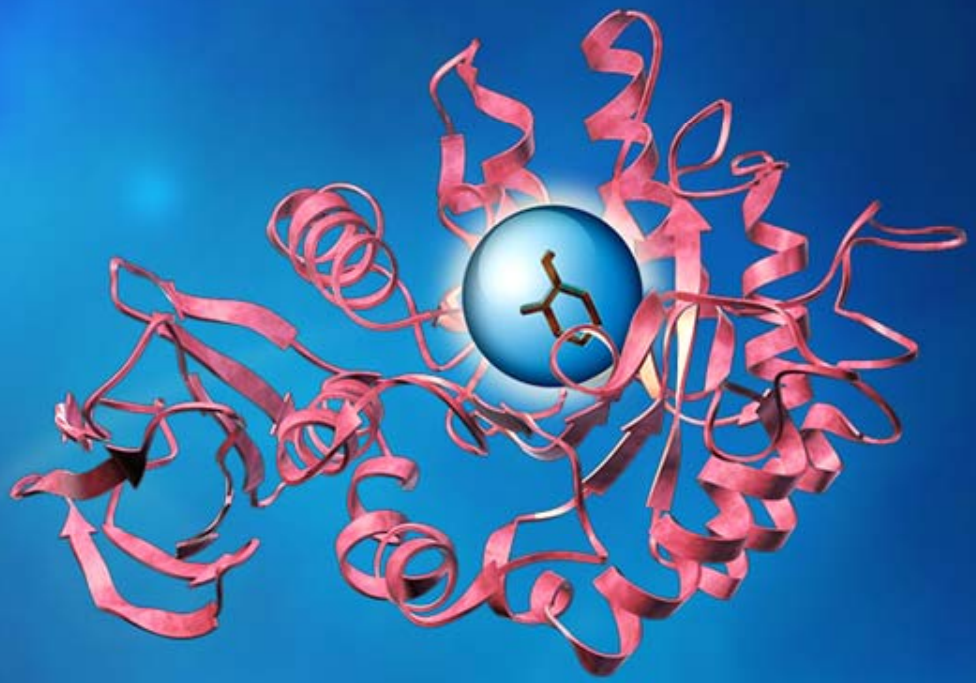
Amicus Therapeutics



Canaccord Genuity 33rd Annual Growth Conference

John F. Crowley,
Chairman and Chief Executive Officer

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, 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.

Amicus Therapeutics

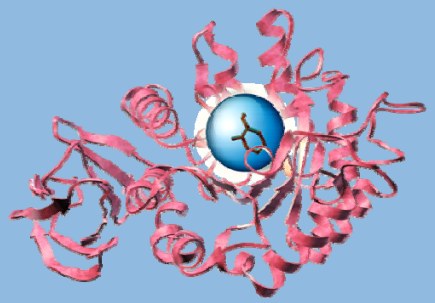
Company Mission

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

JCR Pharmaceuticals Co., Ltd.

FINANCIAL STRENGTH

Core Technology and Focus: Potential to Transform LSD Treatments

Monotherapy

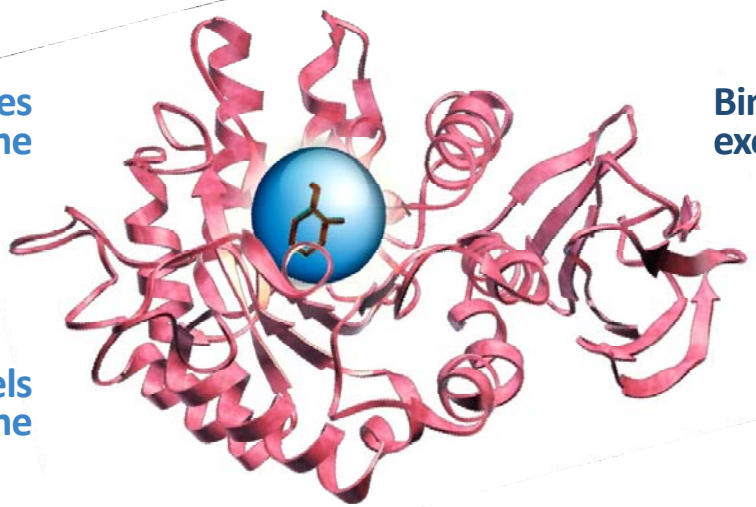
Binds to and stabilizes endogenous target enzyme

Increases lysosomal levels of active enzyme

Combination

Binds to and stabilizes exogenous enzyme (ERT)

Increases tissue uptake of active enzyme



LOWERED SUBSTRATE

Amicus Therapeutics

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						
Mucopolysaccharidosis Type I						
Next-Generation ERT						

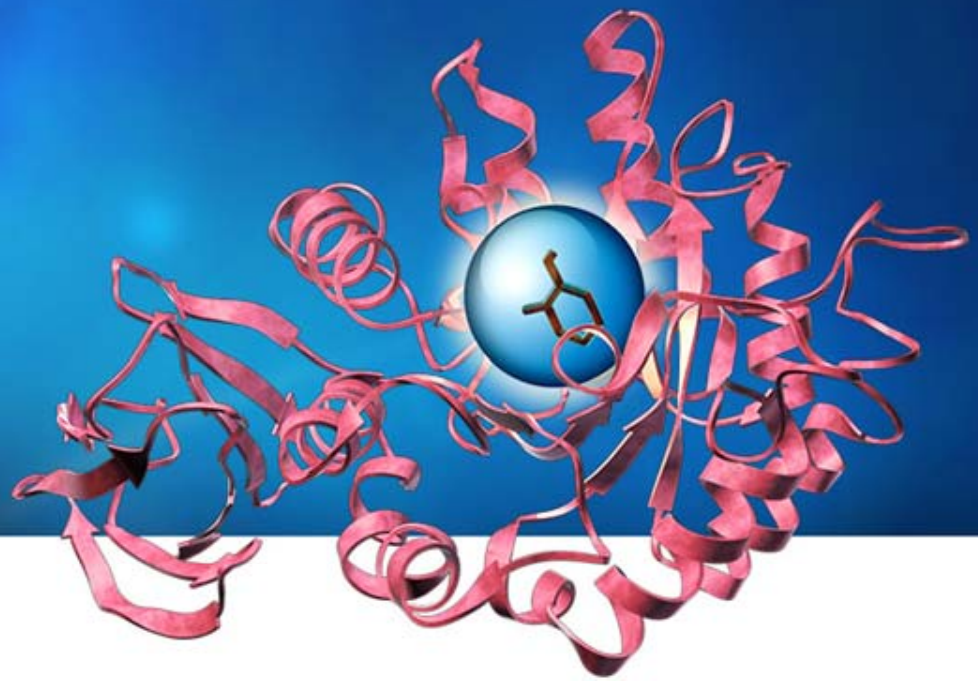
MONOTHERAPY

CO-ADMINISTRATION

NEXT-GENERATION ERTs (co-formulation)

