

**UNITED STATES
SECURITIES AND EXCHANGE COMMISSION**

Washington, D.C. 20549

FORM 8-K

CURRENT REPORT

Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): **May 9, 2014**

AMICUS THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

Delaware

(State or other Jurisdiction of
Incorporation)

001-33497

(Commission File Number)

71-0869350

(IRS Employer Identification No.)

1 Cedar Brook Drive, Cranbury, NJ
(Address of Principal Executive Offices)

08512
(Zip Code)

Registrant's telephone number, including area code: **(609) 662-2000**

(Former name or former address if changed since last report.)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Item 7.01. Regulation FD Disclosure.

On May 9, 2014, Amicus Therapeutics, Inc. (the "Company") updated its Investor Presentation for the Migalastat Monotherapy Program (the "Presentation"). The Company's Chief Financial Officer, William D. Baird III and the Company's Chief Operating Officer, Bradley L. Campbell, will present some or all of this Presentation to various investors in the coming months. A copy of the presentation materials is attached hereto as Exhibit 99.1.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits: The Exhibit Index annexed hereto is incorporated herein by reference.

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SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

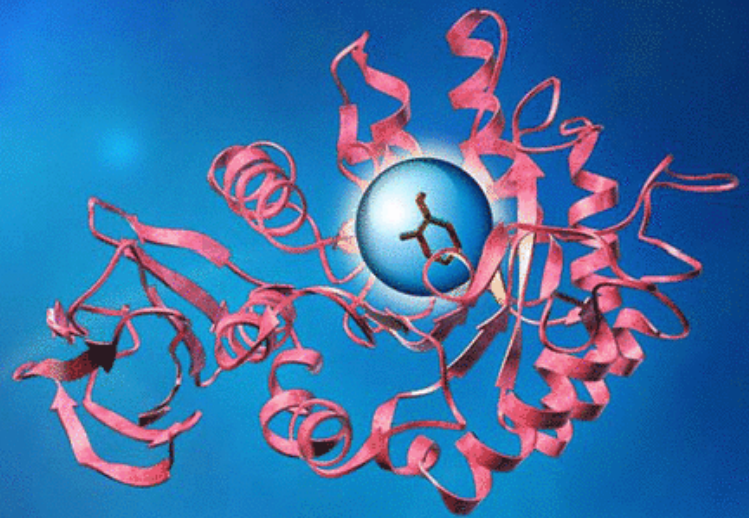
AMICUS THERAPEUTICS, INC.

Date: May 9, 2014

By: /s/ WILLIAM D. BAIRD III
Name: William D. Baird III
Title: Chief Financial Officer

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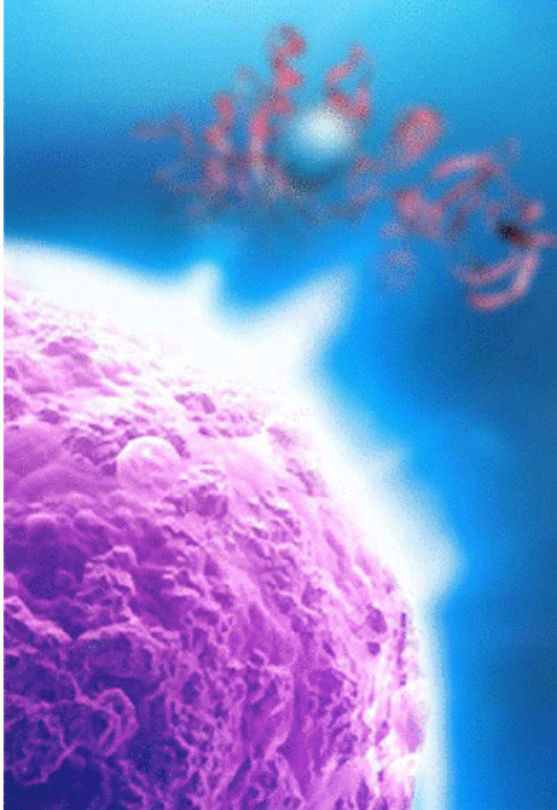
EXHIBIT INDEX



