

Migalastat Monotherapy Phase 3 Cardiac Data

January 8, 2015

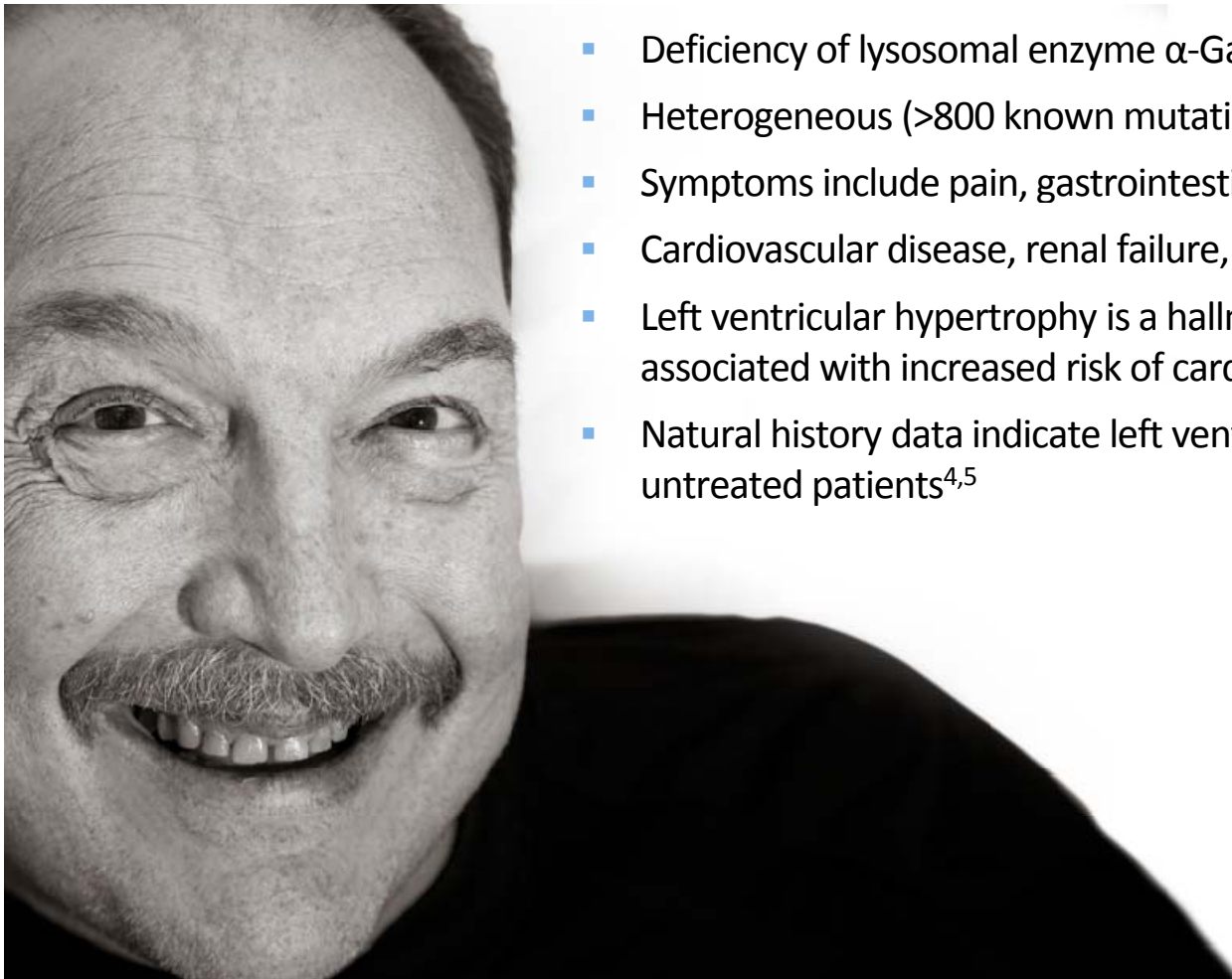
*at the forefront of therapies
for rare and orphan diseases*

Safe Harbor

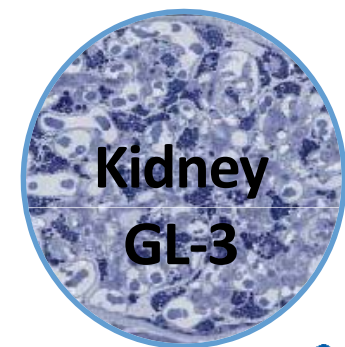
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Fabry Disease Overview

Cardiovascular Disease Recognized as Leading Cause of Death in Fabry Patients^{1,2}



