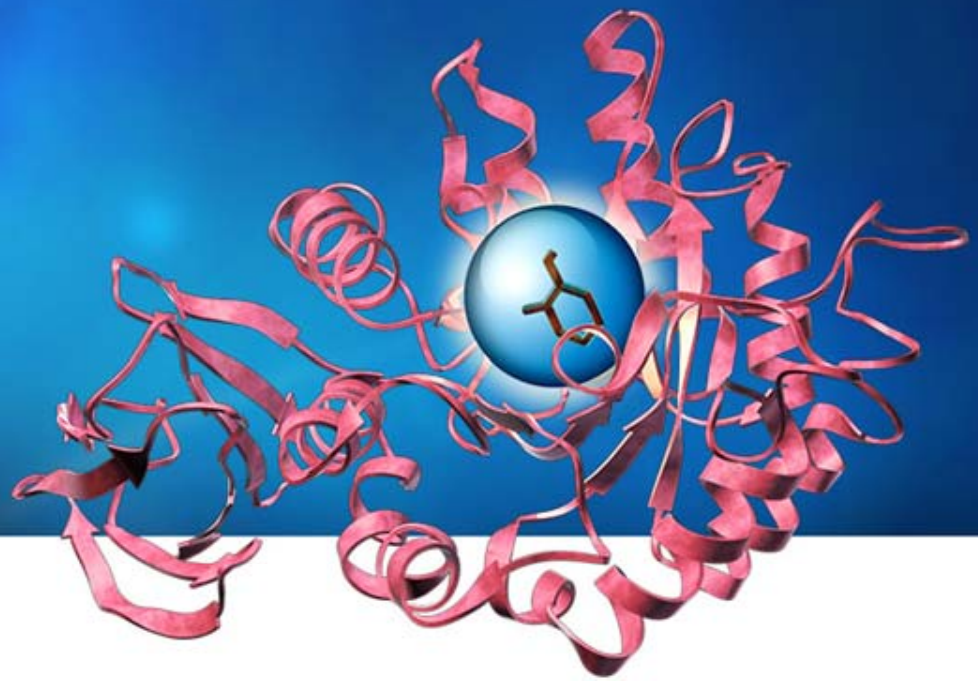
MONOTHERAPY

CO-ADMINISTRATION

NEXT-GENERATION ERTs (co-formulation)

Amicus Therapeutics

Amicus Therapeutics

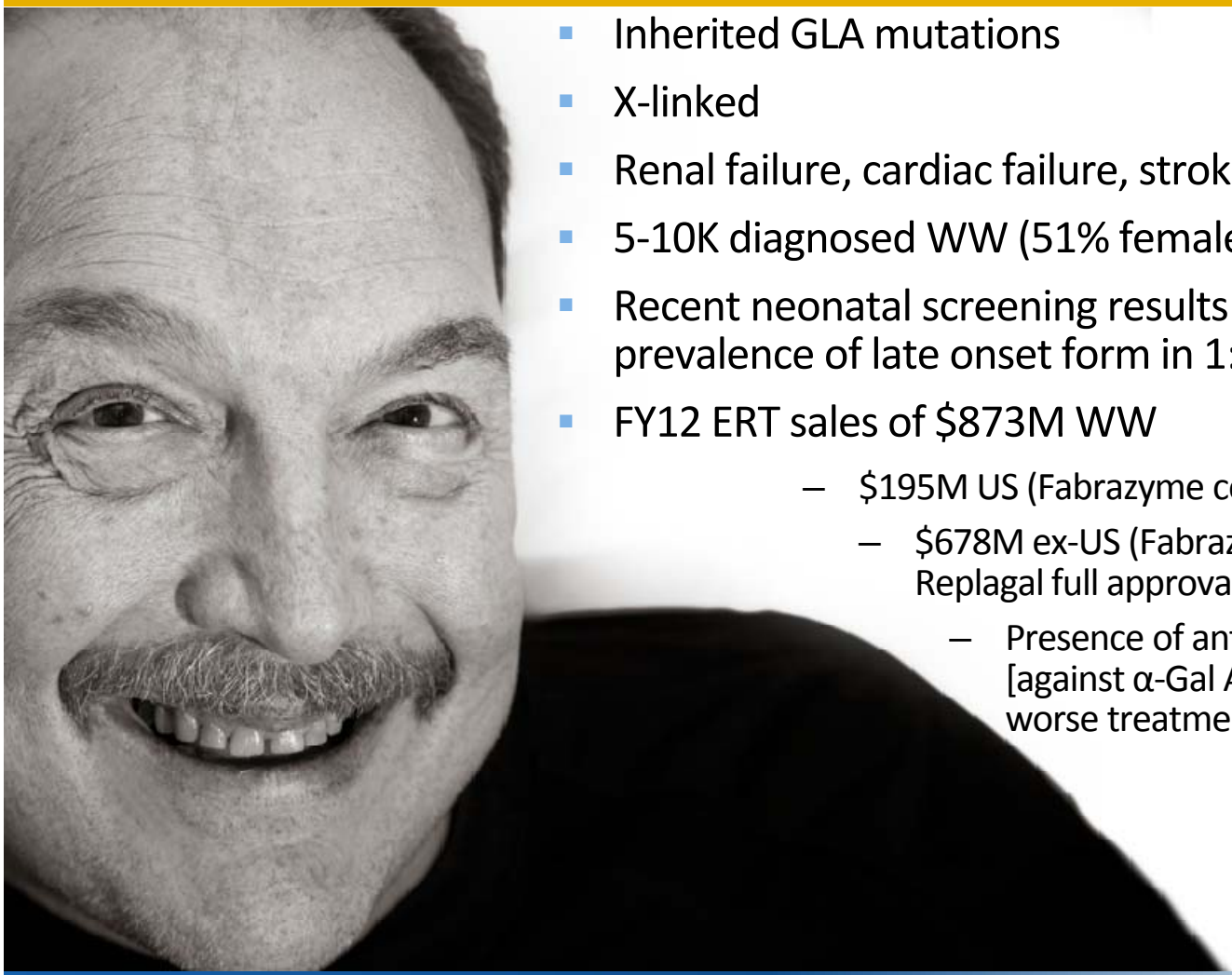


Pharmacological Chaperones

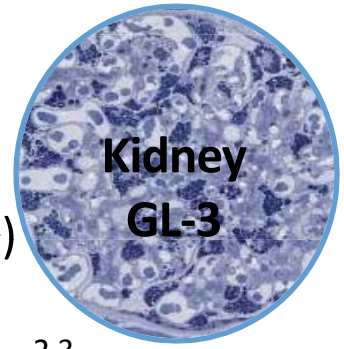
Migalastat HCl Monotherapy for Fabry Disease

Fabry Disease Overview

Fatal, Progressive, Multi-System Lysosomal Storage Disease



- Inherited GLA mutations
- X-linked
- Renal failure, cardiac failure, stroke
- 5-10K diagnosed WW (51% female/49% male[1])
- Recent neonatal screening results suggest prevalence of late onset form in 1:3000 or more[2,3]
- FY12 ERT sales of $873M WW
  - $195M US (Fabrazyme conditional approval)
  - $678M ex-US (Fabrazyme and Replagal full approval)
    - Presence of antibodies [against α-Gal A] may reflect worse treatment outcome[4]