Amicus Therapeutics

Amicus Therapeutics



Chaperone-ERT Combination Platform

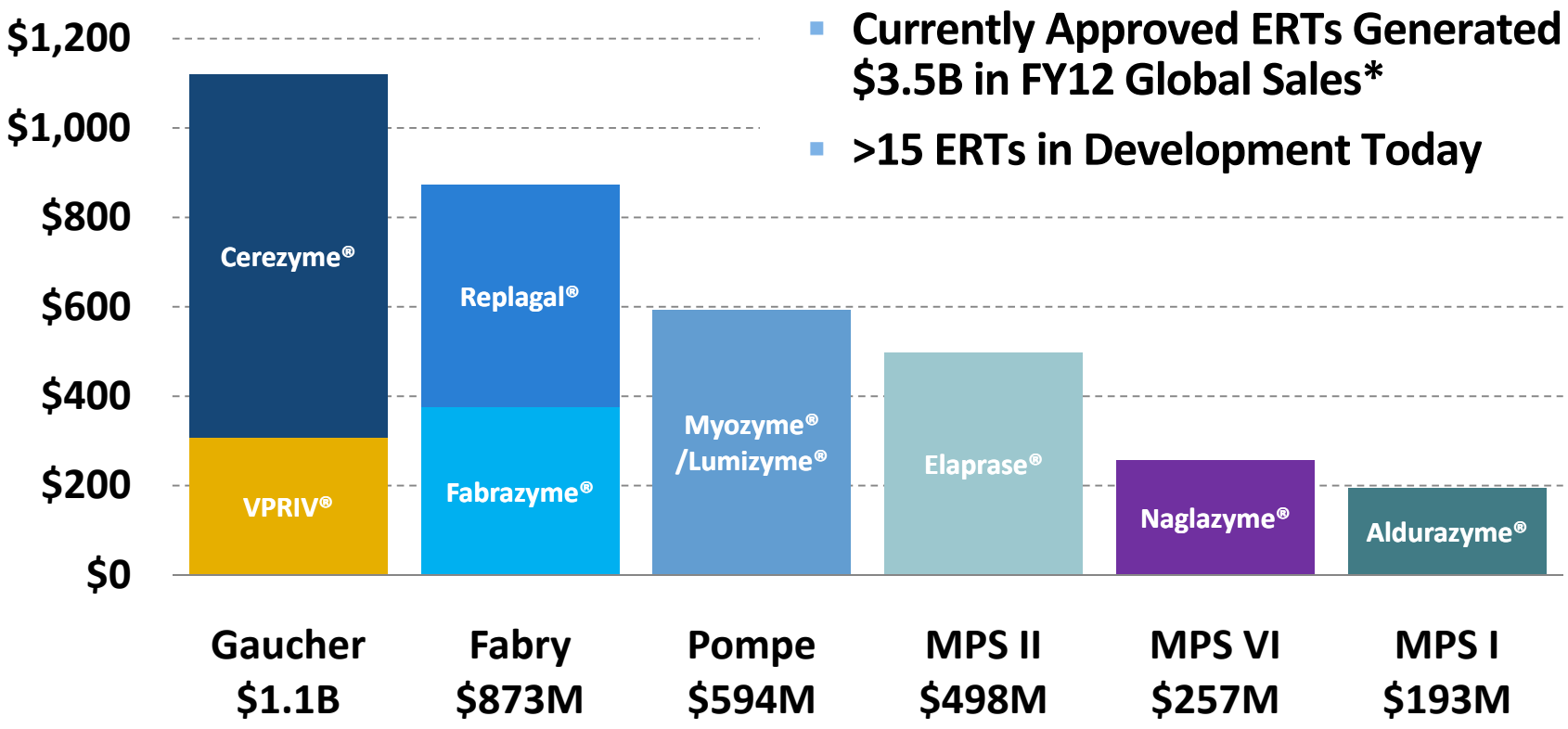
for Lysosomal Storage Diseases

CHART™

CHAPERONE-ADVANCED REPLACEMENT THERAPY

$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.
Fabrazyme, Cerezyme, Myzoyme 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

Amicus Therapeutics

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

CHAPERONE-ADVANCED REPLACEMENT THERAPY

CO-ADMINISTRATION
(Chaperones + Marketed ERTs)

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

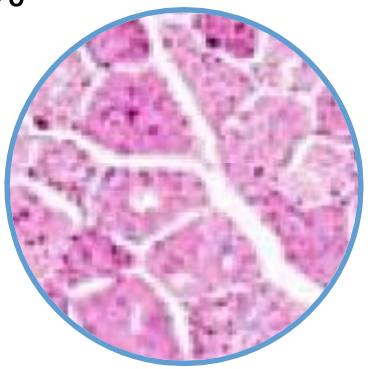
NEXT-GENERATION ERTs WITH IMPROVED DELIVERY REGIMEN

Amicus Therapeutics

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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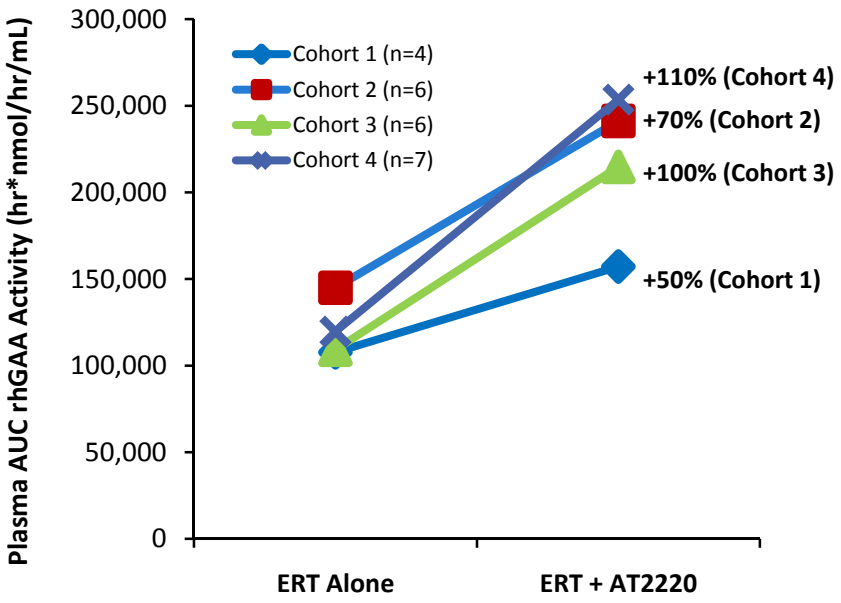
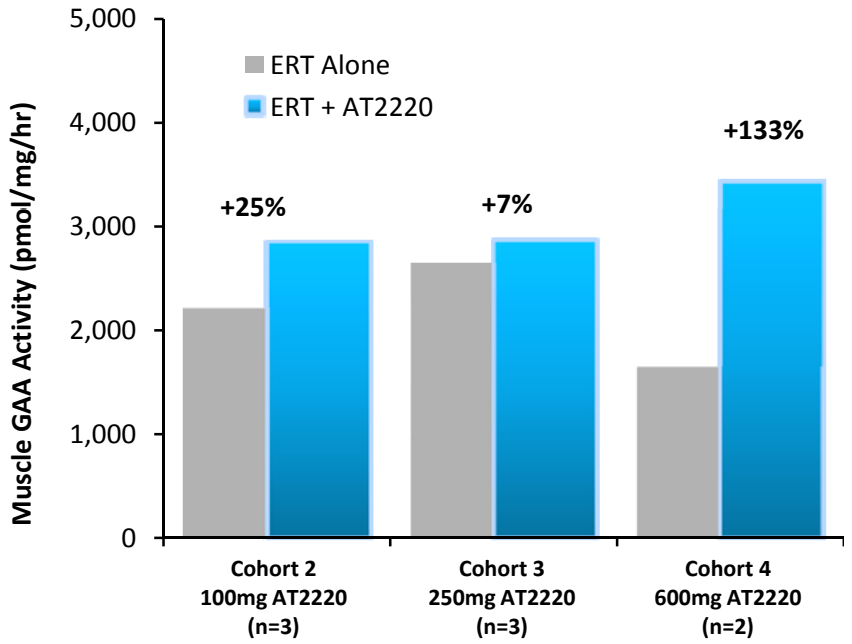
Pompe Co-Administration: Phase 2 Study 010