Corporate Overview

May 2014

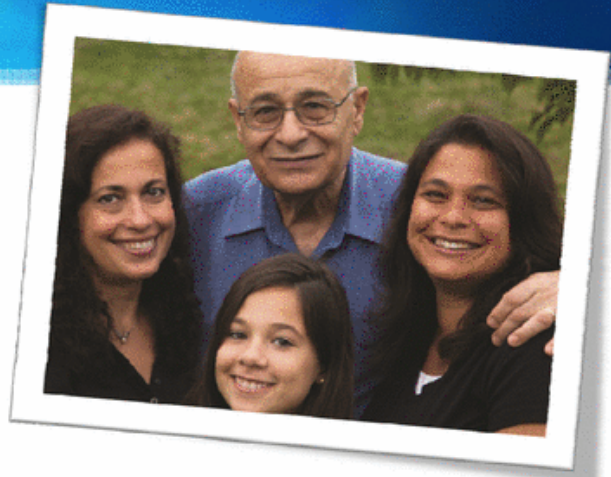
*at the forefront of therapies
for rare and orphan diseases*



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, cash runway, ongoing collaborations 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, 2013. 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

Investment Highlights

Strength of Clinical Programs and Breadth of Technology Platforms With Potential to Create Significant Value for Shareholders and Patients Living with LSDs

- Moving migalastat monotherapy toward approval for Fabry patients with amenable mutations
 - Positive 12- and 24-month data from first Phase 3 Study (O11)
 - Positive feedback from investigators, patients and Fabry community
 - Results from second Phase 3 Study (O12) on track for 3Q14
- Executing 3-in-3 strategy to advance 3 next-generation ERTs into clinic in next 3 years
 - Focus on developing better enzymes for Fabry, Pompe and MPS I
 - Next-generation Fabry ERT expected to enter clinic in 2H14
 - Next-generation Pompe ERT expected to enter clinic in 2015
- Strongly positioned and well-capitalized
 - \$71.6M cash (3/31/14)
 - Cash runway into 2H15

Fabry Disease Overview

Fatal Lysosomal Storage Disease with Significant Unmet Needs Despite Available Treatment Options

- Deficiency of lysosomal enzyme α -Galactosidase A (α -Gal A)
- GL-3 accumulation
- Heterogeneous (>800 known mutations)
- Symptoms include pain, gastrointestinal problems, angiokeratomas
- Leading causes of death are renal failure, cardiac failure, stroke
- Current ERT suboptimal



Currently Approved ERTs Do Not Fully Address Fabry Disease

“Over 40 years of working with patients with Fabry disease, participating in the development of enzyme replacement therapy, and as an early advocate of chaperone therapy, I believe there remains an unmet medical need among these patients. Study 011 has generated an impressive data set demonstrating that patients with amenable mutations respond to migalastat as a chaperone monotherapy.”

-Robert Desnick, M.D.

Dean for Genetics and Genomic Medicine, Professor and Chairman Emeritus, Genetics and Genomic Sciences at Icahn School of Medicine at Mount Sinai

Current Treatment Limitations

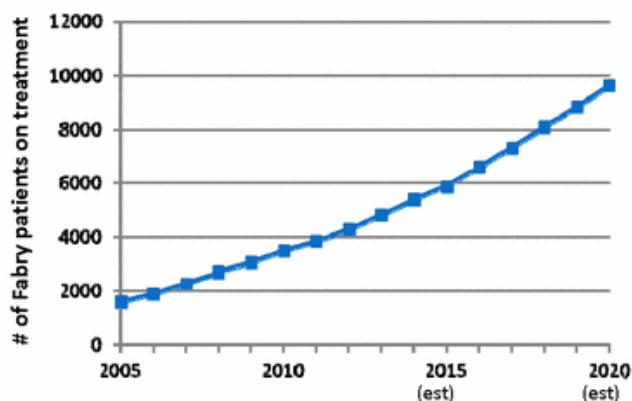
- Long term ERT does not prevent disease progression¹
- Burden of intravenous infusions
- Additional costs for hospital administration
- 50%-55% of patients in Fabrazyme clinical studies experienced at least 1 infusion-related reaction²
- IgG positive patients might have worse clinical outcome than IgG negative patients³

Fabry Commercial Opportunity

Market Size, Growth Rate and Limitations of Current Therapies Make Fabry a Compelling Market Opportunity

Global Fabry Market (\$993M in 2013)