[1]Fabry Registry 2011; [2] Spada *et al.,* **Am J Human Genetics** 2006; [3]Lin, *et al.,* **Circulation** 2009 [4]Rombach *et al.,* **PLoS ONE,** 2012

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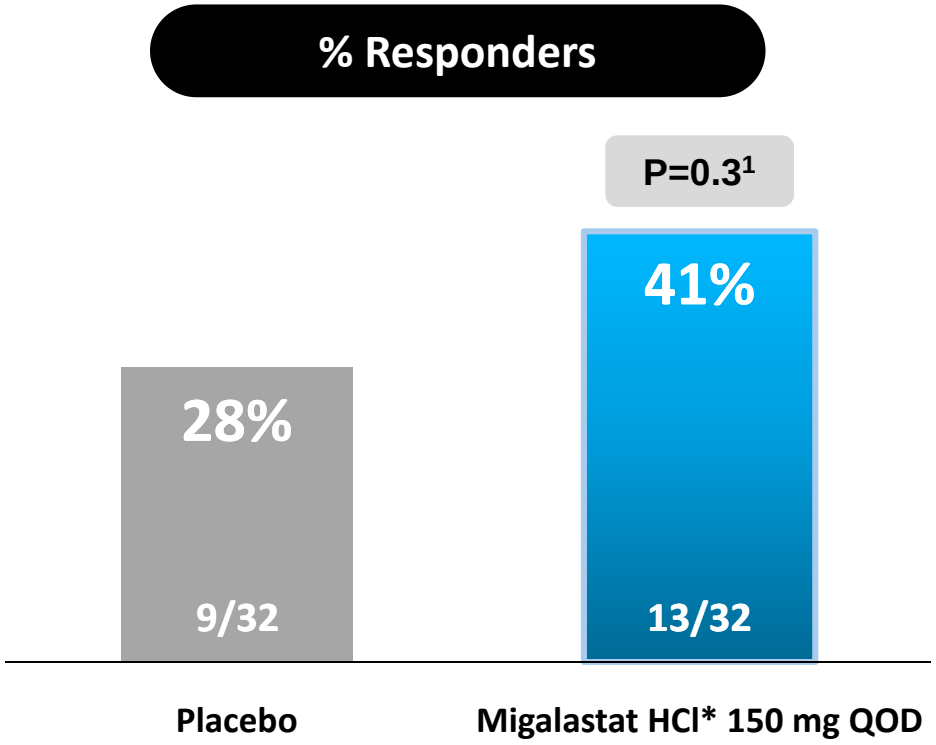
Phase 3 Study 011: Top-Line Stage 1 (6-Month) Results

Primary Endpoint - Responder Analysis (ITT):
≥ 50% Reduction from Baseline in Kidney Interstitial Capillary GL-3



* migalastat is not authorized for use and is an investigational product

1 Difference=12.5% (95% CI: -13.4, 37.3). Migalastat HCl minus placebo in % responders. P-value based on exact Cochran-Mantel-Haenszel test stratified by gender. Subjects with baseline biopsy but missing month 6 biopsy counted as a failure.

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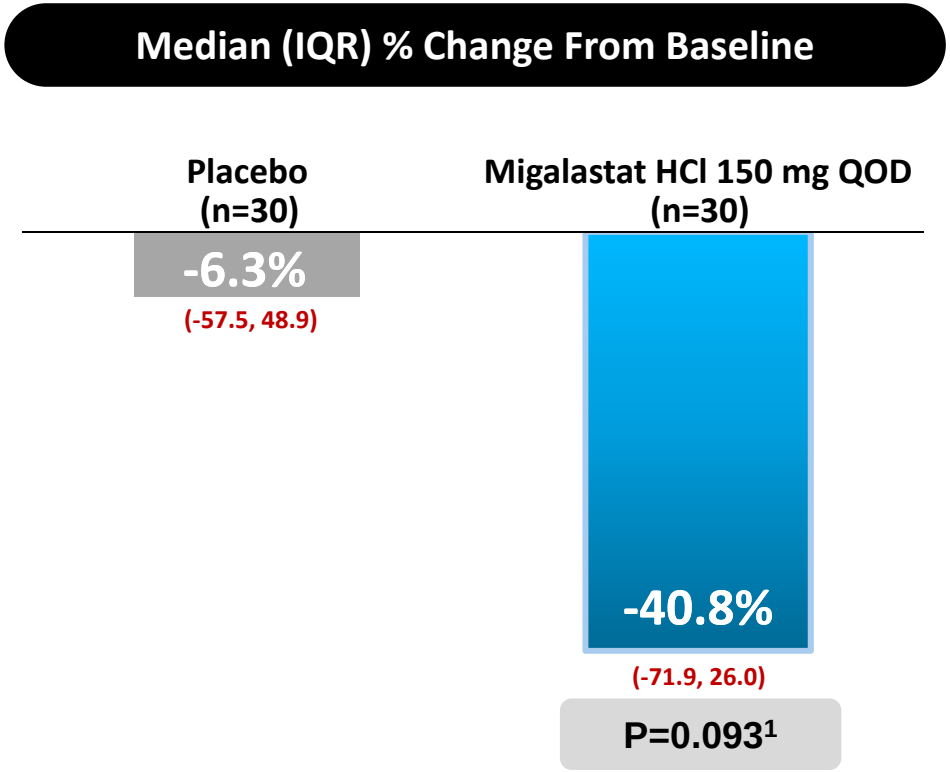
Phase 3 Study 011: Top-Line 6-Month Results

Secondary Analysis of Primary Endpoint (mITT*) Median Percent Change From Baseline in Kidney Interstitial Capillary GL-3



* mITT: Subjects who had a baseline and at least one on-therapy assessment. Median baseline average number of inclusions per capillary (0.23 placebo, 0.18 Migalastat HCl)

[1] p-value from ANCOVA based on ranked observations adjusting for baseline and sex

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Phase 3 Study 011: 6-Month Safety

Most Common Treatment Emergent Adverse Events (≥ 10% of Subjects)

Adverse event	Placebo (n=33)	Migalastat HCl (n=34)
Any Event	91%	91%
Headache	21%	35%
Fatigue	12%	12%
Nausea	12%	9%
Nasopharyngitis	15%	6%
Paresthesia	9%	12%

No serious adverse events deemed by investigators to be treatment-related
No withdrawals due to adverse events

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Phase 3 Study 011: Status and Milestones

1H 2013 → **2H 2013**

12-Month Treatment Extension & Long-Term Open-Label Extension*

Stage 2: (12-month) data 3Q13

- Pre-specified descriptive comparisons
- 12-month data from migalastat HCl group
- 6-month data from placebo crossover group

FDA Meeting Anticipated 2H13

- 6-month analysis is Stage 1 data
- FDA to consider entirety of Study 011 (Stage 1+2) data for NDA submission
- No single endpoint will be determinative