- Deficiency of lysosomal enzyme α -Gal A leading to GL-3 accumulation
- Heterogeneous (>800 known mutations)
- Symptoms include pain, gastrointestinal problems, angiokeratomas
- Cardiovascular disease, renal failure, stroke are leading causes of morbidity
- Left ventricular hypertrophy is a hallmark of Fabry cardiomyopathy, associated with increased risk of cardiac events and mortality³
- Natural history data indicate left ventricular mass increases progressively in untreated patients^{4,5}



(Previously Reported)

Cardiac Data (LVMi): Phase 3 Study 012

Migalastat Previously Demonstrated Statistically Significant Decrease in LV Mass Index (LVMi) in Subjects Switched from ERT

Left Ventricular Mass Index (LVMi) (g/m^2)* in Phase 3 Study 012 Change from Baseline to Month 18

	Migalastat Baseline Mean (% abnormal) n=33	Migalastat Change from Baseline to M18 (mean, 95%CI) n=31	ERT Baseline Mean (% abnormal) n=16	ERT Change from Baseline to M18 (mean, 95%CI) n=13
Study 012 (Month 18)	95.3 (39%)	-6.6 (-11.0, -2.1)***	92.9 (31%)	-2.0 (-11.0, +7.0)

*Read in blinded manner in centralized lab every 6 months. Normal LVMI: 43-95 (female), 49-115 (male)

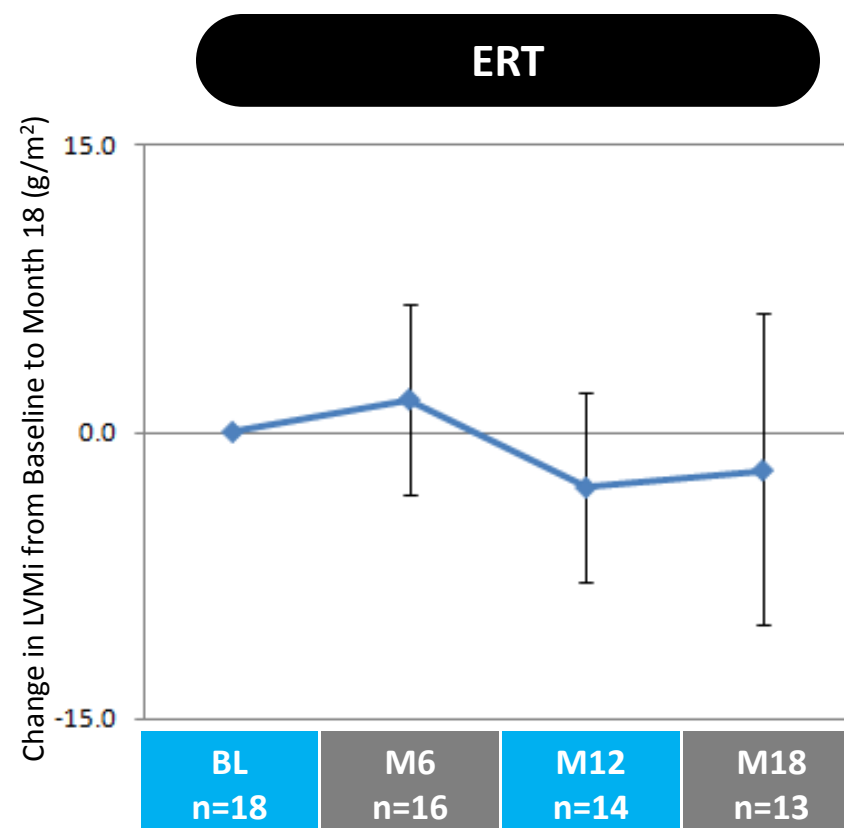
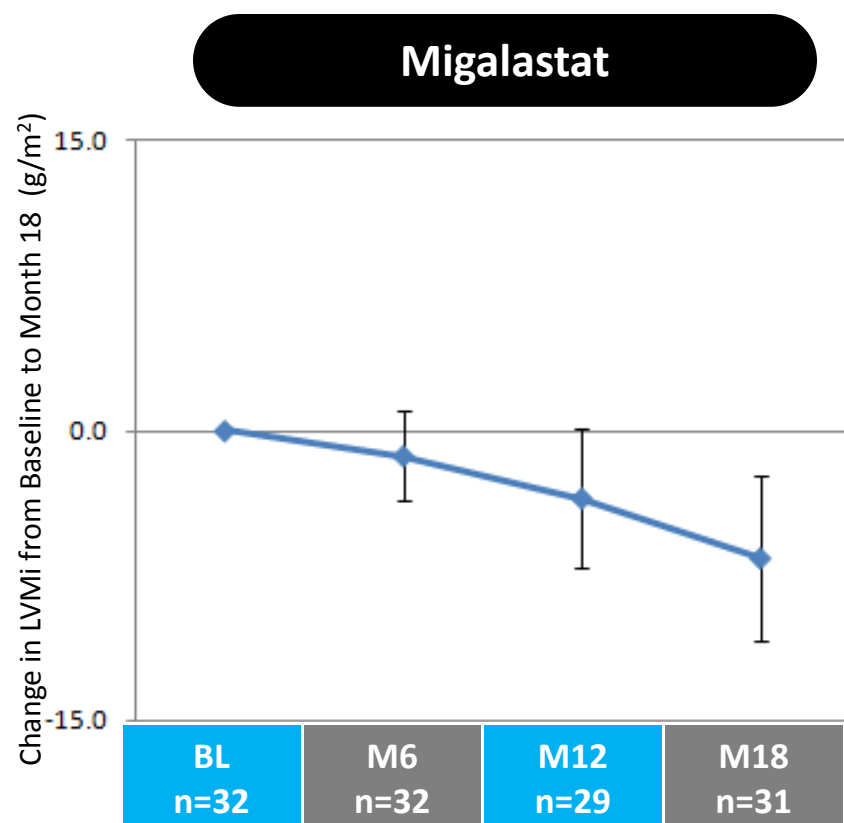
**mITT population