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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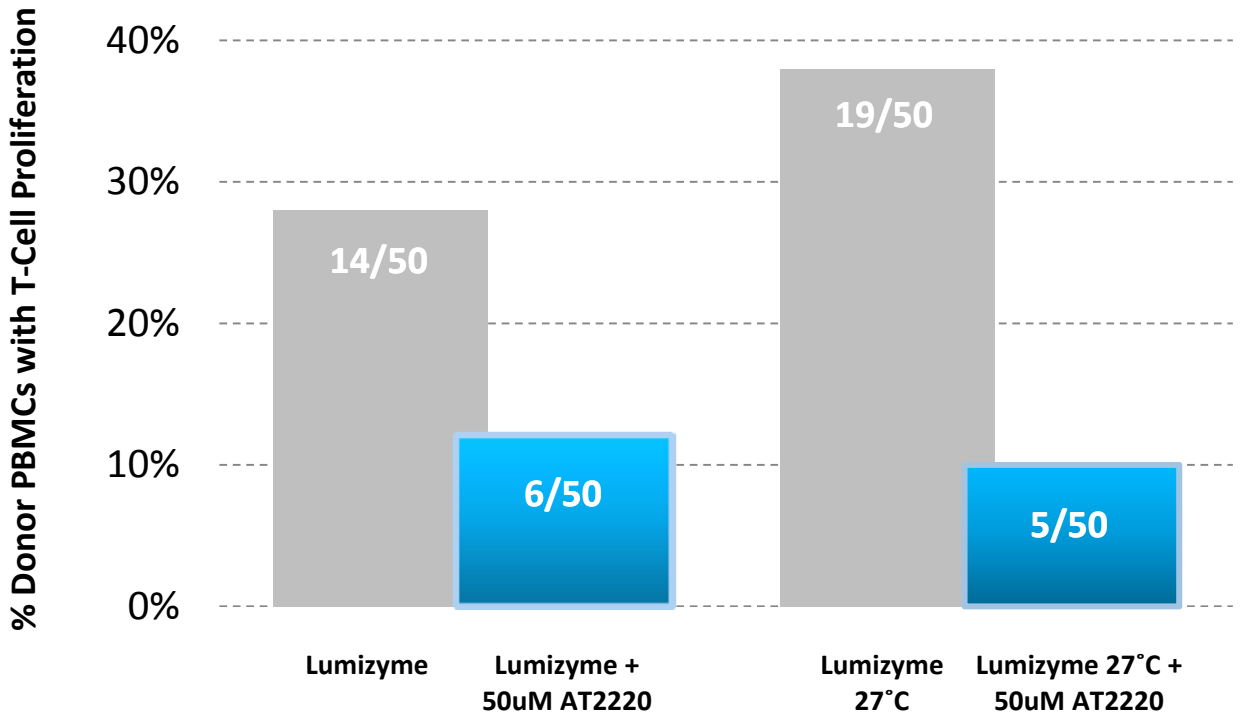
Pompe Co-Administration: Potential to Mitigate ERT Immunogenicity

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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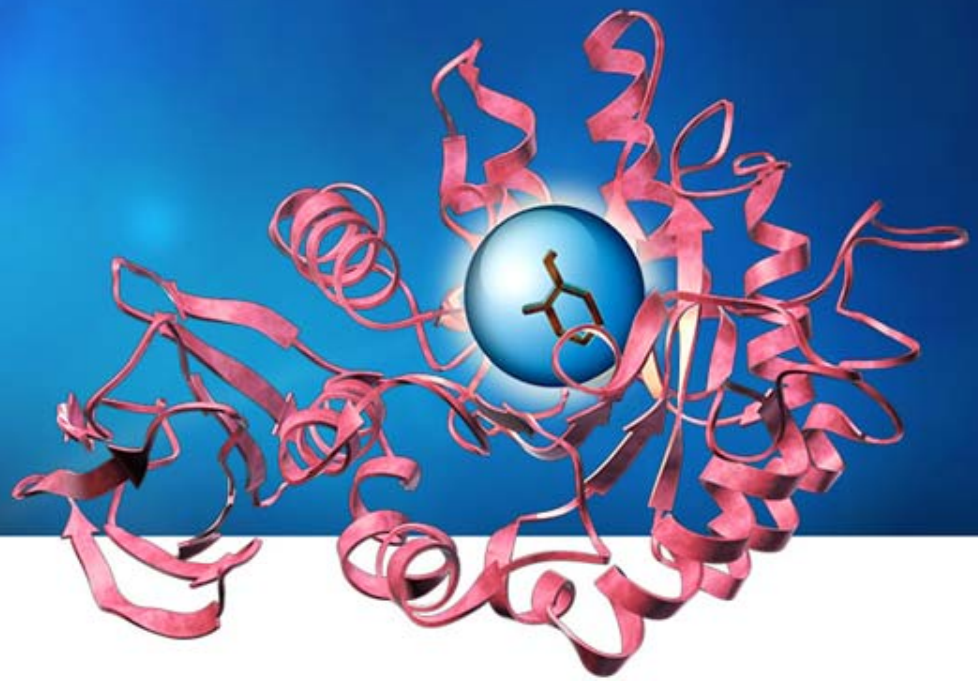
Amicus Therapeutics