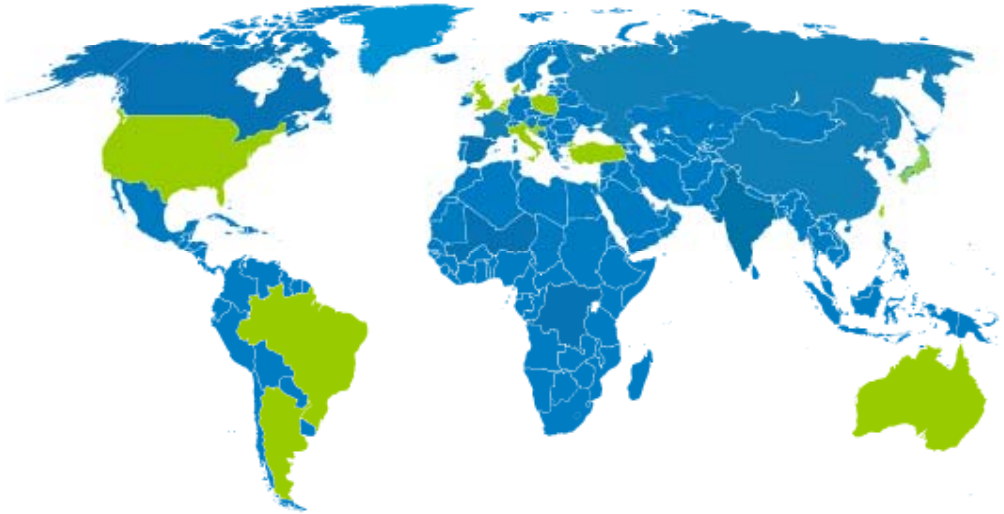
* Patients rolled over following 12-month treatment extension

Phase 3 Study 012

Ongoing 18-Month Open-Label Study Comparing Migalastat HCl (150 mg QOD) to ERT (Fabrazyme and Replagal) in Fabry Patients with Amenable Mutations*

THE ATTRACT STUDY

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



- Switch to migalastat HCl or remain on ERT
- 60 total patients (1.5:1 randomization)
- No kidney biopsies
- Clinical outcome is renal function (Iohexol GFR)
- Data anticipated 2H14

*GLA mutations identified as amenable to migalastat HCl in a cell-based assay

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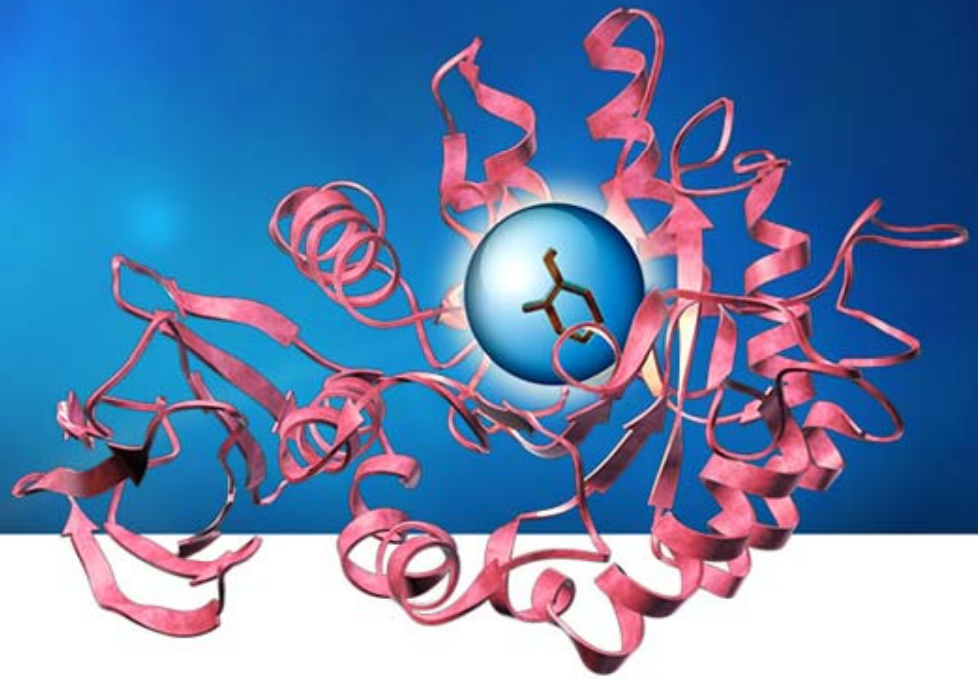


Chaperone-ERT Combination Platform

for Lysosomal Storage Diseases

CHART™

CHAPERONE-ADVANCED REPLACEMENT THERAPY

LSD Products Today

Potential Limitations

- Enzyme Instability in Blood & Infusion Bag
- Dosing Limitations & Duration of Infusion
- Poor Enzyme Uptake into Tissues
- Immunogenicity

CHART™: Chaperone-Advanced Replacement Therapy

CHART Offers Multiple Potential Ways to Improve ERT Outcomes for Patients

CHAPERONE-ADVANCED REPLACEMENT THERAPY

Proposed MOA:

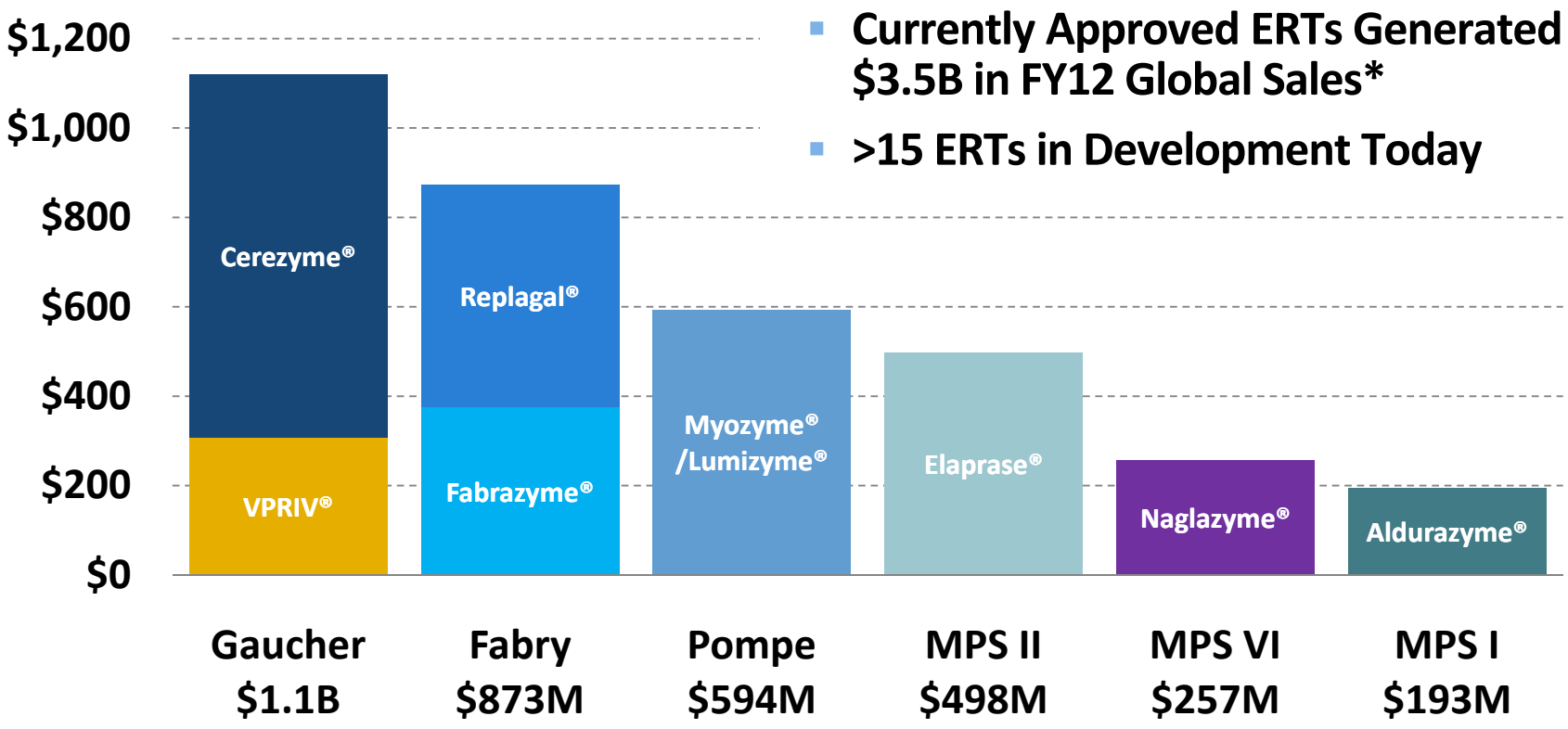
Bind to and Stabilize Enzyme, Keeping Properly Folded

