UBS Annual Global Life Sciences Conference

Bradley L. Campbell

Chief Business Officer

At the Forefront of Therapies for Rare and Orphan Diseases™

September 20, 2012

Safe Harbor

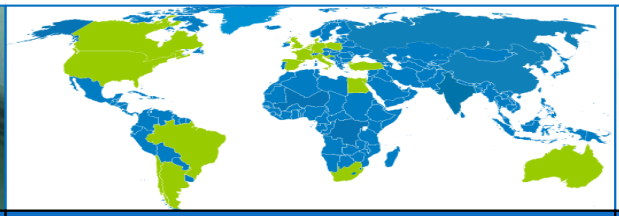
This presentation contains “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995 relating to business, operations and financial conditions of Amicus including but not limited to preclinical and clinical development of Amicus’ candidate drug products, 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, 2011. All forward-looking statements are qualified in their entirety by this cautionary statement, and Amicus undertakes no obligation to revise or update this news release to reflect events or circumstances after the date hereof.

Amicus is Business Led & Science Driven

At the Forefront of Therapies for Rare and Orphan Diseases™

Pharmacological Chaperone Platform Technology

- Proprietary platform & IP
- Small molecules targeting misfolded and unstable proteins
- Stabilize & enhance patient's own enzyme; *or*
- Potential to stabilize & enhance ERT products for lysosomal storage disorders (LSDs)



Global Clinical Capabilities & Pipeline

- Global expertise in rare disease clinical research, medical affairs and patient advocacy
- Clinical sites in over 20 countries
- Multiple Fabry Phase 3 & Phase 2 programs
- Pompe Phase 2 program underway

gsk GlaxoSmithKline

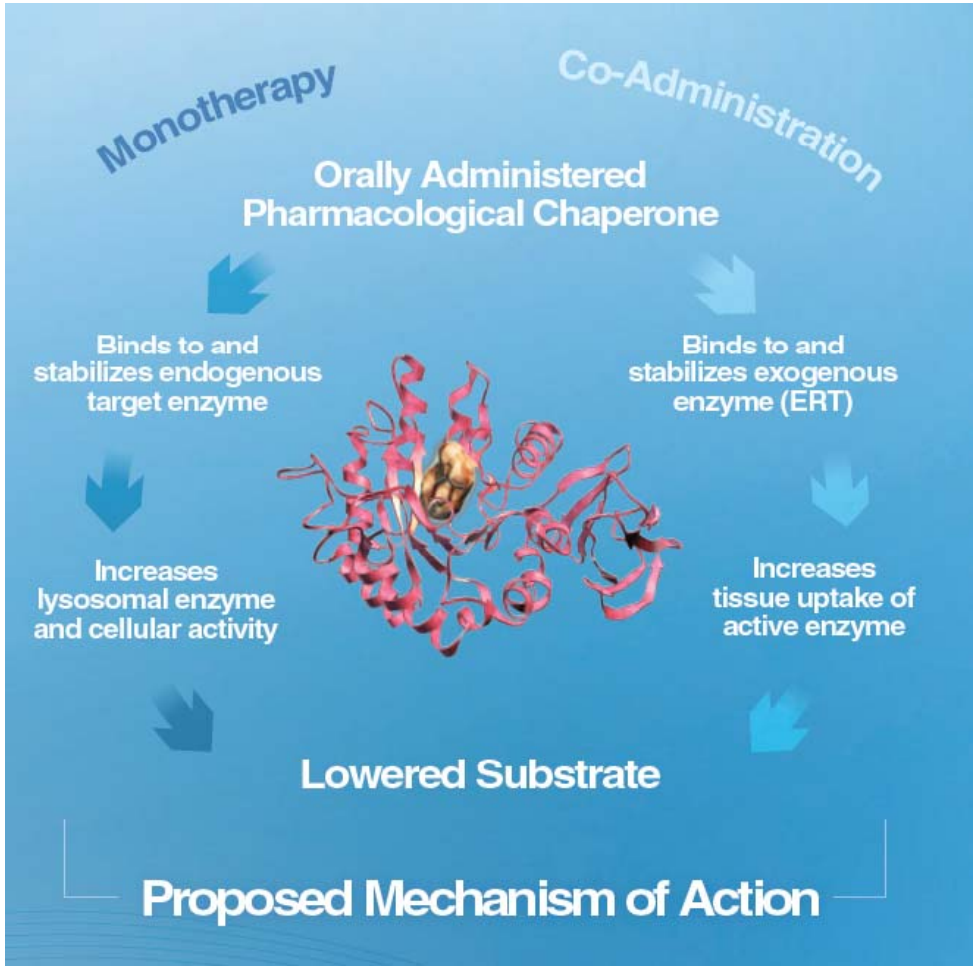
Alliance with GSK Rare Diseases

- ~19.9% FOLD ownership stake
- All uses of migalastat HCl for Fabry disease
- Global co-development and cost-sharing
- Amicus to commercialize in U.S.; GSK ex-U.S.