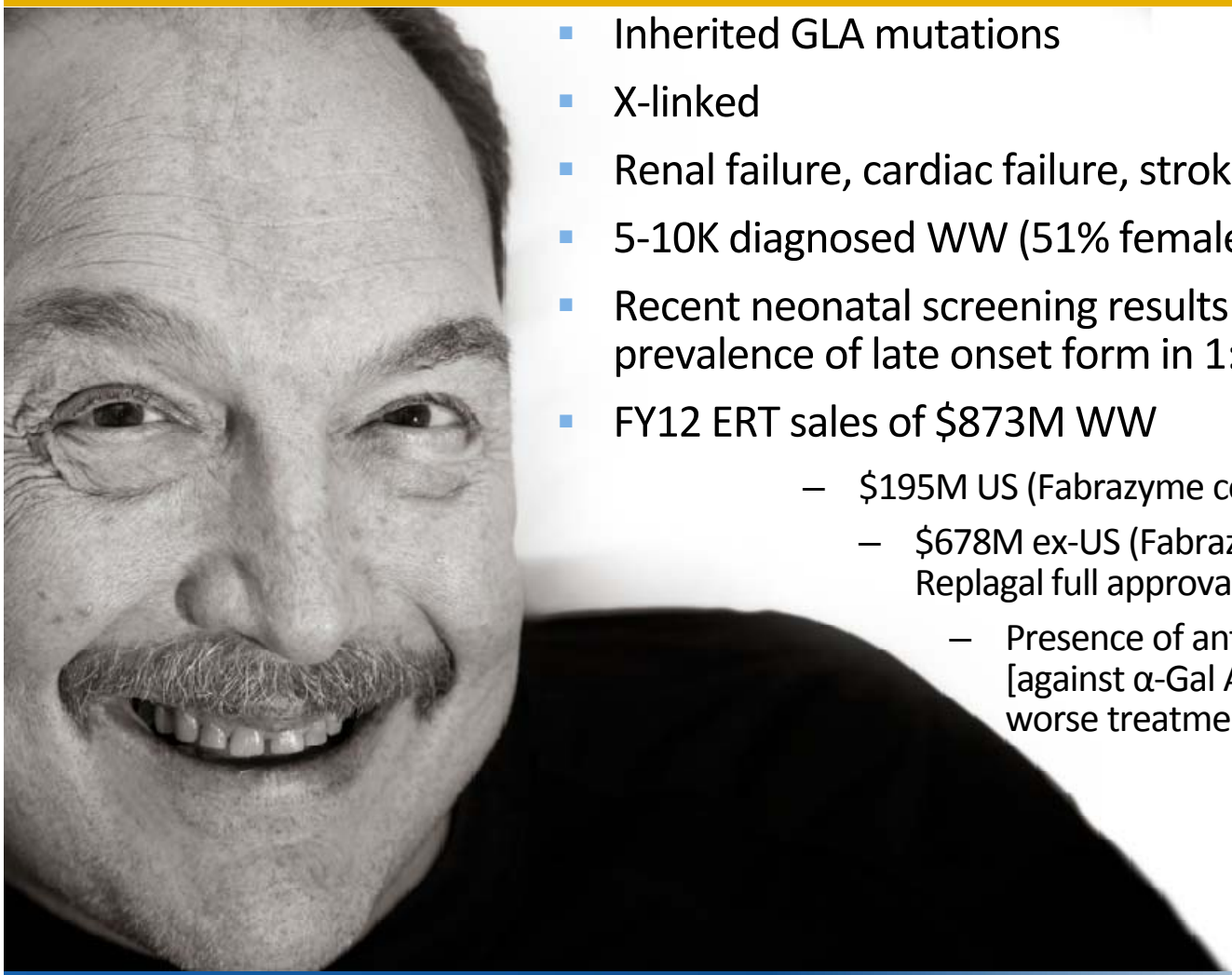


Chaperone-ERT Combinations

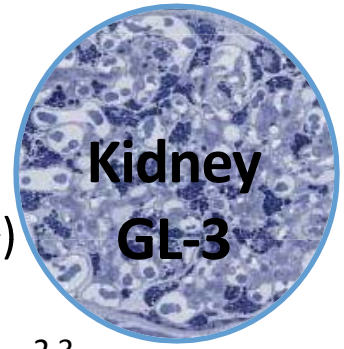
for Fabry Disease

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]



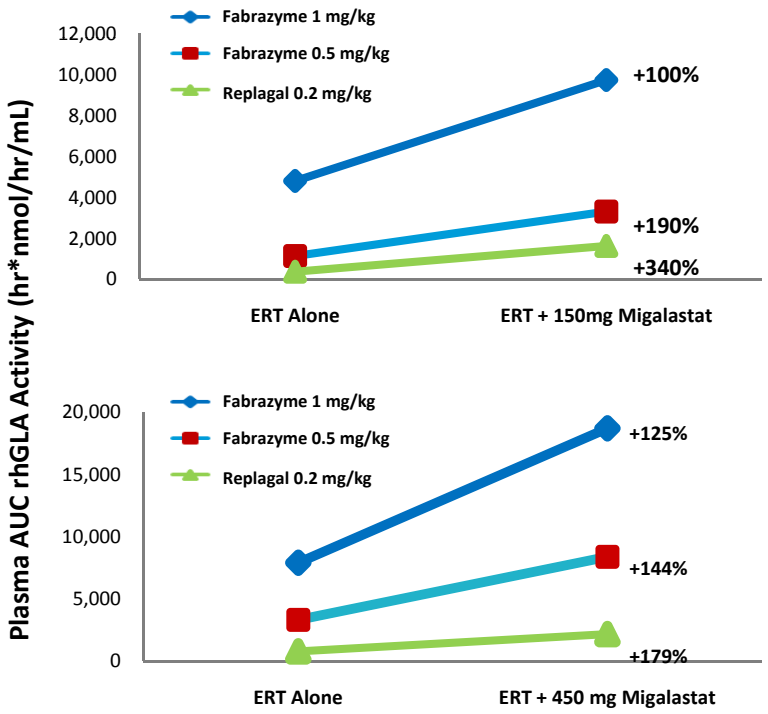
[1]Fabry Registry 2011; [2] Spada *et al.,* **Am J Human Genetics** 2006; [3]Lin, *et al.,* **Circulation** 2009 [4]Rombach *et al.,* **PLoS ONE,** 2012

Amicus Therapeutics

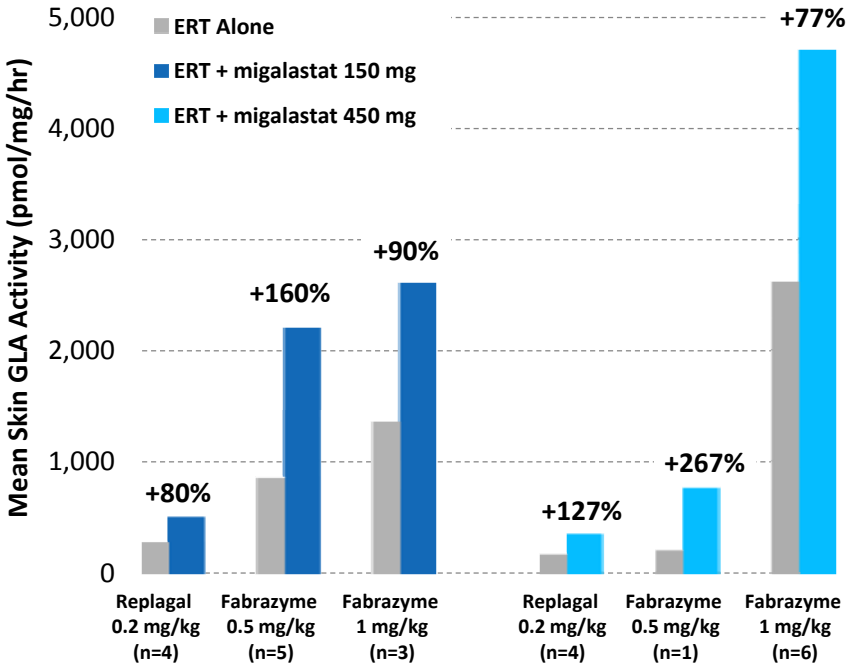
Fabry Co-Administration: Phase 2 Study 013