- **Increase Active Enzyme in Circulation**
- **Increase Enzyme Uptake into Tissues**
- **Mitigate Immune Response**
- **Improve Dosing/Delivery**

Potential benefits under investigation in clinical and preclinical studies

$3.5B Current ERT Market for LSDs

FY12 Global Sales ($M)



- **Currently Approved ERTs Generated $3.5B in FY12 Global Sales***
- **>15 ERTs in Development Today**

*Source: 10-Ks from Shire, Sanofi, and BioMarin. Sales of Elelyso for Gaucher disease not shown.

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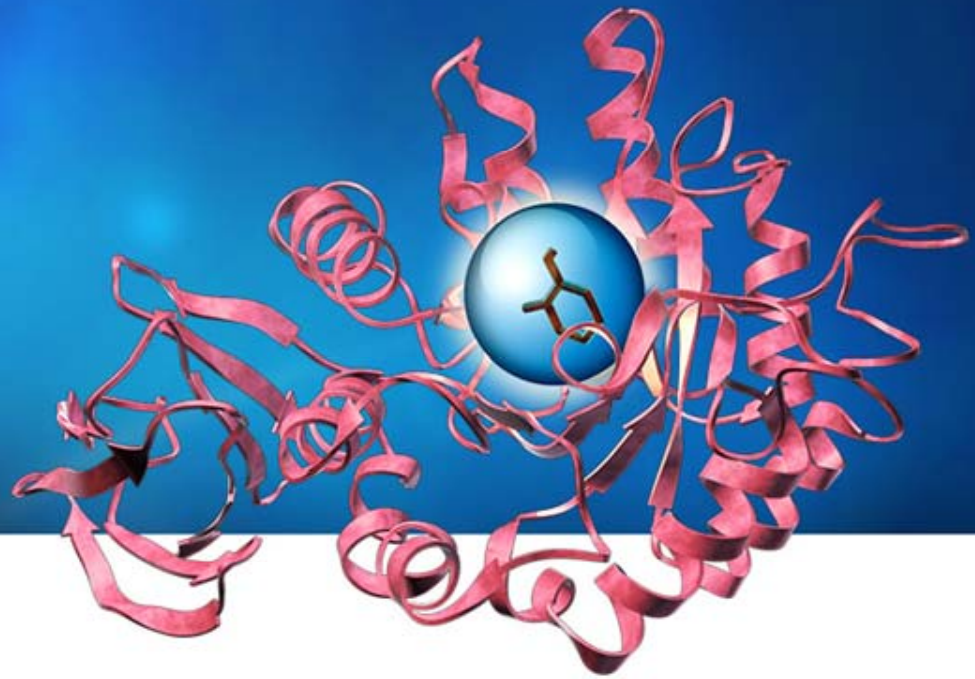
Amicus Therapeutics