12% = CAGR

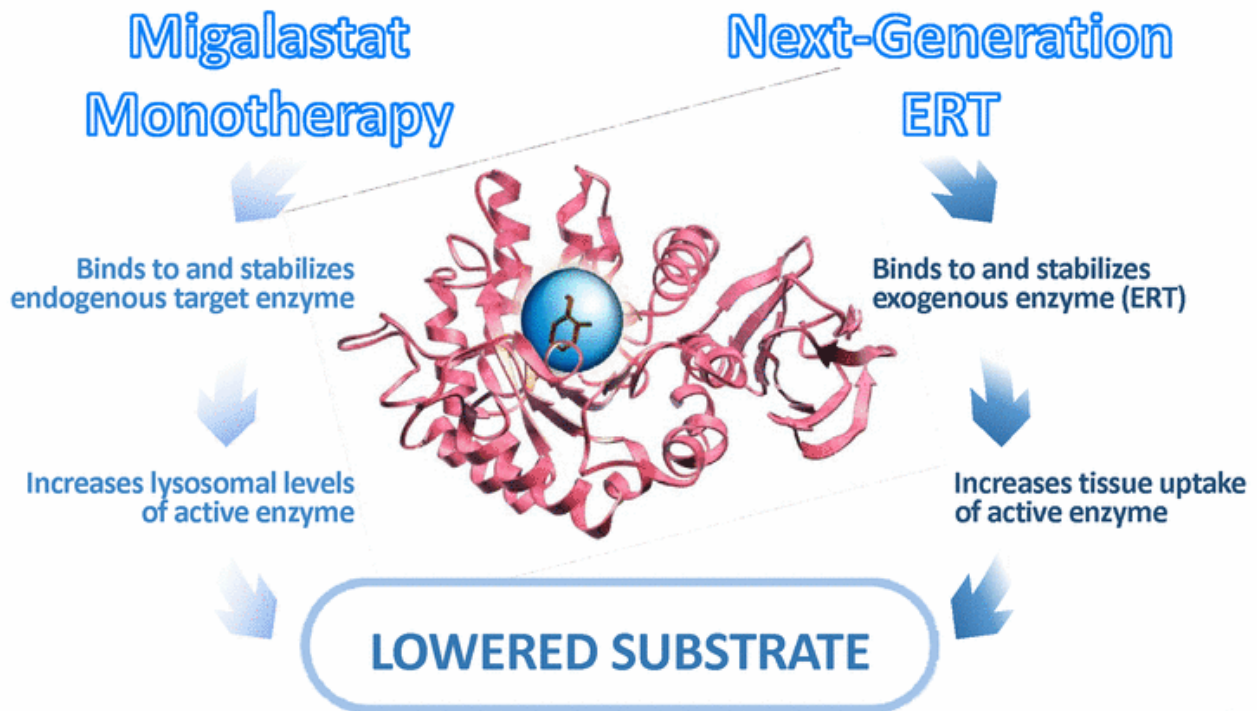


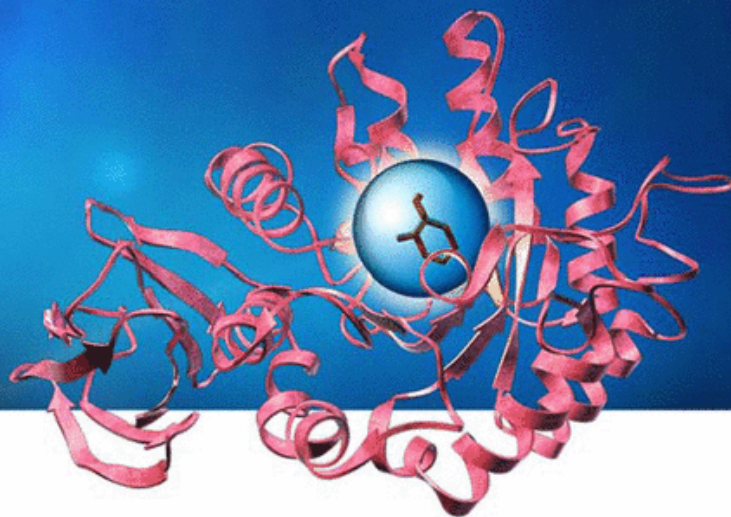
Sources: Analyst Reports, Company 10Ks, Market Research

- \$993M in FY13 global ERT sales (Fabrazyme and Replagal)
- 5-10K diagnosed WW (51% female/49% male¹)
- <50% of diagnosed patients are currently treated with ERT
- Newborn screening suggests prevalence of 1:3000 or higher^{2,3}