***Statistically significant (95% CI does not overlap zero)

(Previously Reported)

Cardiac Data (LVMi): Phase 3 Study 012

Reductions in LVMi Observed in Patients Switched from ERT Through Month 18 *



(NEW Data)

Cardiac Data (LVMI): Phase 3 Study 011+041

Migalastat Now Shows Statistically Significant Reduction in LVMI in Previously Untreated Patients

Left Ventricular Mass Index (LVMI) (g/m²)* Change from Baseline to Last Available Time Point

	Migalastat Baseline Mean (% abnormal) n=42	Migalastat Change from Baseline (mean, 95%CI) n=42
Study 011+041 (Avg 22 Months)**	97.5 (26%)	-8.0 (-13.5, -2.5)***

*Read in blinded manner in centralized lab every 6 months. Normal LVMI: 43-95 (female), 49-115 (male)

**All patients with amenable mutations with baseline and post-baseline values

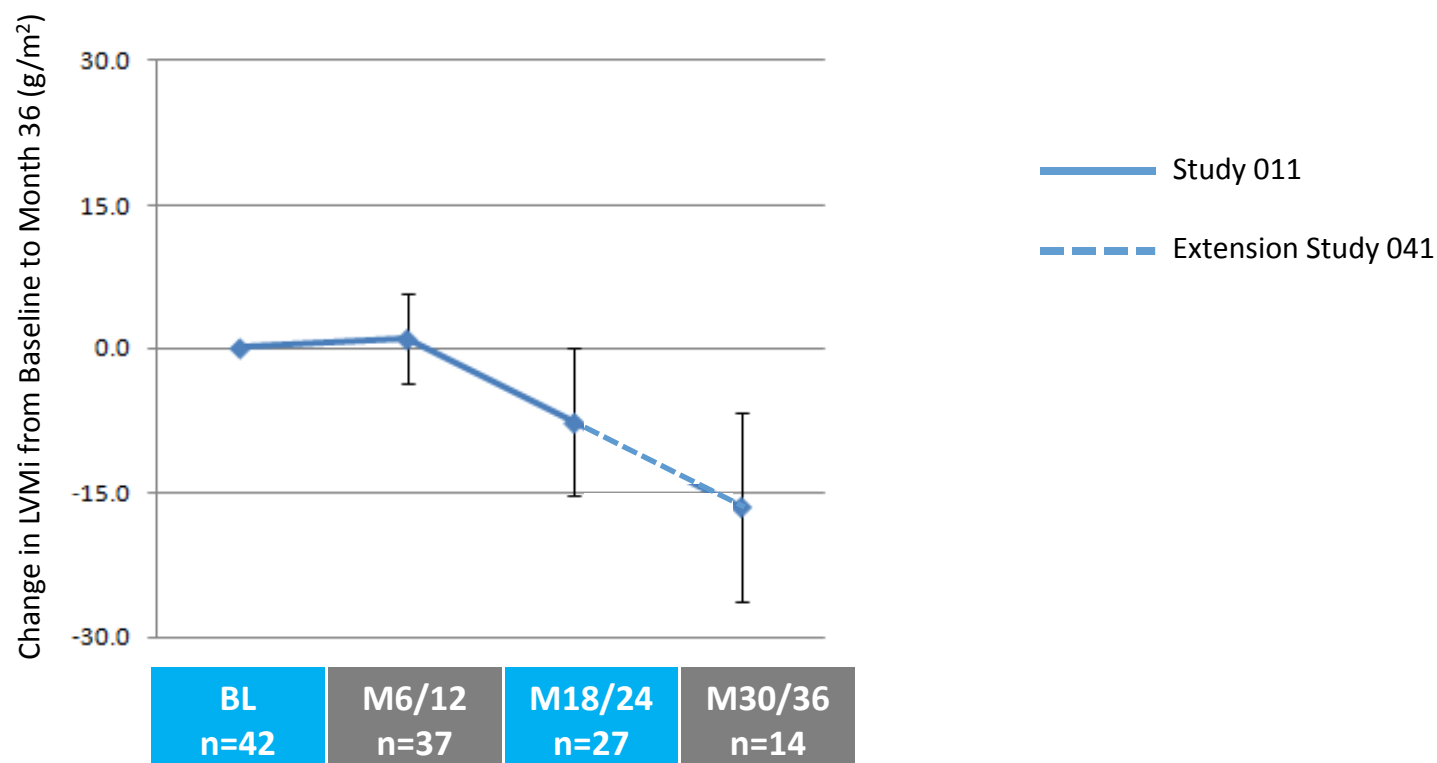
***Statistically significant (95% CI does not overlap zero)