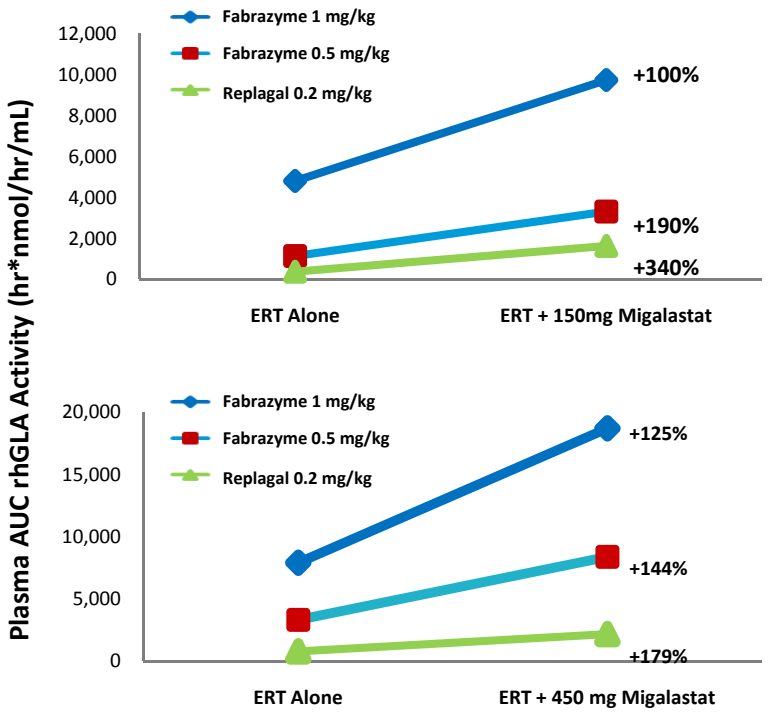
Chaperone-ERT Combinations

for Fabry Disease

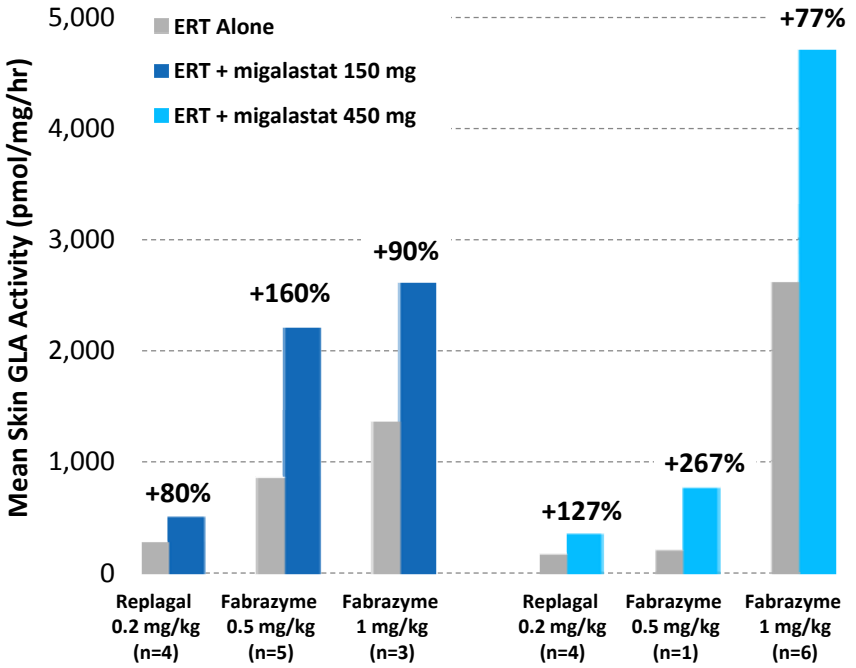
Fabry Co-Administration: Phase 2 Study 013

Oral Migalastat HCl[1] Co-Administered with Fabrazyme or Replagal Led to Consistent Increases in Active Plasma Enzyme Levels and Tissue Uptake[2]

Plasma rhGLA Activity (Area Under Curve)



Mean Skin GLA Activity (Day 2)



[1] Single oral dose 2 hours prior to ERT infusion ; [2] 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.

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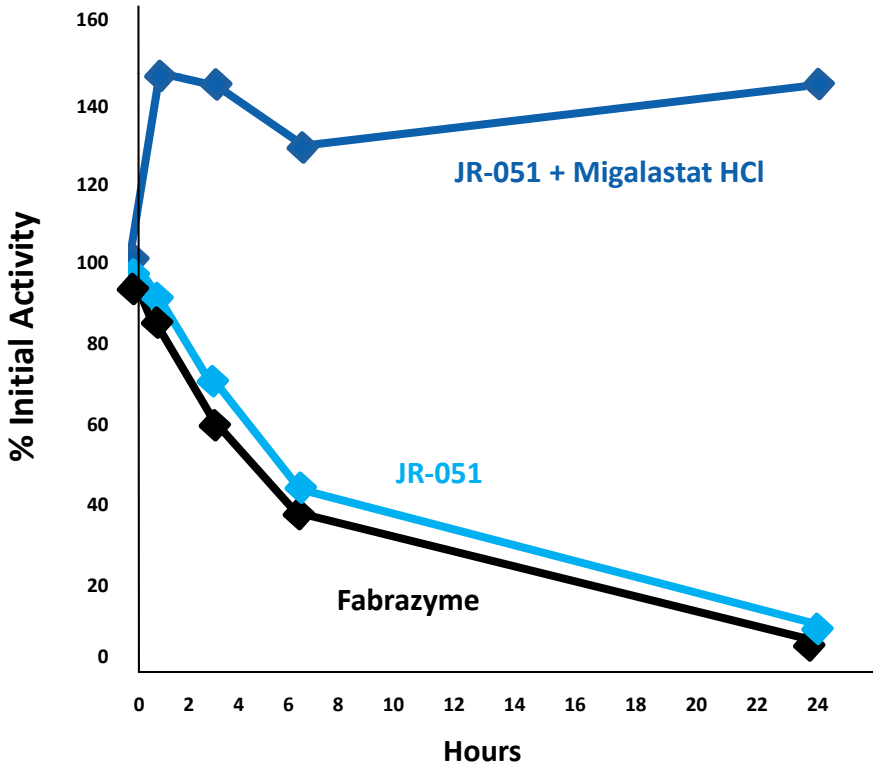
Fabry Co-Formulation: Proprietary ERT JR-051* + Migalastat HCl

(Preliminary Results)

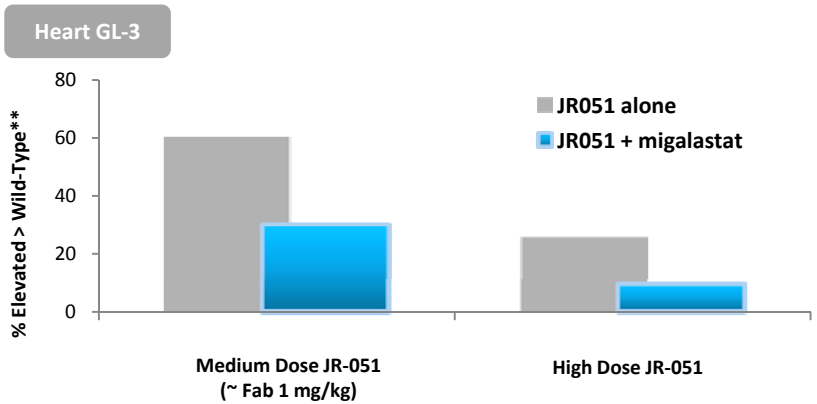
Stabilization of JR-051 *ex vivo*

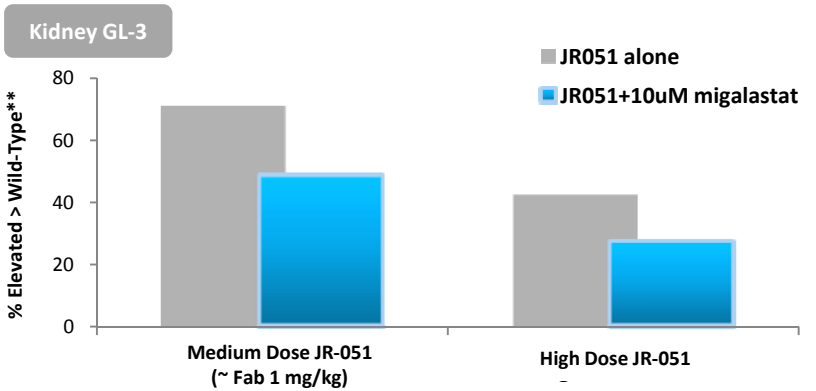
Migalastat HCl Prevents Loss of Enzyme Activity in Blood



JR-051 +/- Migalastat HCl in GLA Knock-Out Mice (Repeat-Dose IV Administration)

Co-Formulation Results in Significantly Greater GL-3 Reduction than Previously Observed





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

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Fabry Co-Formulation

Advancing JR-051 + Migalastat HCl Toward Clinic

GlaxoSmithKline Rare Diseases

JCR Pharmaceuticals Co., Ltd.