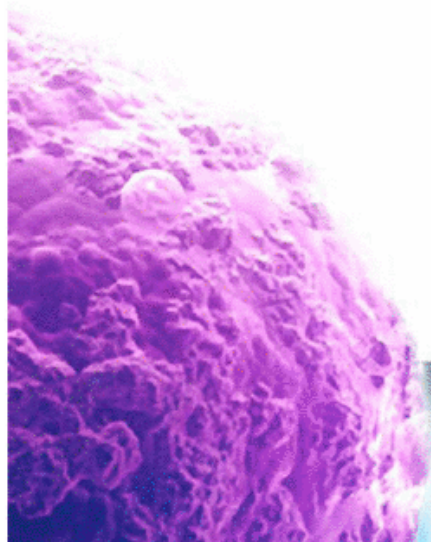
Amicus Fabry Franchise

Migalastat May Treat All Patients with Fabry as a Monotherapy or Combination with ERT





Migalastat Monotherapy

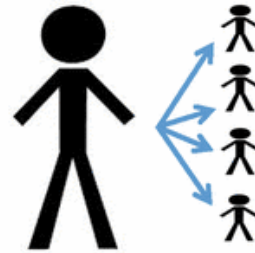


Amenable Mutations (~30%-50% of Fabry Patients)

Patients Identified by Screening Typically Yield 3-5 Affected Family Members¹

Newborn Screening Study	# Newborns Screened	# Confirmed Fabry Mutations	% Amenable
Burton, 2012, US	8,012	7 [1: ~1100]	TBD
Mechtler, 2011, Austria	34,736	9 [1: ~3,800]	100%
Hwu, 2009, Taiwan	171,977	75 [1: ~2300]	75%
Spada, 2006, Italy	37,104	12 [1: ~3100]	86%

Index Patient



Burton, LDN WORLD Symposium, 2012 Feb. Hwu *et al.*, Hum Mutation, 2009 Jun
Mechtler *et al.*, The Lancet, 2011 Dec. Spada *et al.*, Am J Human Genet., 2006 Jul

10 ¹Weidemann 2010

Global Registration Studies

Assembling Robust Dataset to Maximize Chances for Global Approvals of Migalastat Monotherapy for Fabry Patients with Amenable Mutations



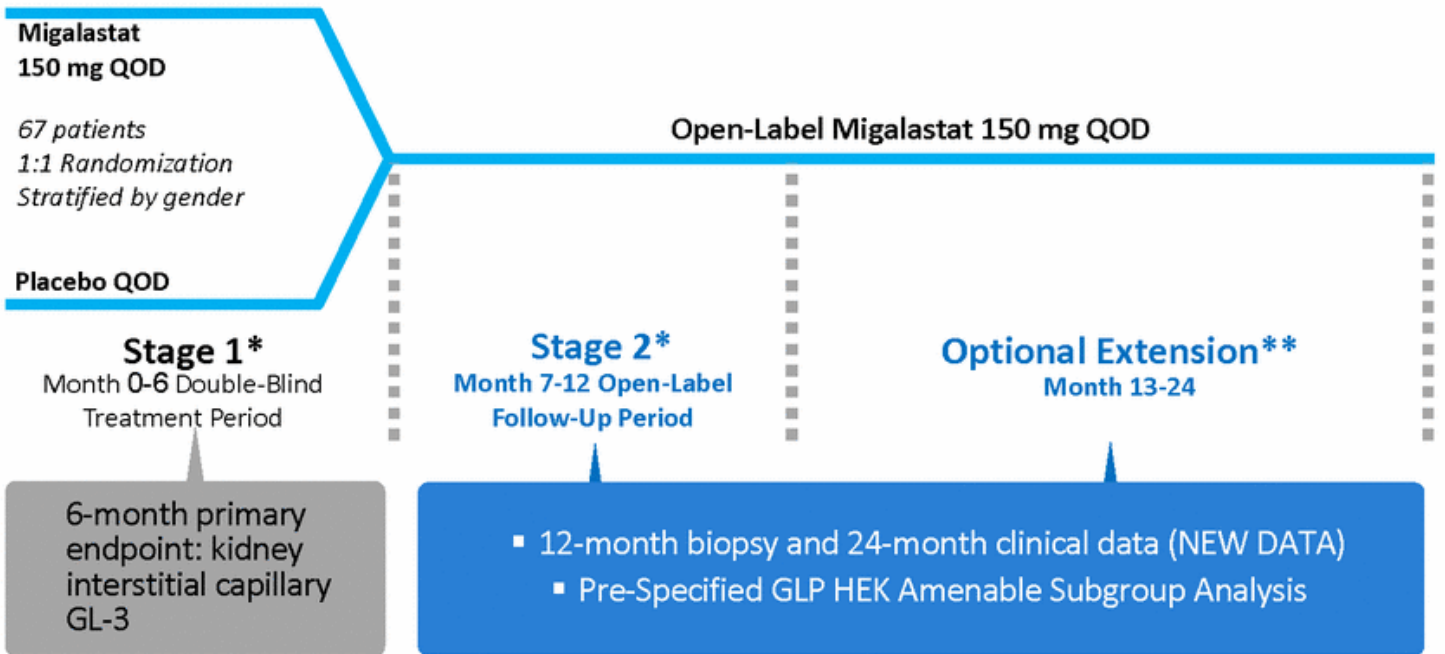
- Placebo-controlled (6 months)
- 67 patients naïve to ERT
- 6-month surrogate primary endpoint: kidney GL-3 (reported 4Q12)
- 12-month biopsy and 24-month clinical data (expected 2Q14)