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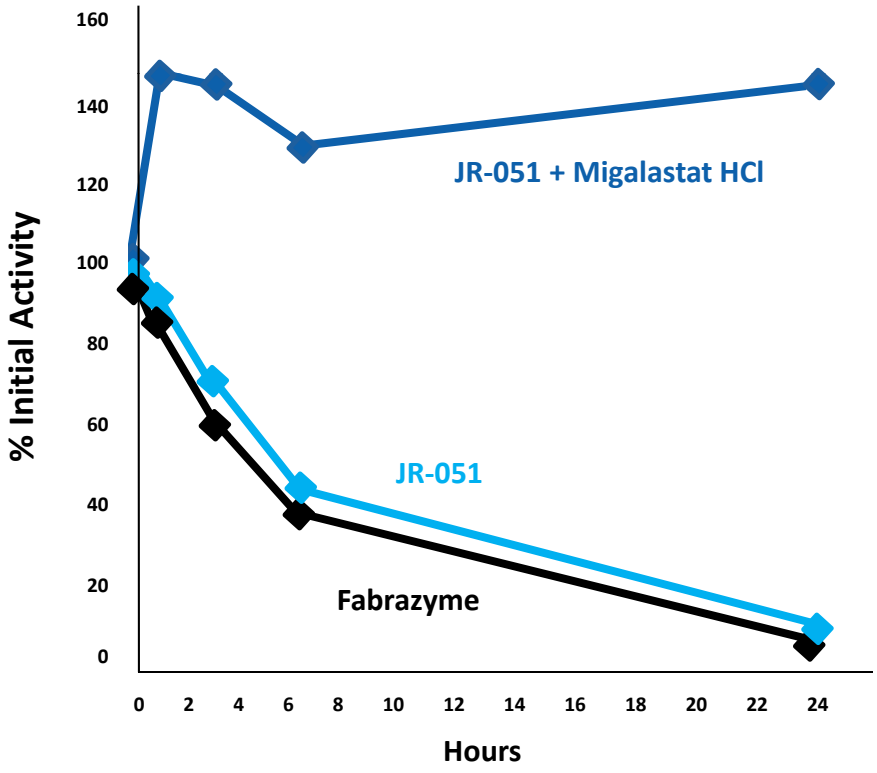
Fabry Co-Formulation: Proprietary ERT JR-051* + Migalastat HCl

(Preliminary Results)

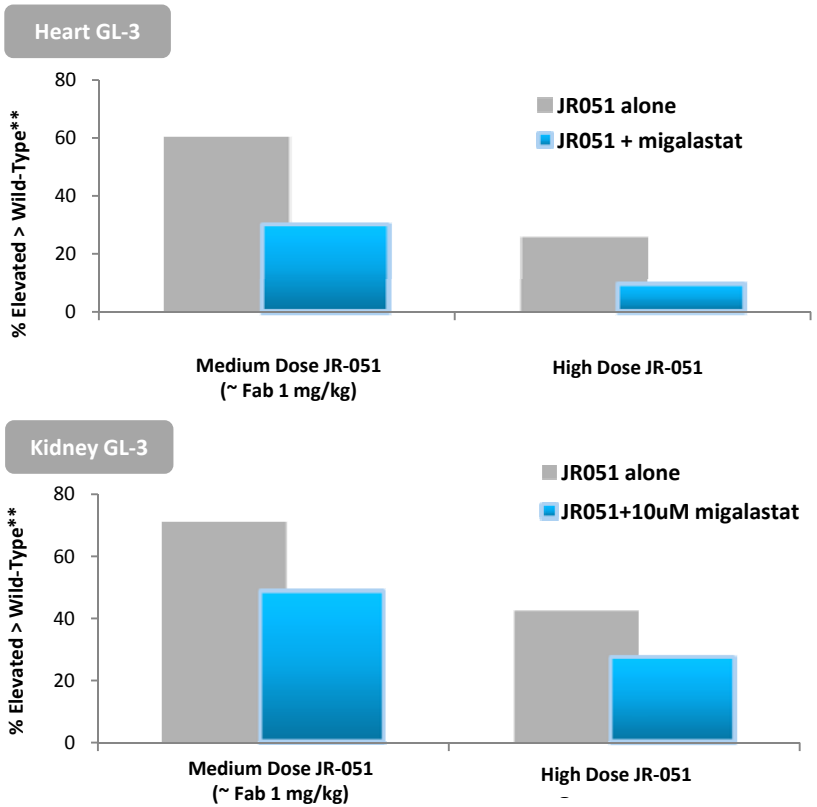
Stabilization of JR-051 *ex vivo*

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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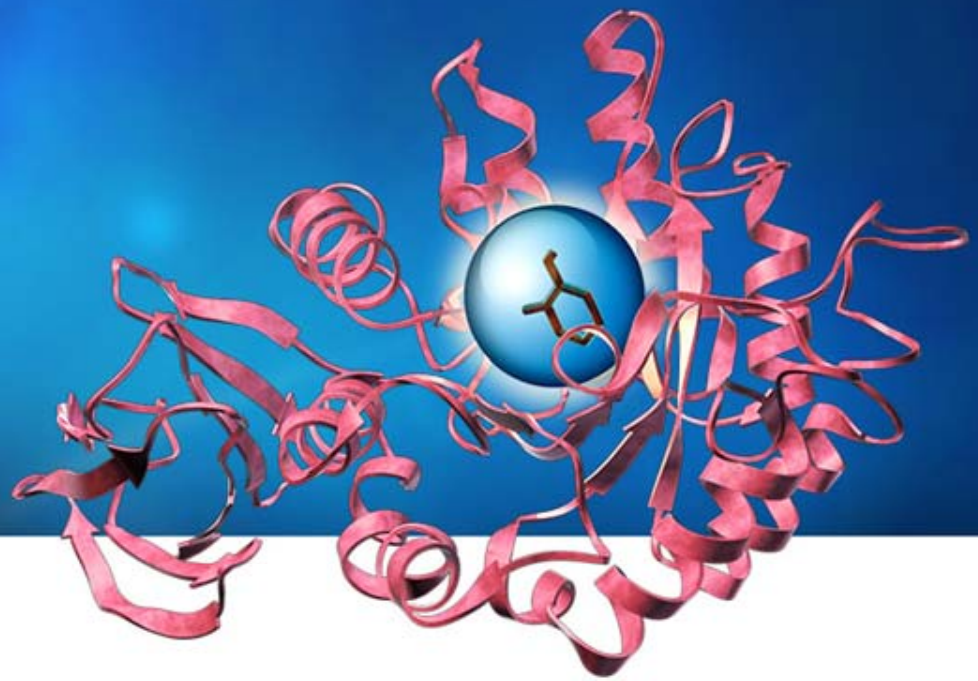
Amicus Therapeutics