- Now manufacturing at 2,000 L scale
- IND-enabling studies underway
- IND submission planned 4Q13 for potential entry into clinic in early 2014

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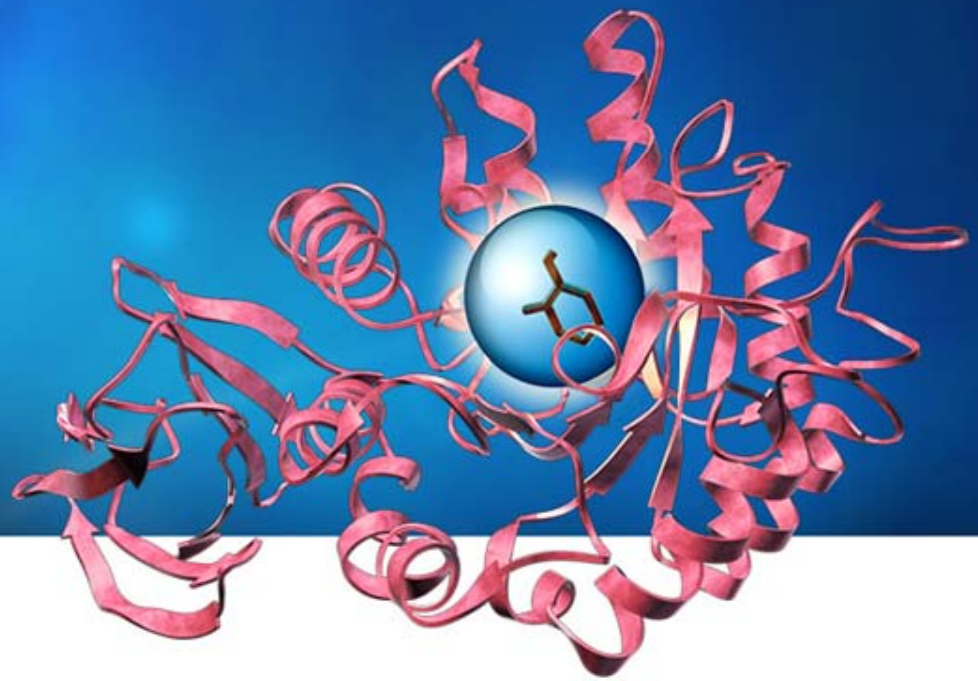


CHART™ Programs

for Pompe Disease

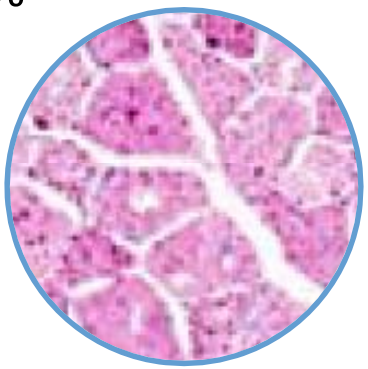
CHART™

CHAPERONE-ADVANCED REPLACEMENT THERAPY

Pompe Disease Overview

Severe, Fatal Neuromuscular Disease

- Inherited deficiency in lysosomal enzyme GAA
- Glycogen accumulation
- First and only approved ERTs (recombinant GAA): Myozyme/Lumizyme ($594M in FY12 sales)
  - Standard infusion every-other-week
  - Infusion-associated reactions in ~50% of late-onset patients[1]
  - Attenuated therapeutic response in infantile Pompe patients with high sustained antibody titer[2]
  - High antibody titer shown to affect treatment in adults[3]



Elevated Glycogen in Muscle

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

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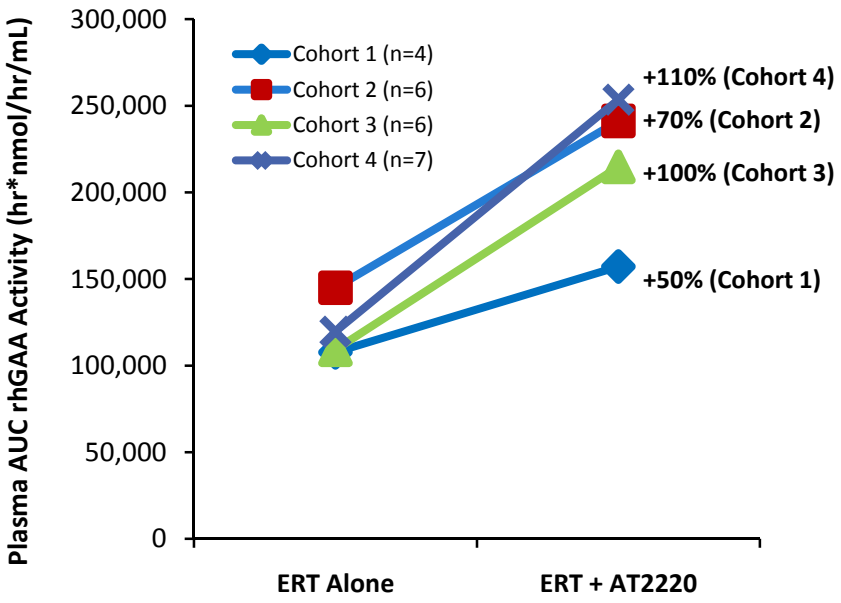
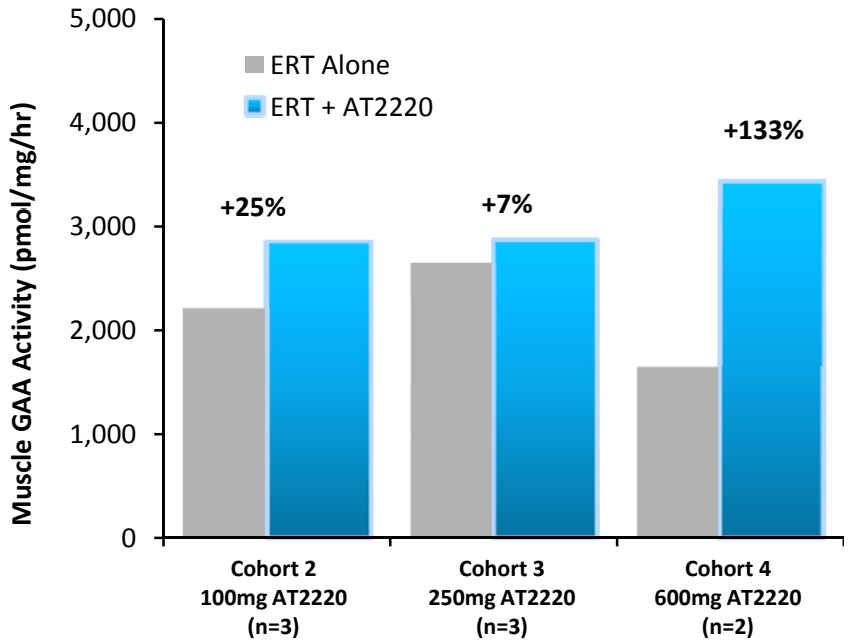
Pompe Co-Administration: Phase 2 Study 010

Oral AT2220 Co-Administered with Myozyme/Lumizyme Also Leads to Consistent Increases in Plasma Enzyme Activity and Tissue Uptake[1]