THE ATTRACT STUDY

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

- ERT switch study
- 60 patients (1.5:1 randomization)
- 18-month clinical endpoint: kidney function (GFR)
- Data expected 3Q14

Phase 3 FACETS Study (Study 011)



Study 011 12- and 24-Month Data - Key Findings

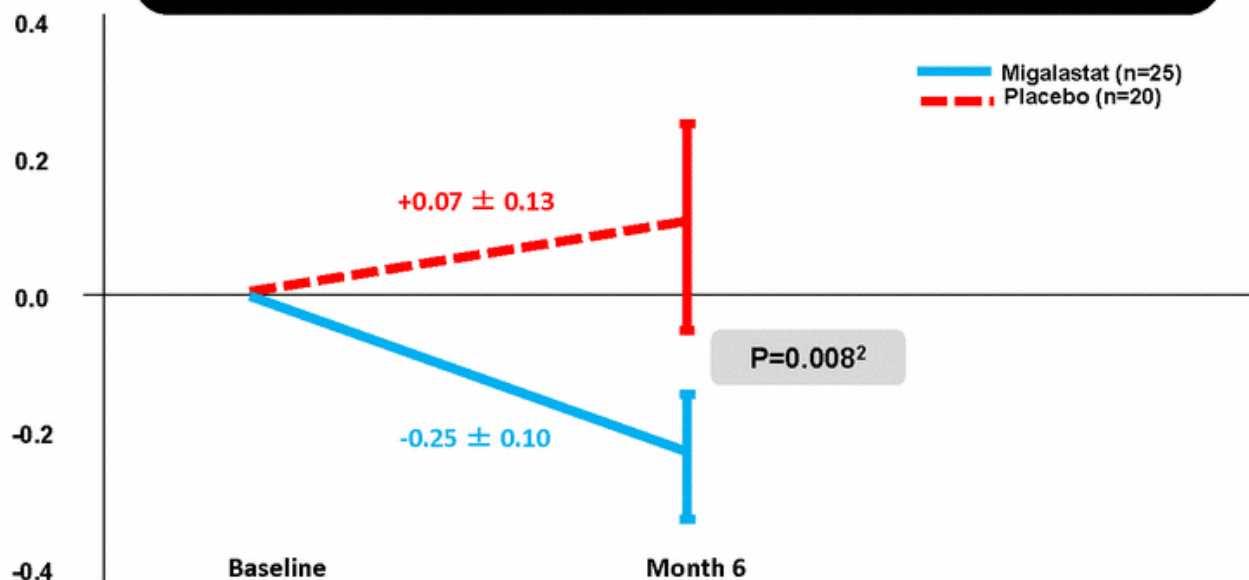
Migalastat Demonstrated Statistically Significant and Durable Substrate Reductions on 12-Month Pre-Specified Primary Analysis in Fabry Patients with Amenable Mutations