Pharmacological Chaperones

One Technology, Two Novel Applications

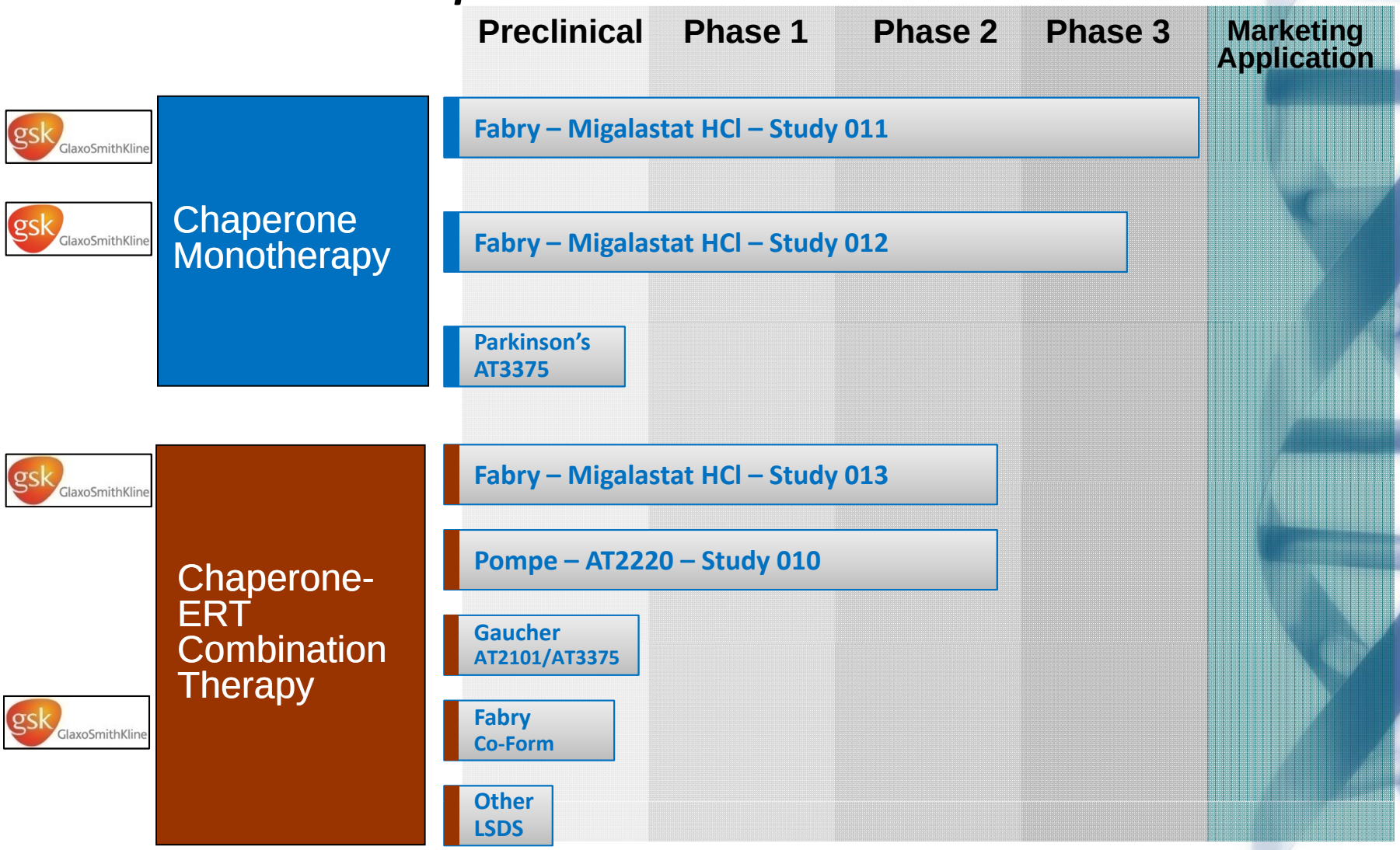
- Based on patient's own mutated enzyme
- Potential alternative to Enzyme Replacement Therapies (ERTs)



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

Pharmacological Chaperones:

Advanced Product Pipeline



Amicus & GSK Expand Fabry Collaboration

Product and Strategic Alliance

Migalastat HCl for Fabry Disease

- Collaboration to co-develop all uses of migalastat HCl
- Migalastat HCl to be commercialized by Amicus (US) and GSK (ROW)
- Additional $18.6M GSK equity investment (19.9% FOLD ownership)
- Global development cost sharing
- GSK eligible for U.S. approval and launch milestones for all Fabry programs

Strong Financial Position

- **Cash position**
  - $95.8M at June 30, 2012 vs. $56.0M at December 31, 2011
  - ≥ $90M projected at December 31, 2012, expected to fund current operating plan beyond 2013
- **Strengthening balance sheet in 3Q12**
  - $18.6M GSK equity investment
  - $3.5M development milestone received from GSK
- **FY12 OpEx guidance:**
  - Upper end of previous guidance range of $37M - $43M
  - Net of anticipated Fabry cost-sharing

Miglastat HCl Monotherapy for Fabry Disease

Global Phase 3 Registration Studies

Both Studies Evaluating Migalastat HCl 150 mg, Every-Other-Day in Patients with Amenable Genetic Mutations

STUDY 011

- U.S. Registration Study
- Placebo-controlled
- 67 patients
- 6-month surrogate endpoint: kidney GL-3
- Eligible for accelerated approval
- 6-month primary treatment period complete – data expected 4Q12
- 6-month open-label treatment extension and Phase 3 extension studies ongoing

STUDY 012

- Global Registration Study
- Switch from ERT
- 50 patients (> majority enrolled)
- 18-month clinical endpoint: kidney function (GFR)
- Full enrollment targeted by YE12

Fabry Study 011

Phase 3 Confidence

Study 011 Design Contributes to Potential for Phase 3 Success

Phase 2 Experience	▪ >150 patient-years of experience ▪ 17 Phase 2 patients remain on migalastat HCl monotherapy ▪ Positive results on renal and urine GL-3 clearance ▪ Long-term trends toward stabilization of kidney function
Strict Entry Criteria	▪ Naïve to ERT / no ERT in past 6 months ▪ Amenable mutations ▪ Urine GL-3 ≥ 4x normal
Improved Histological Methodology	▪ Published BLISS-VM methodology more advanced, sensitive & objective* vs. Thurberg-LM
Study 011 Status	▪ 6-month primary treatment period completed 2Q12 – data anticipated 4Q12 ▪ 6-month treatment extension period expected to complete 4Q12

*Barisoni *et al.,* **Archives of Pathology & Laboratory Medicine**, 2012

Fabry Study 011

Patient Disposition to Date (as of 7/31/12)