Plasma AUC rhGAA Activity



Muscle GAA Activity (Day 3)*



1Kishnani, 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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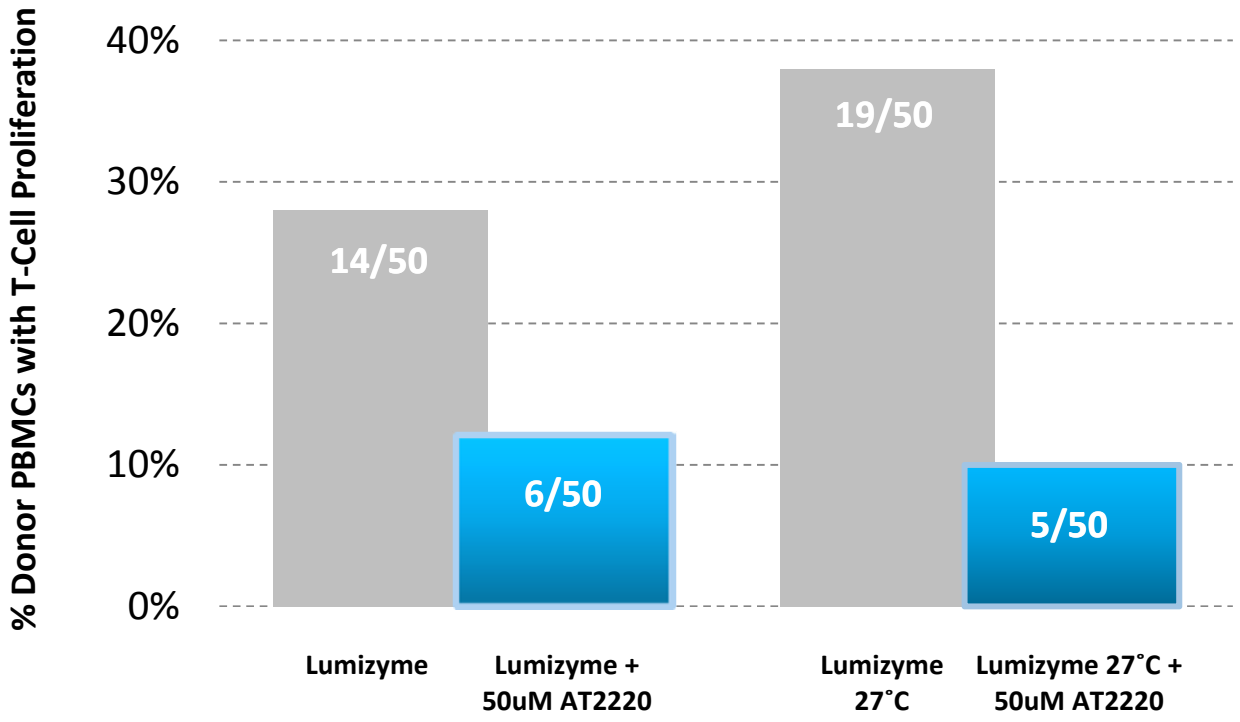
Pompe Co-Administration: Potential to Mitigate ERT Immunogenicity

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

T-Cell Proliferation in PBMCs from 50 Healthy Volunteers



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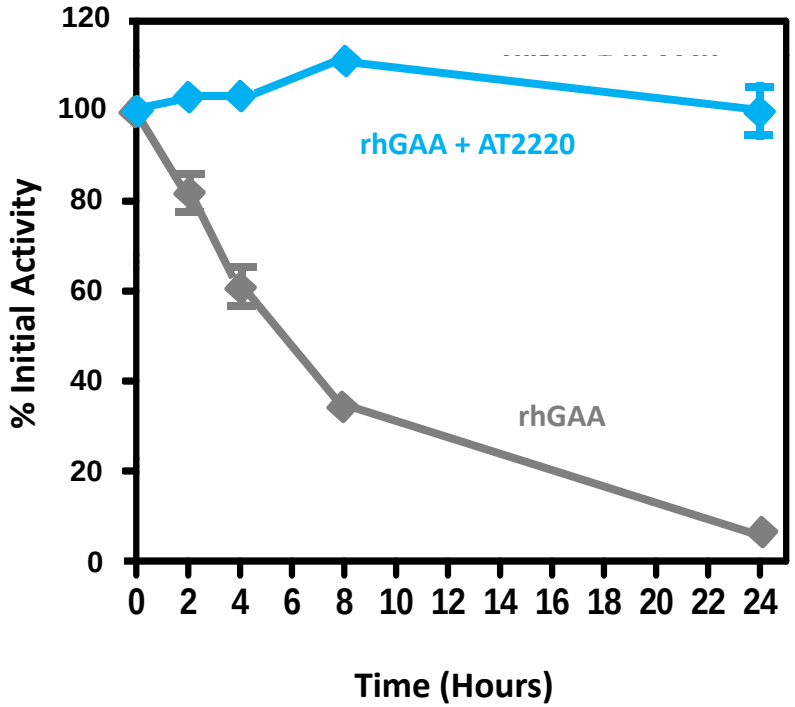
Pompe Co-Formulation: AT2220 + Myozyme/Lumizyme (rhGAA)[1]

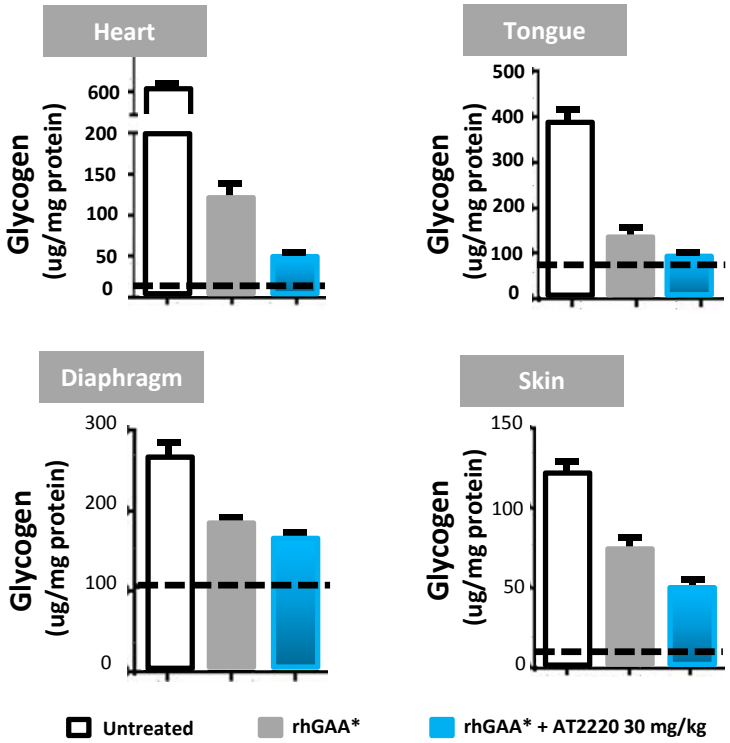
AT2220 Prevents Loss of Enzyme Activity in Blood and Co-Formulation Results in Significantly Greater Glycogen Reduction