Next-Generation Therapies

for Pompe Disease and Mucopolysaccharidosis Type I

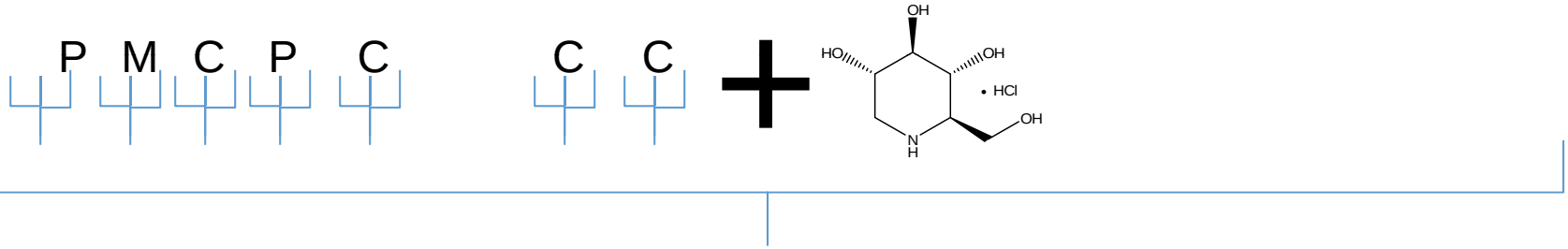
CHART™

CHAPERONE-ADVANCED REPLACEMENT THERAPY

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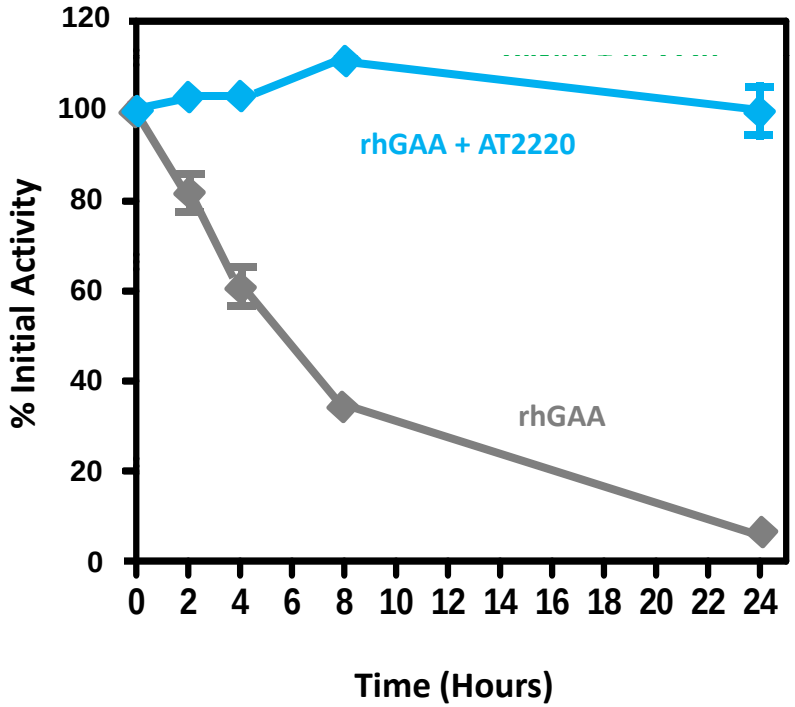
Pompe Co-Formulation: AT2220 + Myozyme/Lumizyme (rhGAA)[1]

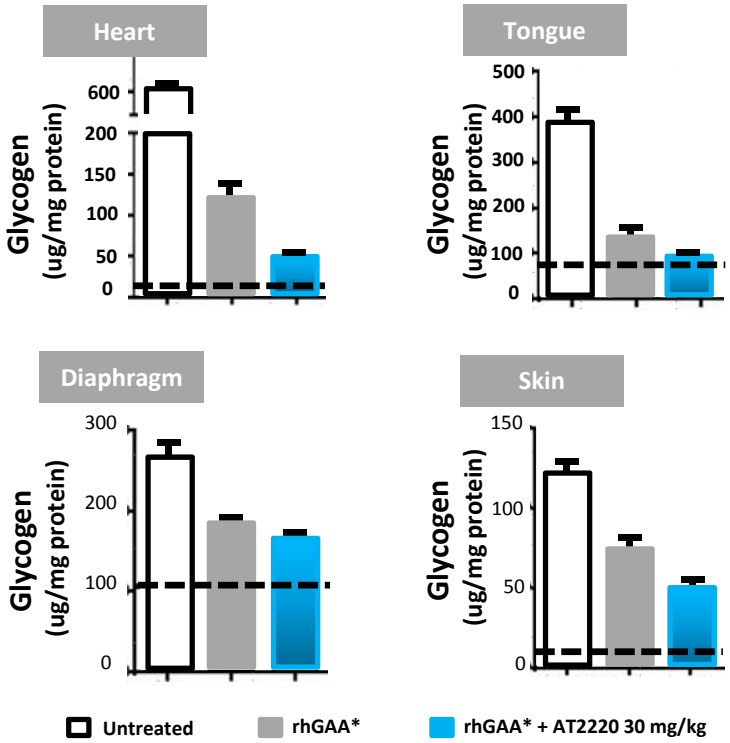
AT2220 Prevents Loss of Enzyme Activity in Blood and Co-Formulation Results in Significantly Greater Glycogen Reduction in Preclinical Studies

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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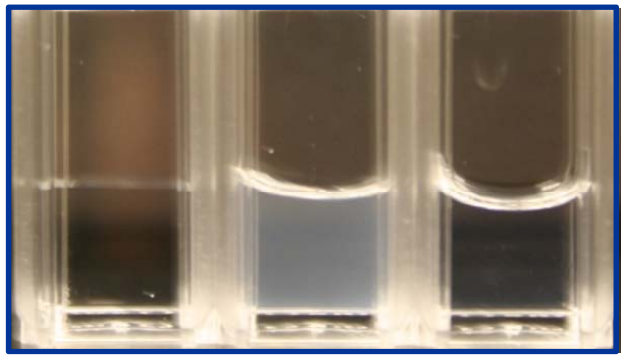
Next-Generation Pompe ERT

SubQ Delivery Potential

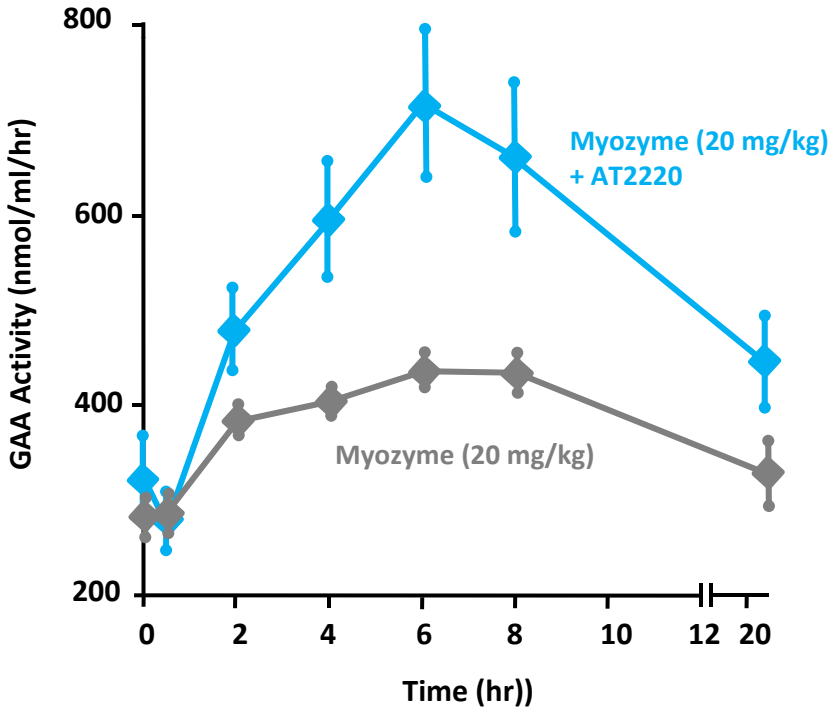
Increased ERT Stability and Prevention of Aggregation