Low dropout rate and high conversion to extension studies

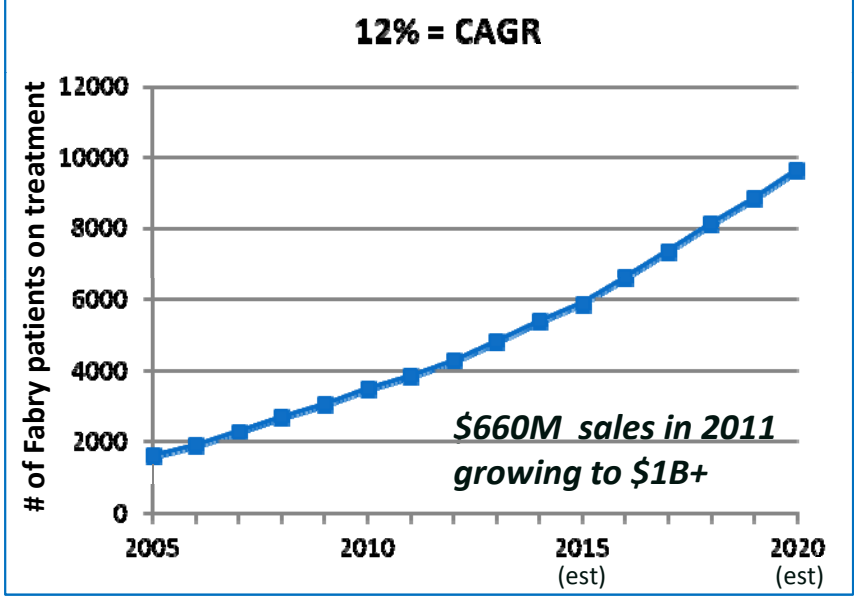
- 63 completed 6-mo. double-blind treatment period (~6% dropout rate)
- 63 continued in 6-mo. open-label treatment extension
- 40 to date completed Study 011 (6-mo. treatment + 6-mo. extension)
- 38 of 40 currently enrolled in open-label extension studies

WW Fabry Market

Significant Growth Opportunities

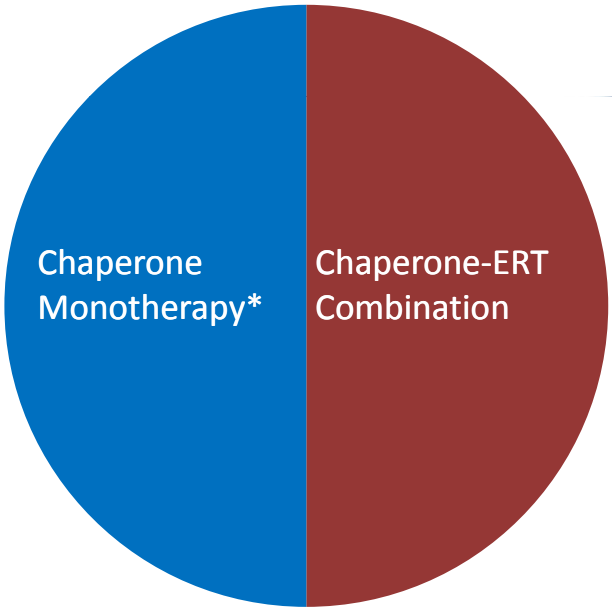
All Fabry patients potentially eligible to receive migalastat HCl upon approval as monotherapy or in combination with ERT