Stabilization of rhGAA *ex vivo*



rhGAA +/- AT2220 in GAA Knock-Out Mice

Repeat-Dose IV Administration



[1]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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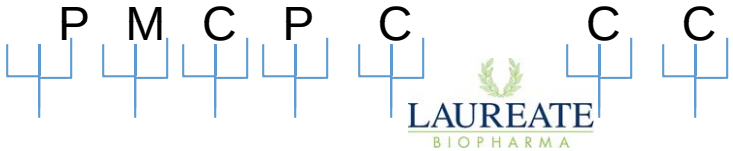
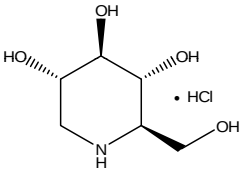
Next-Generation Pompe ERT: AT2220 + Proprietary rhGAA Enzyme

Leveraging CHART Platform with Internal Biologics Capabilities to Develop Next-Generation ERT

Proprietary rhGAA Enzyme



AT2220 Small Molecule Stabilizer



Potential Improvements

- **Optimized glycosylation**
- **De-immunization**
- **Increased exposure and tissue uptake**
- **Reduced immunogenicity**
- **SubQ delivery**

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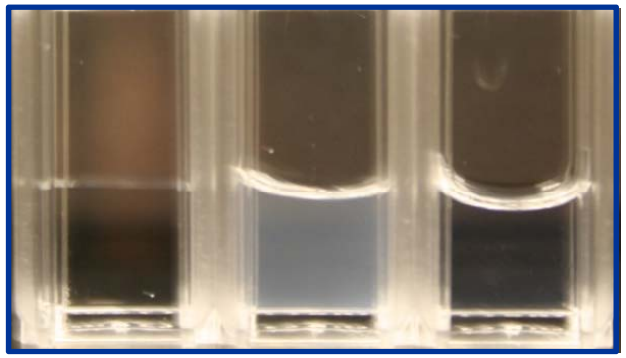
Next-Generation Pompe ERT

SubQ Delivery Potential

Increased ERT Stability and Prevention of Aggregation



Myozyme	-	+	+
AT2220	-	-	+

Aggregation assessed after 4 weeks at 37˚C

Increased Circulating Levels of Active rhGAA in Rats

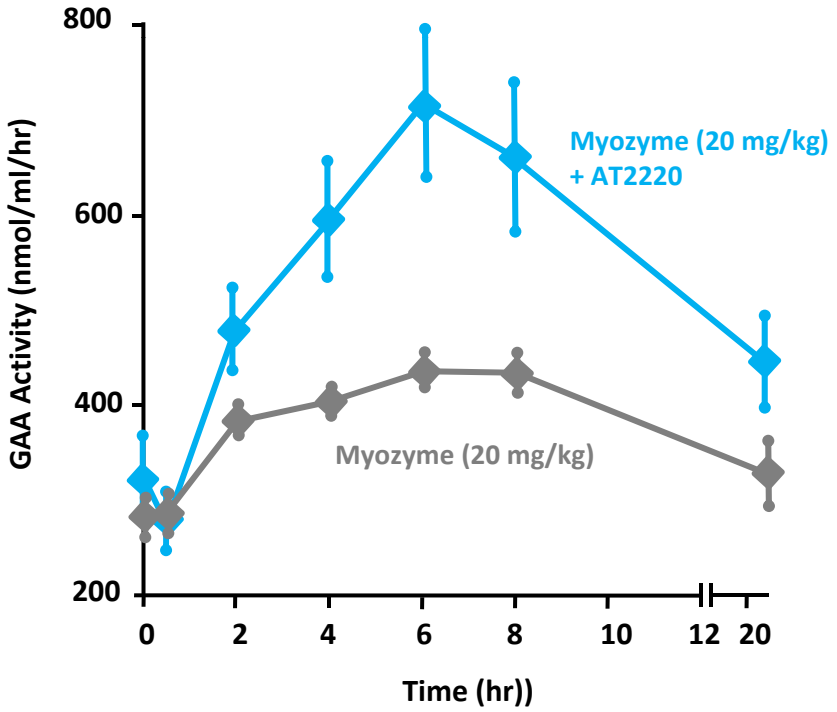


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CHART Pathways for Pompe Disease

Co-Administration (AT2220-IV + Marketed ERTs)	**Repeat-dose clinical study expected to begin 3Q13**
Next-Generation ERT (AT2220 + Proprietary rhGAA Enzyme)	**Preclinical studies underway**

Amicus Therapeutics

2013 Anticipated Clinical Milestones

Building Shareholder Value

Migalastat HCl Monotherapy for Fabry Disease	Study 011 6-Month data at LDN WORLD	Feb 2013
	Top-line Study 011 12-month data	3Q 2013
	FDA meeting to discuss U.S. approval pathway	2H 2013
Pompe Co-Administration	Phase 2 Study 010 data at LDN WORLD	Feb 2013
	Initiation of repeat-dose clinical study	3Q 2013
Fabry Co-Formulation (Migalastat HCl+JR-051)	IND Submission	4Q 2013
	Entry into Clinic	1Q 2014

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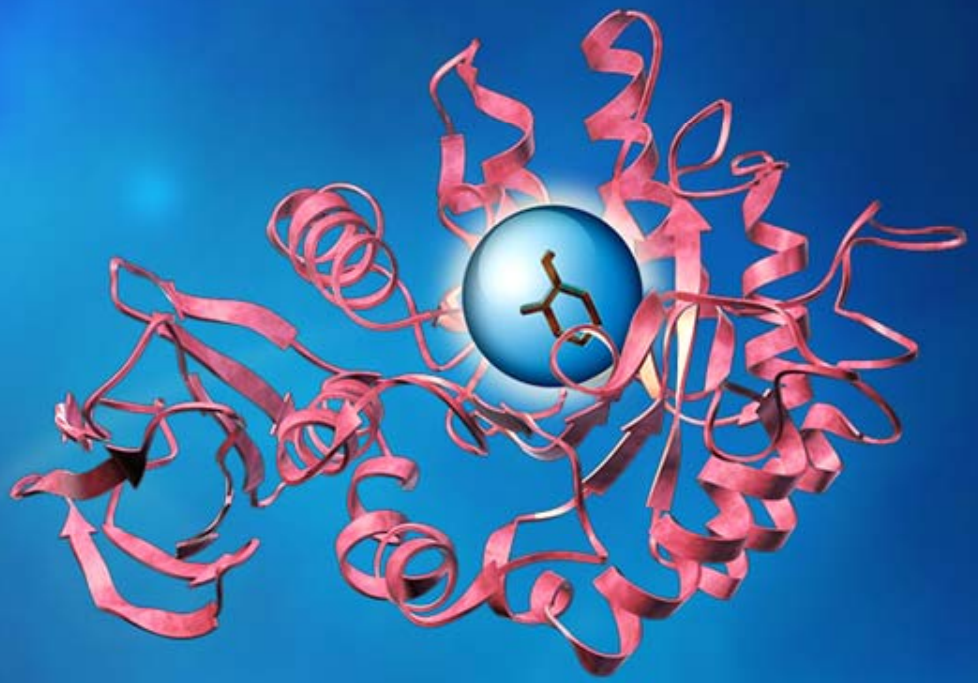

UBS Global Healthcare Conference

May 21, 2013

at the forefront of therapies for rare and orphan diseases