Myozyme	-	+	+
AT2220	-	-	+

Aggregation assessed after 4 weeks at 37˚C

Increased Circulating Levels of Active rhGAA in Rats



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Mucopolysaccharidosis Type I (MPS I)

Severe, Fatal Multi-System Disease

- Inherited deficiency in lysosomal enzyme alpha-L-iduronidase (IDUA)
- Accumulation of complex carbohydrates (glycosaminoglycans, GAGs) dermatan sulfate and heparan sulfate
- Affects physical abilities, organ and system functioning, mental and skeletal development
- Estimated 3,000 diagnosed in US, EU and Japan[1]
- First and only approved ERT: rhIDUA, Aldurazyme® ($193M in FY12 sales)
  - rhIDUA does not access CNS
  - Little effect on bone growth and heart valve disease
  - Significant number of patients develop antibodies that may negatively impact efficacy[2]

[1]Genzyme 10-K. [2]Bigger and Jones, WORLD 2012 presentation, March 2012; Dickson, *et al.*, Molecular Gen and Metab, 2012; Clarke, *et al.*, Pediatrics, 2009. Ponder, *et al.*, Journal of Clin Investigation, 2008; Wraith, *et al.*, Pediatrics, 2007.

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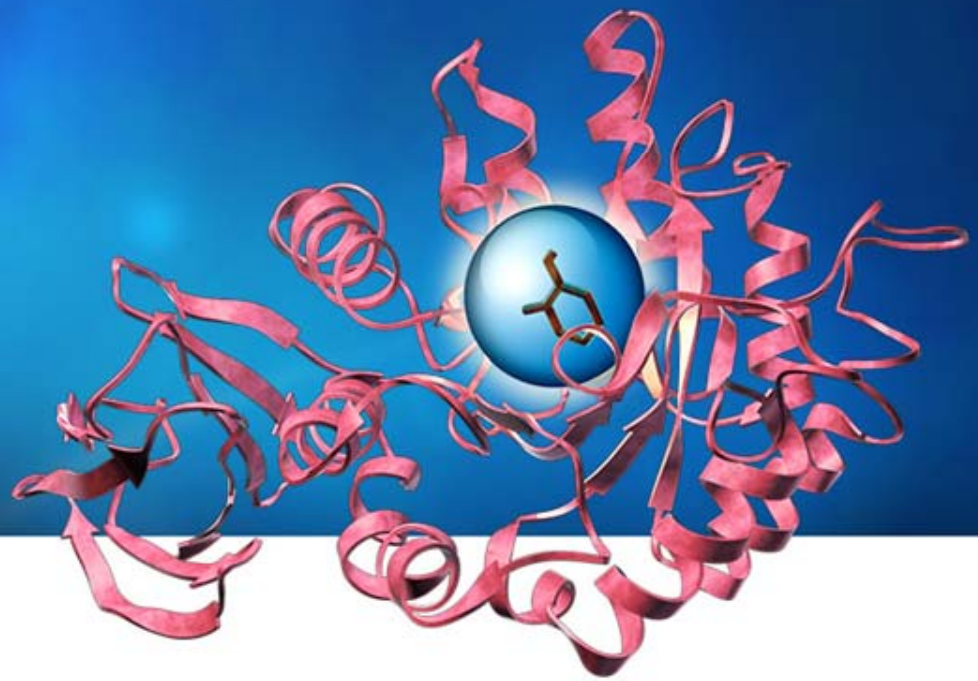
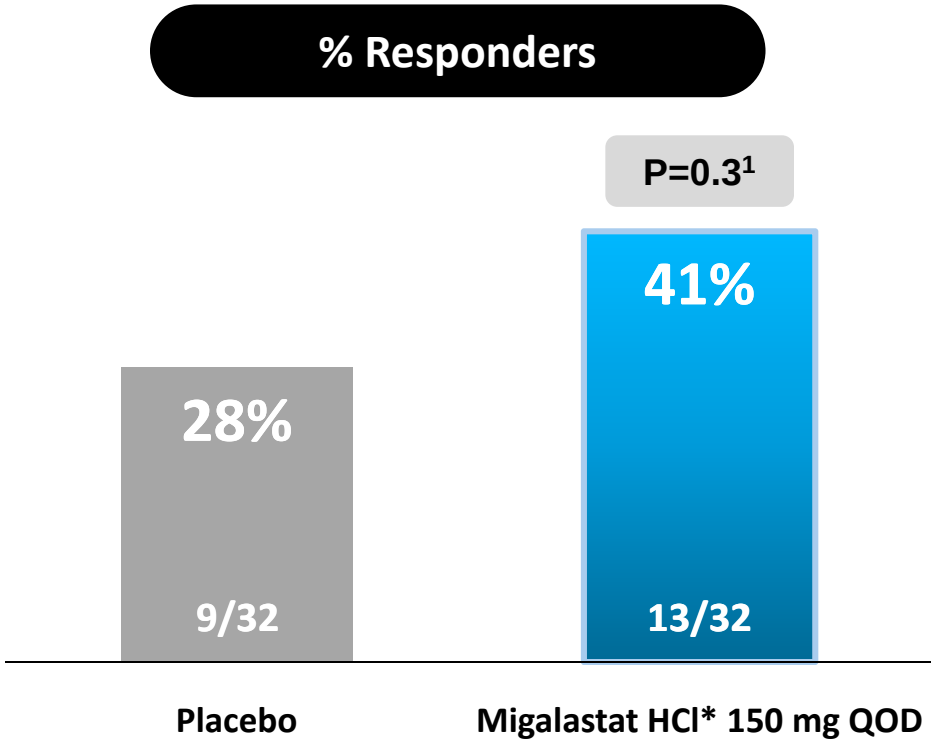