Market Opportunity



Sources: Analyst Reports, Company 10Ks, Market Research

Projected Future Market Distribution

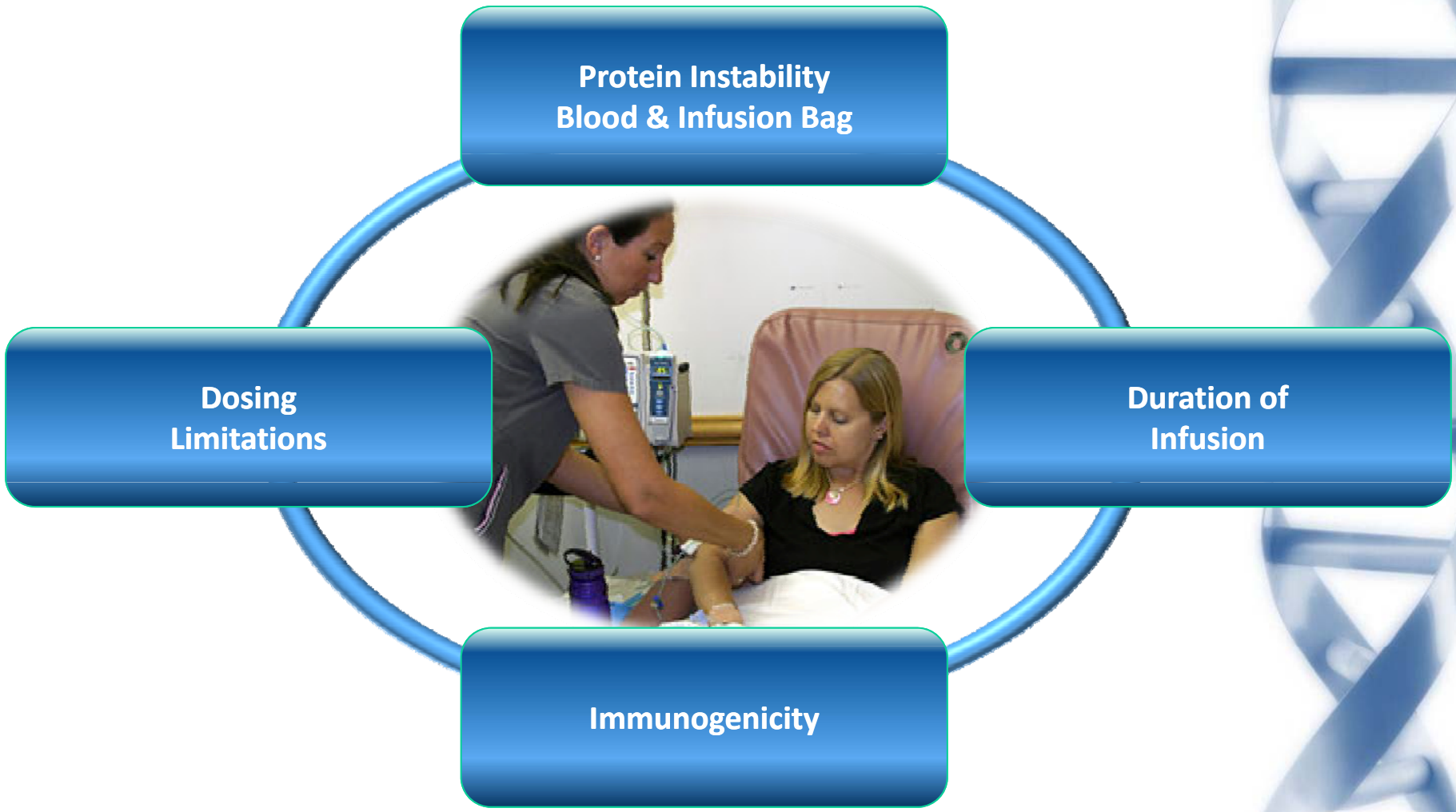


* Includes estimated size of undiagnosed population with amenable mutations; Spada 2006, Hwu 2009, Mechtler 2011, Burton 2012

CHAPERONE-ERT COMBINATION TECHNOLOGY

LSD Products Today

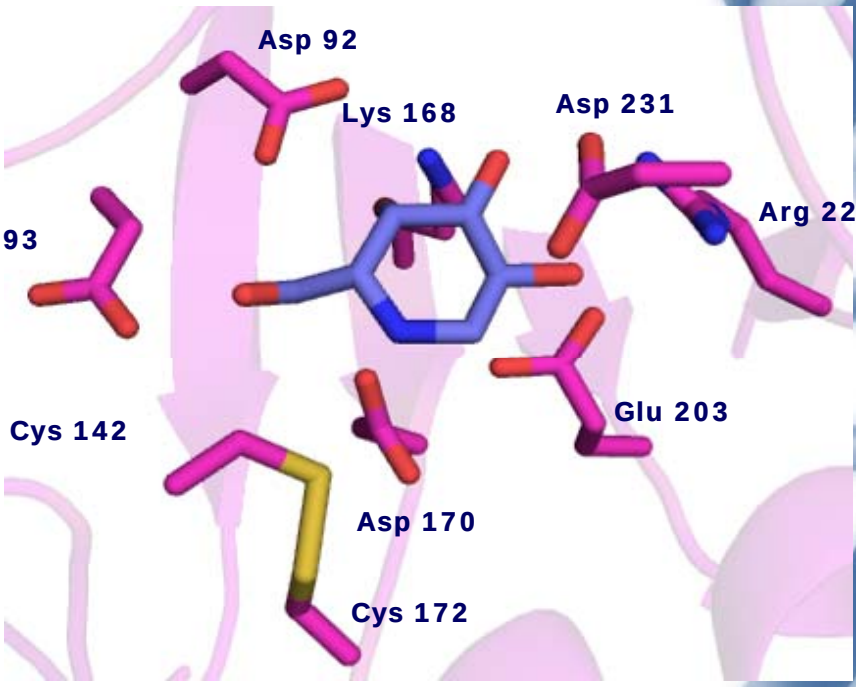
Potential Issues



ERT-Chaperone Central Principle

Protein Stability & Conformation

Structure of α-Gal A Active Site Bound to AT1001*



*Lieberman, D'aquino, Ringe and Petsko, **Biochemistry**, 2009

Phase 2 Chaperone-ERT Co-Administration Studies

Positive Preliminary Results in Different LSDs with 2 Different Chaperones

FABRY STUDY 013

- Drug-drug interaction study
- Single administration of migalastat HCl (2 ascending doses), prior to ERT (Fabrazyme® or Replagal®)
- Plasma PK & PD (skin biopsies)
- **Positive preliminary results vs. ERT alone**
  - Migalastat HCl 150 mg + Fabrazyme
- 4 cohorts completed
  - Migalastat HCl 150 mg + Fabrazyme
  - Migalastat HCl 150 mg + Replagal
  - Migalastat HCl 450 mg + Fabrazyme
  - Migalastat HCl 450 mg + Replagal
- Additional results expected at Fall 2012 scientific congress

POMPE STUDY 010

- Drug-drug interaction study
- AT2220 (4 ascending doses), prior to ERT (Myozyme®/Lumizyme®)
- Plasma PK & PD (muscle biopsies)
- **Positive preliminary results vs. ERT alone**
  - AT2220 Cohort 1 + ERT
  - AT2220 Cohort 2 + ERT
- **Cohorts 1-3 completed, reviewed by DSMB**
- Cohort 4 now enrolling
- Additional results expected at Fall 2012 scientific congress
- Final results anticipated 4Q12

Fabry Study 013

Overview

Study Population

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

ERTs Evaluated

- 0.5 mg/kg Fabrazyme (every 2 weeks) – complete
- 1.0 mg/kg Fabrazyme (every 4 weeks) – complete
- 1.0 mg/kg Fabrazyme (every 2 weeks)/labeled dose – complete
- 0.2 mg/kg Replagal (every 2 weeks) – complete

Migalastat HCl Doses Evaluated

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

Endpoints Studied

- Safety
- α-Gal A activity in plasma and in skin +/- Migalastat HCl

Day 1 — ERT Alone (period 1); ERT + Migalastat HCl (pd 2)

- Baseline Skin Biopsy for α-Gal A Activity (predose)
- Serial Blood Sampling for Plasma α-Gal A Activity

Day 2 (24 hours)