(NEW Data)

Cardiac Data (LVMI): Phase 3 Study 011+041

New Data Also Show Migalastat Has Persistent and Increasing Positive Effect on LVMI Over Longer Periods of Time (Up to 36 Months)

Migalastat



Sample size differences are due to subjects not yet reaching a given timepoint or due to missing Echos

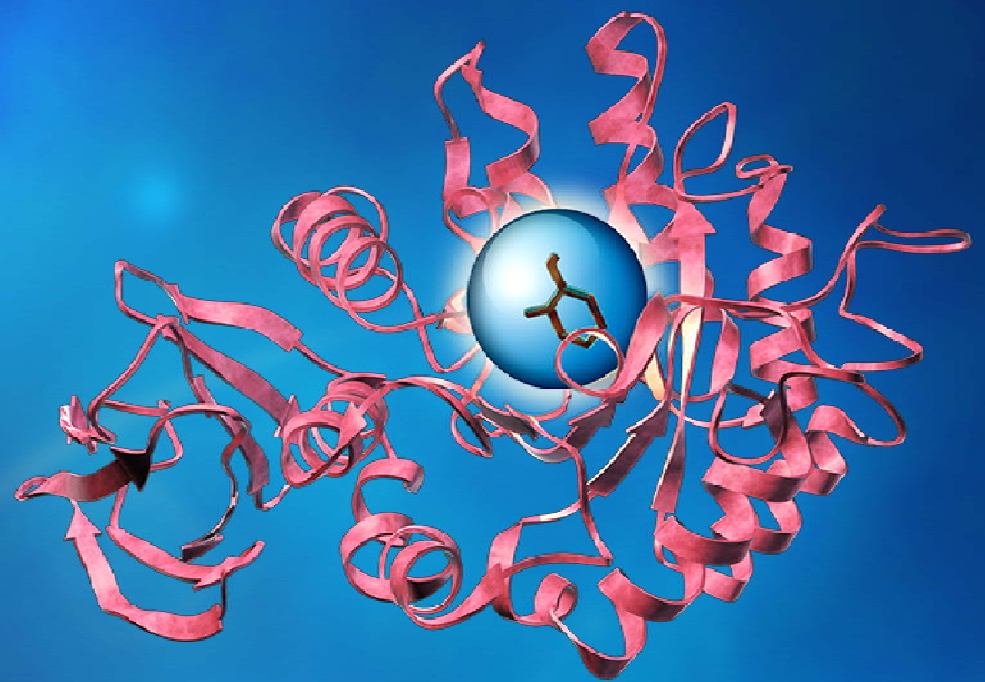
Global Regulatory Strategy

Cardiac Effect Strengthens Totality of Clinical Data Supporting Marketing Applications

- Complete data set from Phase 3 studies (011 and 012)
- 9 years of data in extension studies
- FDA meeting planned 1Q15

- MAA submission planned mid-2015 (Centralized Procedure)
- Comparability to ERT (Study 012)

- ROW regulatory path to be based on EMA and FDA submissions



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