Pharmacological Chaperones

Migalastat HCl Monotherapy for Fabry Disease

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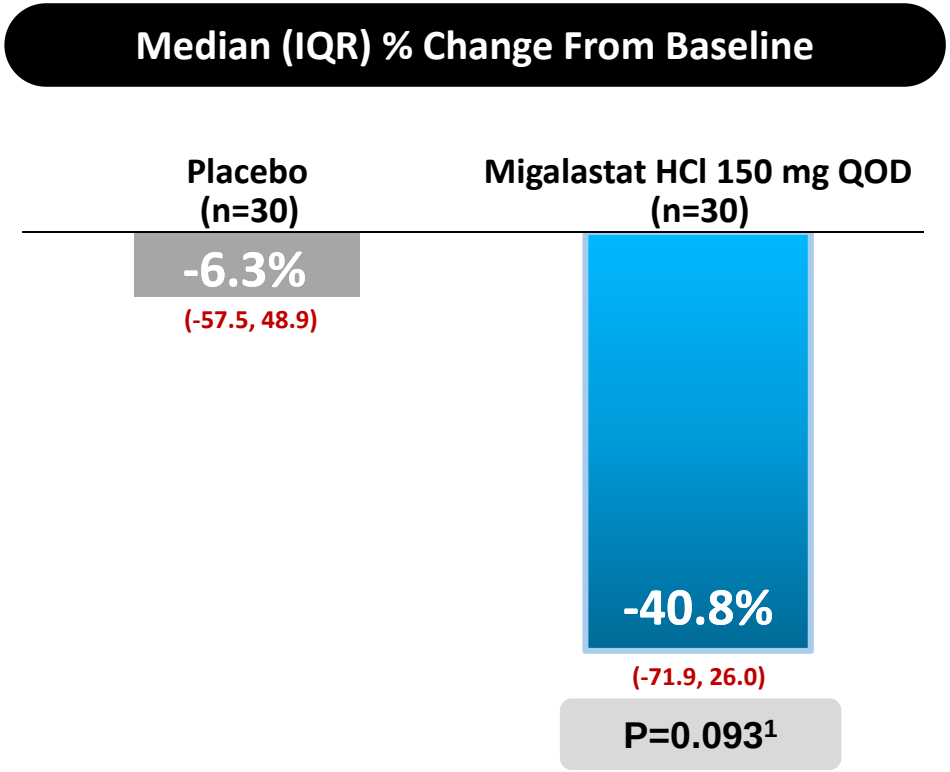
Phase 3 Study 011: Top-Line 6-Month Results

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%

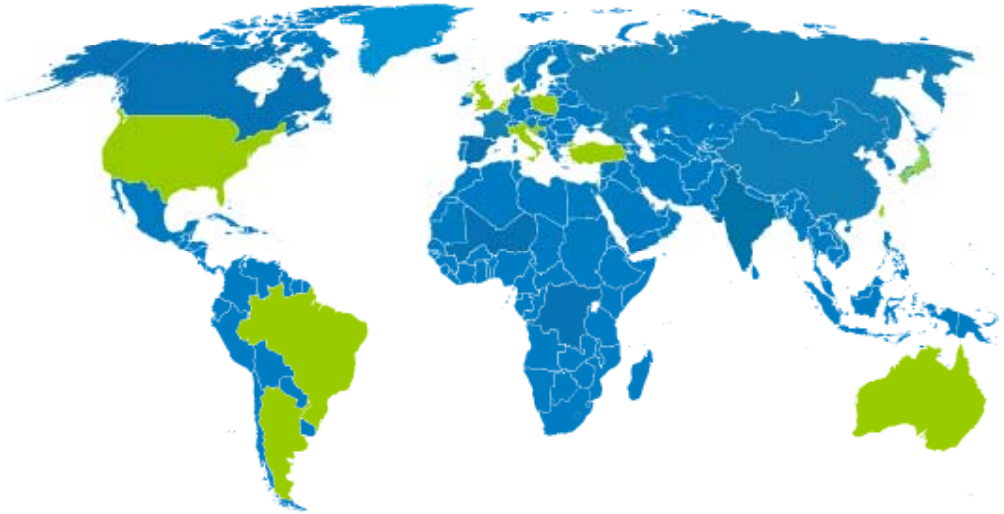
No Serious Adverse Events Deemed by Investigators to be Treatment-Related
No Withdrawals Due to Adverse Events

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

Migalastat HCl Monotherapy: Development Strategy

Assembling Robust Dataset to Maximize Chances for U.S. Approval of Migalastat HCl Monotherapy for Fabry Patients with Amenable Mutations

- We remain fully committed to advancing migalastat HCl monotherapy, which we are developing with GSK
- Study 011 Stage 2 (12-month) top-line data expected 4Q13
- Study 012 top-line data anticipated 2H14
- FDA meeting anticipated 2H14 to discuss U.S. approval pathway

Anticipated Milestones

Building Shareholder Value

2H13	Top-Line 12-Month Data from Phase 3 Fabry Monotherapy Study 011
	Initiation of Phase 2 Repeat-Dose Pompe Co-Administration Study
1H14	Initiation of Phase 1/2 Fabry Co-Formulation Study
	Initial Data from Phase 2 Repeat-Dose Pompe Co-Administration Study
2H14	Top-Line Data from Phase 3 Monotherapy Study 012
	FDA Meeting to Discuss Fabry Monotherapy Approval Pathway
	Phase 1 Data from Phase 1/2 Fabry Co-Formulation Study

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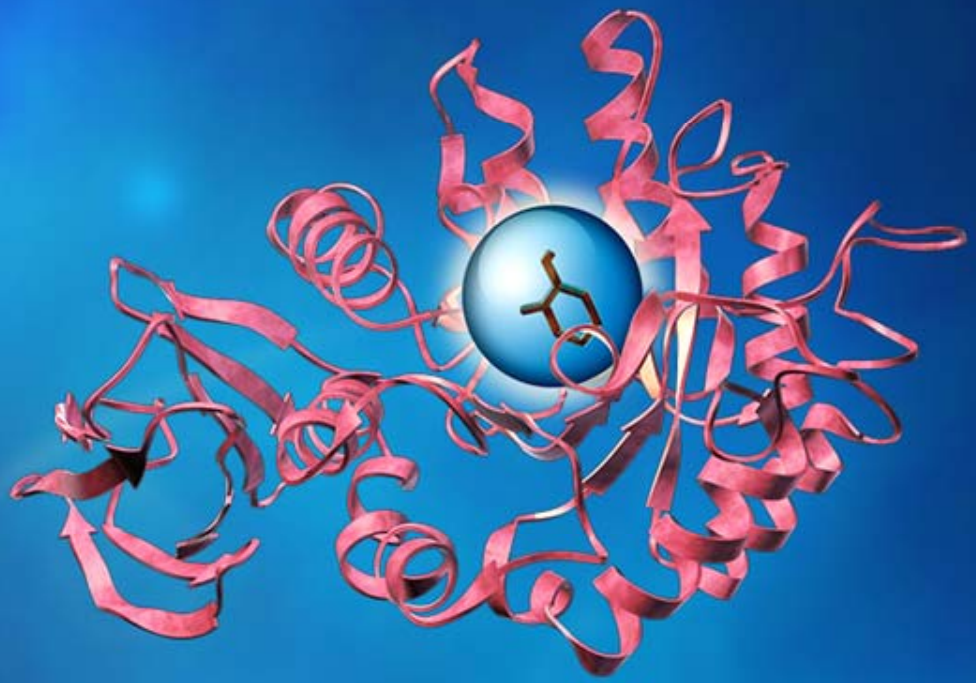

Canaccord Genuity 33rd Annual Growth Conference

John F. Crowley,
Chairman and Chief Executive Officer

at the forefront of therapies for rare and orphan diseases