- Skin Biopsy for α-Gal A Activity

Day 7

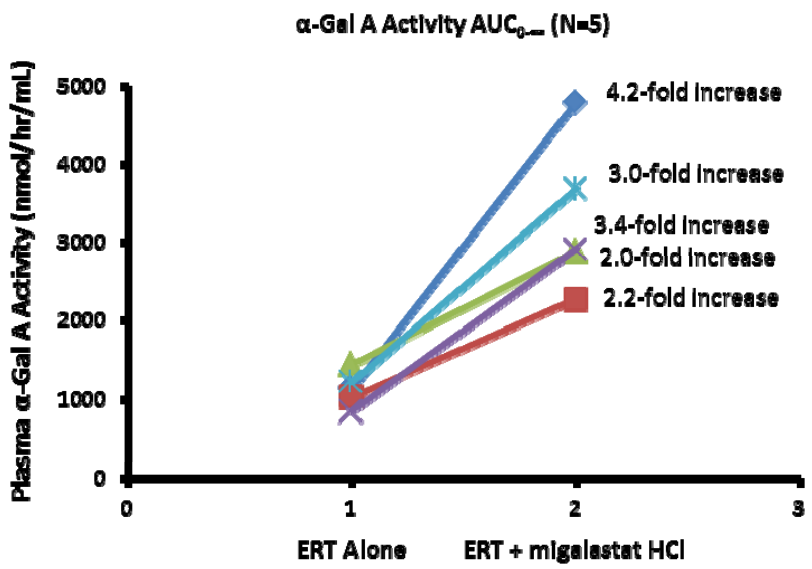
- Skin Biopsy for α-Gal A Activity

Fabry Study 013

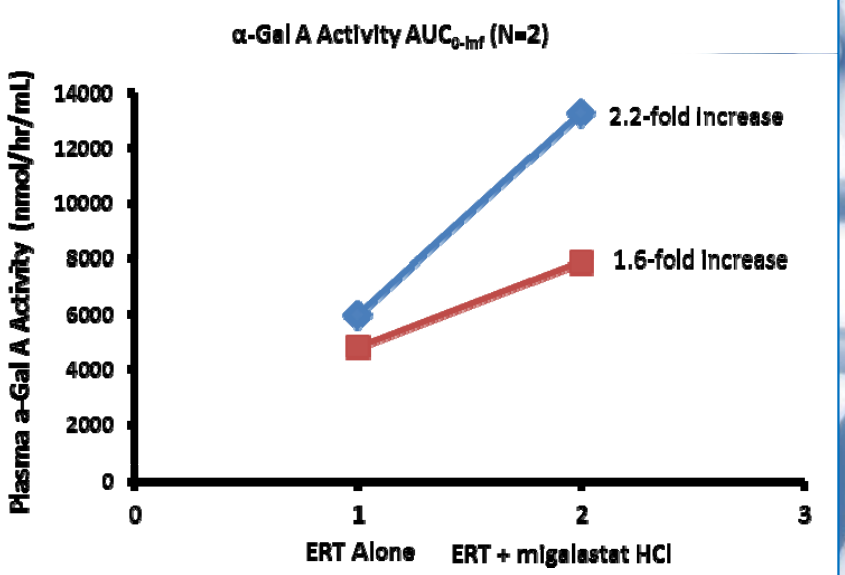
Plasma PK Preliminary Data (n = 7)

Co-Administration Increases Levels of Active Enzyme in Plasma ~2- to 4-Fold vs. ERT Alone in First 7 Patients

Migalastat HCl 150 mg + Fabrazyme 0.5 mg/kg



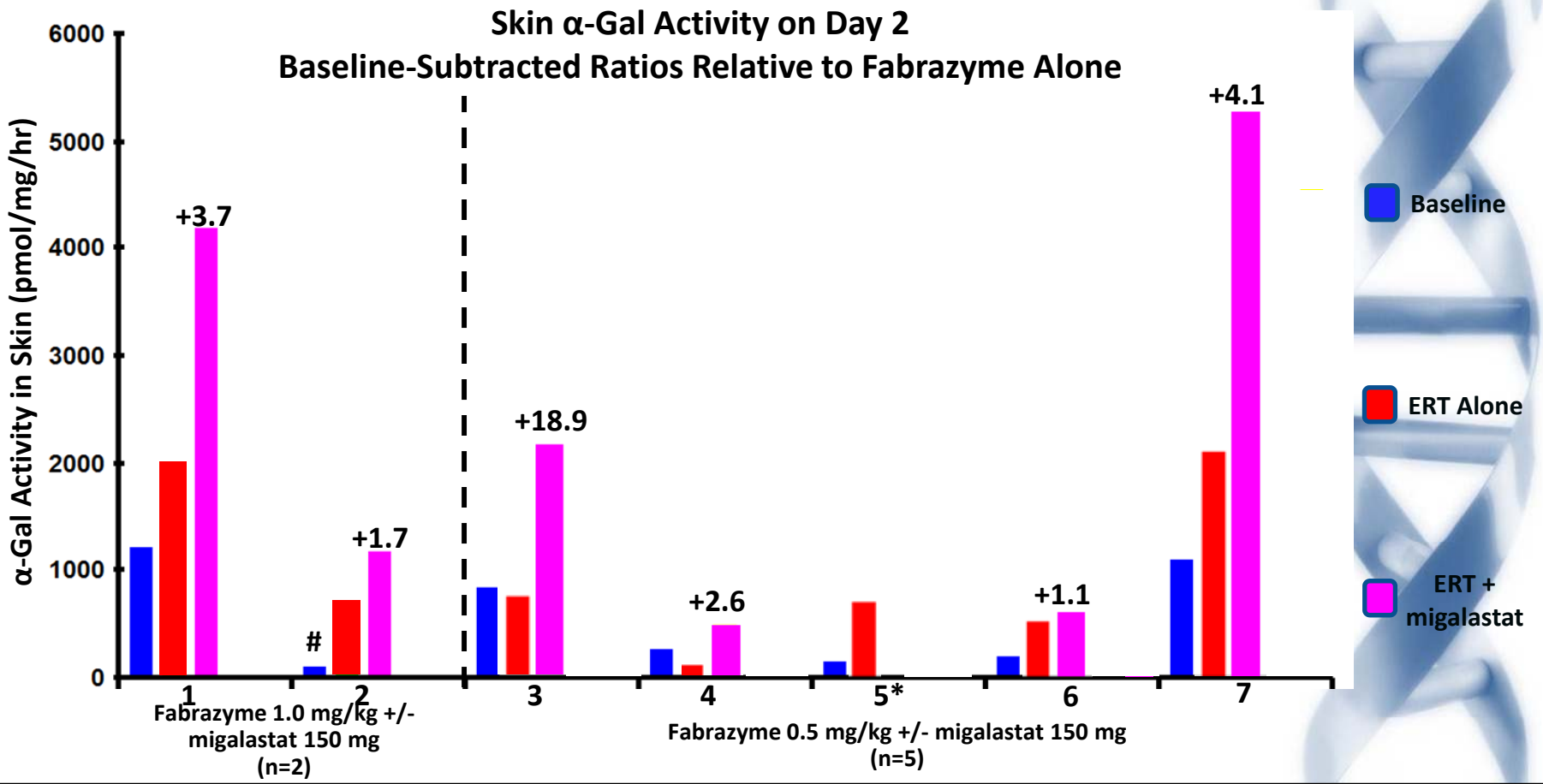
Migalastat HCl 150 mg + Fabrazyme 1 mg/kg



Fabry Study 013

Skin Biopsies – Preliminary Data (n = 7)

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



*Day 2 Biopsy Sample Lost

Below limit of quantitation; lower limit of analytical range used as baseline value

Pompe Co-Administration

Phase 2 PK/PD Study 010

Study Population	▪ ≤ 22 Pompe Patients on ERT
ERT Evaluated	▪ Myozyme/Lumizyme
AT2220 Doses Evaluated	▪ 4 increasing doses (single dose, given prior to ERT infusion)
Endpoints Studied	▪ Safety ▪ GAA activity in plasma and in muscle +/- AT2220
Status	▪ Positive preliminary results: increases in Pompe enzyme (GAA) activity in 10/10 patients in first 2 dose cohorts ▪ Final results expected 4Q12

Day 1 — ERT Alone (period 1); ERT + AT2220 (pd 2)

- Serial blood sampling for plasma GAA activity
- Total protein concentration

Day 3 - Muscle Biopsy

- GAA activity in skeletal muscle
- AT2220 clearance
- Half of subjects in groups 2-4

Day 7 - Muscle Biopsy

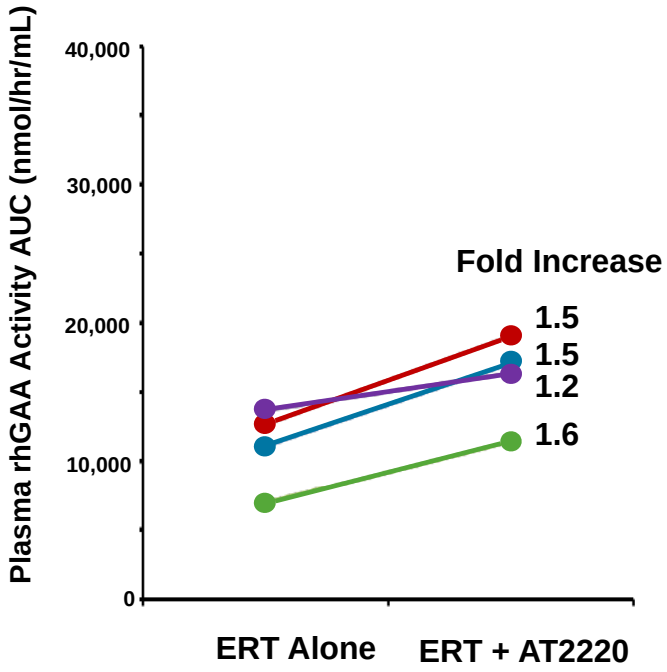
- GAA activity in skeletal muscle
- AT2220 clearance
- All subjects in group 1; half of subjects in groups 2-4

Pompe Study 010

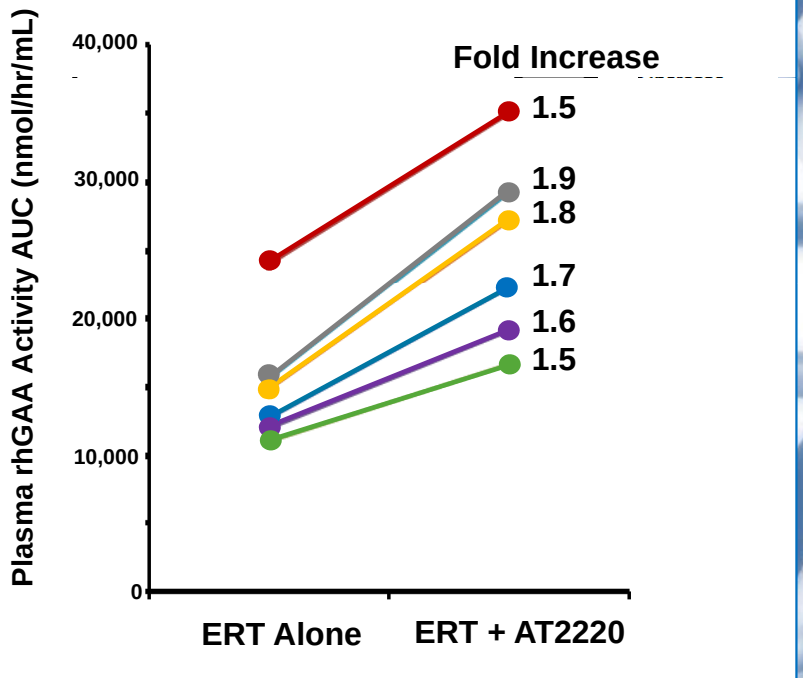
Plasma PK Preliminary Data (n=10)

Co-Administration Increases Levels of Active Enzyme (GAA) in Plasma up to ~2-Fold vs. ERT Alone in Two Lowest Dose Cohorts of AT2220

Cohort 1: AT2220 (50 mg) + Myozyme/Lumizyme (n=4)



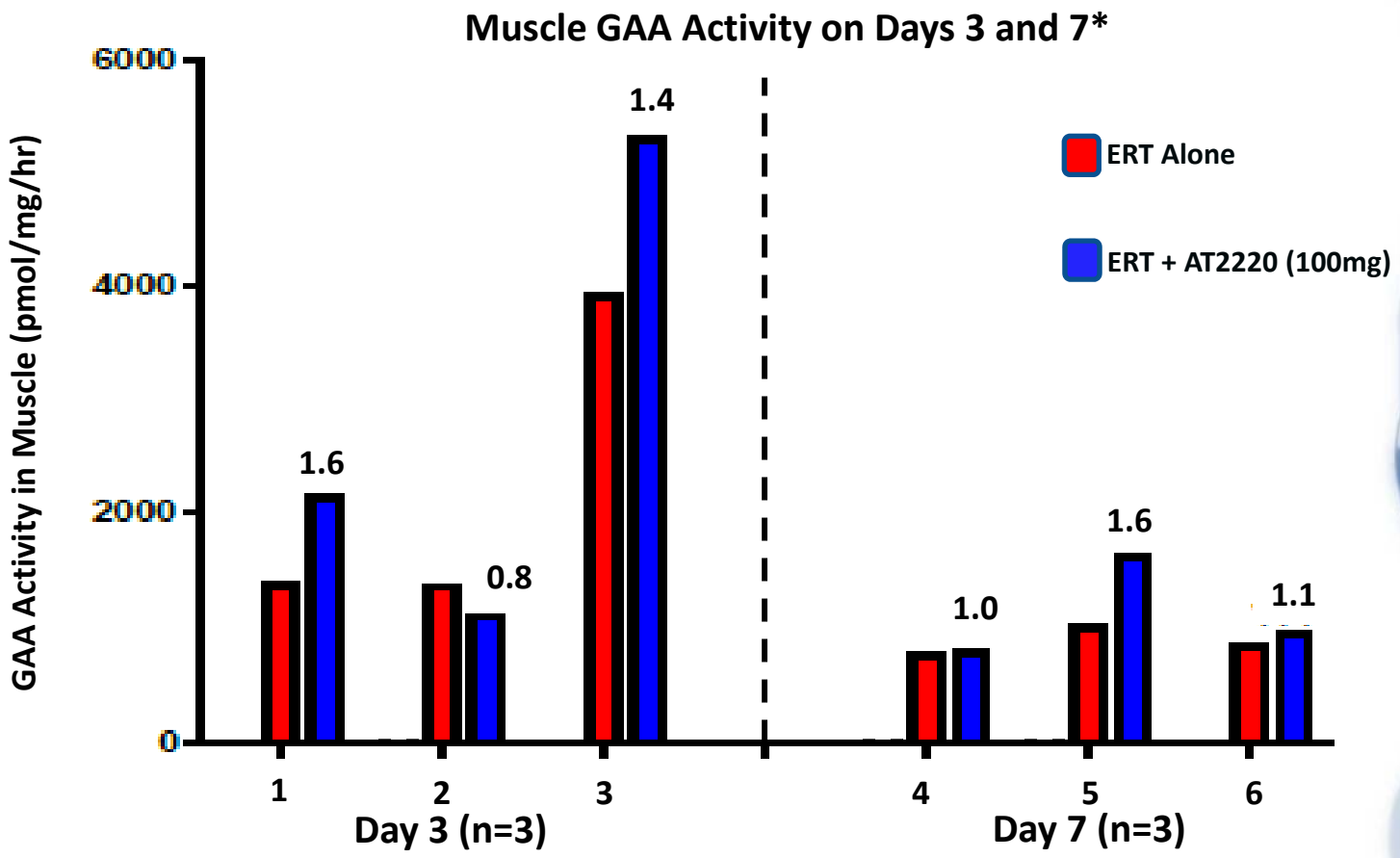
Cohort 2: AT2220 (100 mg) + Myozyme/Lumizyme (n=6)



Pompe Study 010

Needle Core Muscle Biopsies – Preliminary Data (n = 6)

Cohort 2 Muscle Biopsies Suggest Co-Administration Increases Enzyme Uptake into Muscle vs. ERT Alone

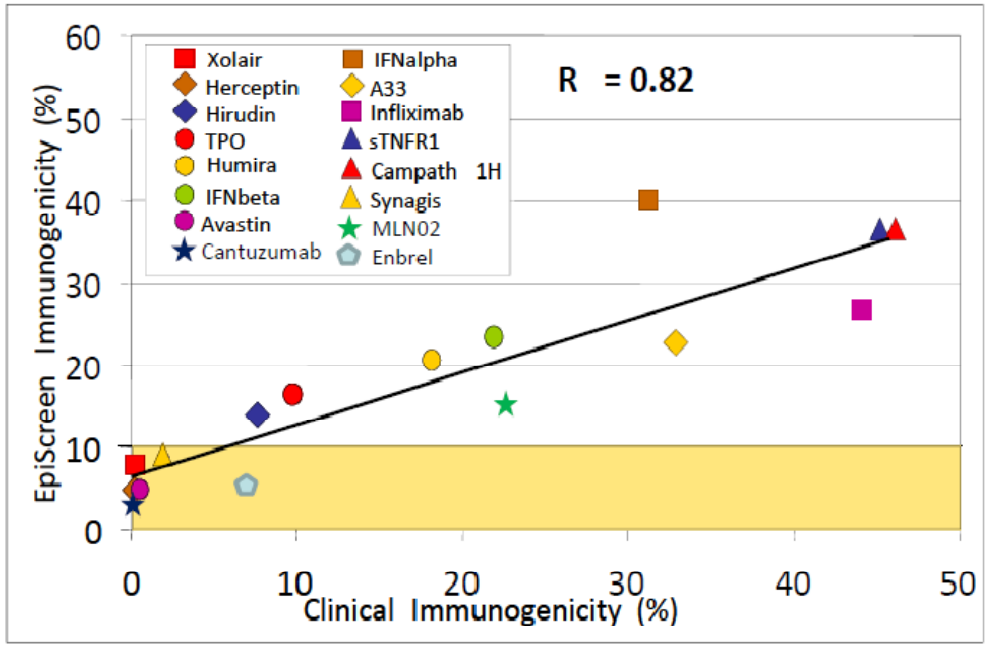


*Ratios Relative to ERT Alone

Pompe ERT-Related Immunogenicity

MDA Grant Supports Ongoing Studies to Evaluate Immunogenicity of Pompe ERT +/- AT2220

Episcreen™ Assays
Predictive of Clinical Immunogenicity for Existing Therapeutic Proteins



Mean frequency of anti-therapeutic antibodies (source PubMed)

- **Investigating T-cell response in blood samples from:**
  1) 50 healthy volunteers with different HLA types
  2) Study 010 patients (correlate HLA type, IgG titer and neutralizing antibody responses with T-cell stimulation index)
- **Correlation between HLA type and immune response may help design future studies**

Continuum of Innovation

Multiple Paths Forward for Chaperone Therapy

Envisioning New Product Advances Unique to Each LSD

- Standard of Care ERTs
- Chaperone Monotherapy
- Chaperone-ERT Co-Administration
- Chaperone-ERT Co-Formulation
- Chaperone-ERT Co-Formulation + Improved Delivery/Regimen
- Chaperones Co-Formulated with Proprietary ERTs
- Chaperones Co-Formulated with Proprietary ERTs + Improved Delivery/Regimen

Chaperone-ERT Co-Formulation:

Strategic relationship leverage JCR's Biological Expertise

Formulation and Preclinical Studies Conducted Over Past 16 Months

- Headquartered in Japan, listed on Tokyo & Osaka exchange
- >20 years in biologics manufacturing
- 2 marketed recombinant proteins (HGH, EPO)
- 5 recombinant proteins in development
- JCR / GSK collaboration established 2009

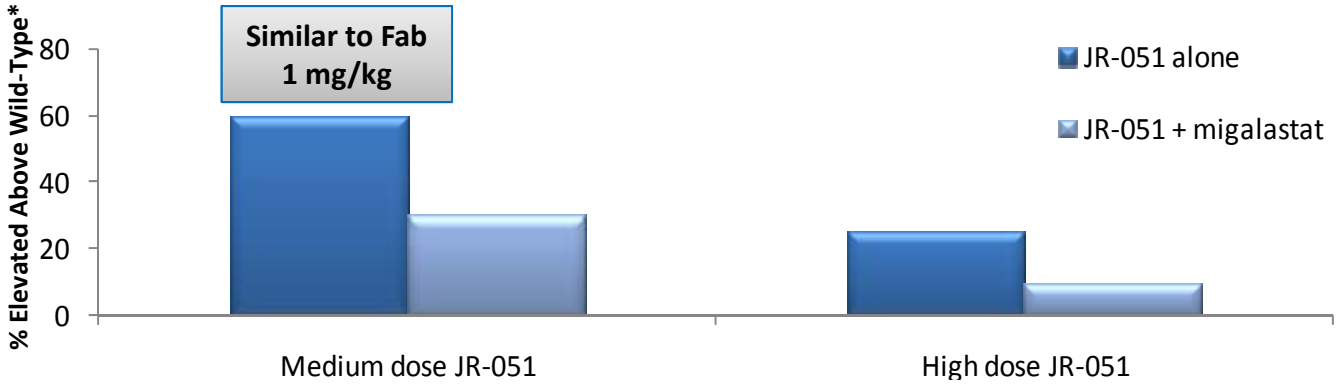
Chaperone-ERT Co-Formulation for Fabry Disease

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

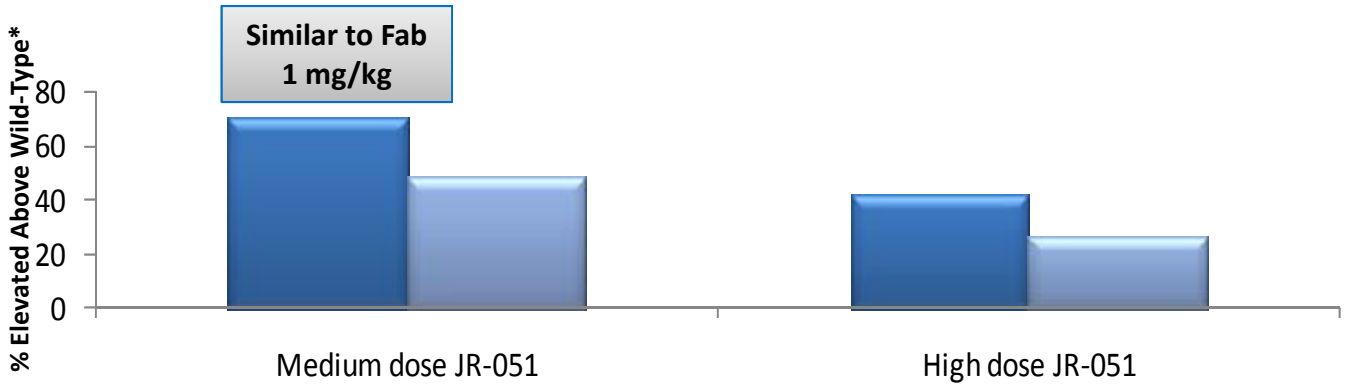
Co-formulation with Migalastat Results in Significantly Greater GL-3 Reduction

Preliminary Results

Heart GL-3



Kidney GL-3



*0 = wild-type, 100 = untreated KO mouse

2012 Anticipated Milestones

Building Shareholder Value

Fabry	✓ Phase 2 Study 013 Preliminary Co-Administration Data	Q1
	✓ Preclinical Chaperone-ERT Co-Formulation Results	Q3
	✓ Phase 3 Monotherapy Study 011 – 6-mo. treatment completed	Q3
	▪ Phase 2 Study 013 Data	Fall 2012
	▪ Phase 3 Study 011 6-Month Data	Q4
	▪ Phase 3 Study 012 Completion of Enrollment	Q4
Pompe	✓ MDA Grant to Investigate ERT Immunogenicity	Q1
	✓ Phase 2 Study 010 Preliminary Co-Administration Data	Q2
	▪ ERT Immunogenicity Preclinical Results	Q3
	▪ Additional Phase 2 Study 010 Data	Fall 2012
	▪ Final Phase 2 Study 010 Data	Q4
Parkinson's	▪ Completion of Additional AT3375 IND-Enabling Studies	Q4

Amicus Therapeutics

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