- Subjects who switched from placebo to migalastat after month 6 demonstrated a statistically significant reduction in kidney interstitial capillary GL-3 at month 12 ($p=0.013^*$)
- Subjects who remained on migalastat for 12 months demonstrated a durable reduction in kidney interstitial capillary GL-3
- Reduction in disease substrate also observed in plasma lyso-Gb3 in subjects who switched from placebo to migalastat ($p<0.0001^{**}$). Subjects who remained on migalastat demonstrated a durable reduction in lyso-Gb3
- Kidney function (estimated glomerular filtration rate (eGFR), iohexol mGFR) remained stable over 18-24 months
- Migalastat was generally safe and well-tolerated
- Of 41 subjects with GLP HEK amenable mutations who completed Study 011, 35 (85%) remain in voluntary extension study (Study 041)

6-Month Post-Hoc Analysis (Reported February 2014)

Statistically Significant Mean Change in Kidney Interstitial Capillary GL-3
Compared to Placebo (GLP HEK Amenable)*

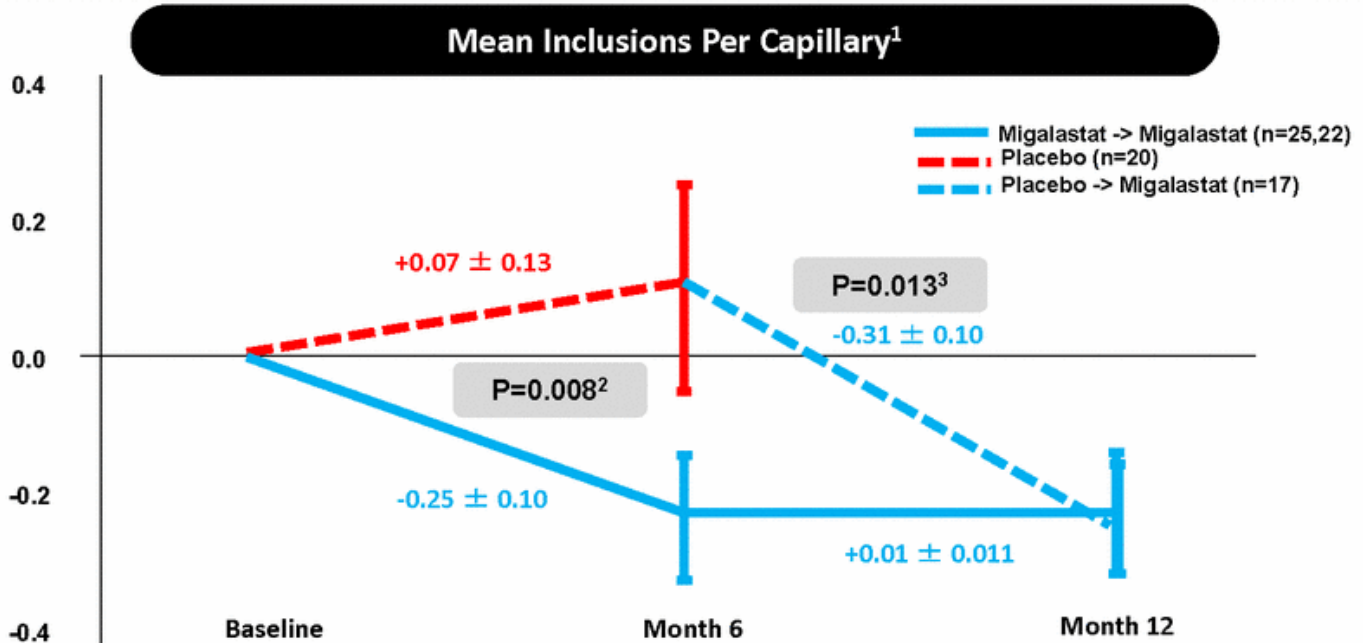
Mean Inclusions Per Capillary¹



¹All patients with evaluable paired biopsies and amenable GLA mutations in GLP-validated HEK assay – post hoc at month 6 and pre-specified at month 12 ²Data points are baseline corrected; represent mean ± standard error (SEM) change from baseline in the mean number of GL-3 inclusions per capillary after 6 months of treatment with migalastat or placebo. ²Analysis of covariance (ANCOVA) model with covariate adjustment for baseline value and factors for treatment group and treatment by baseline interaction. P-value corresponding to least-square mean difference between migalastat and placebo is displayed.

12-Month Pre-Specified Primary Analysis

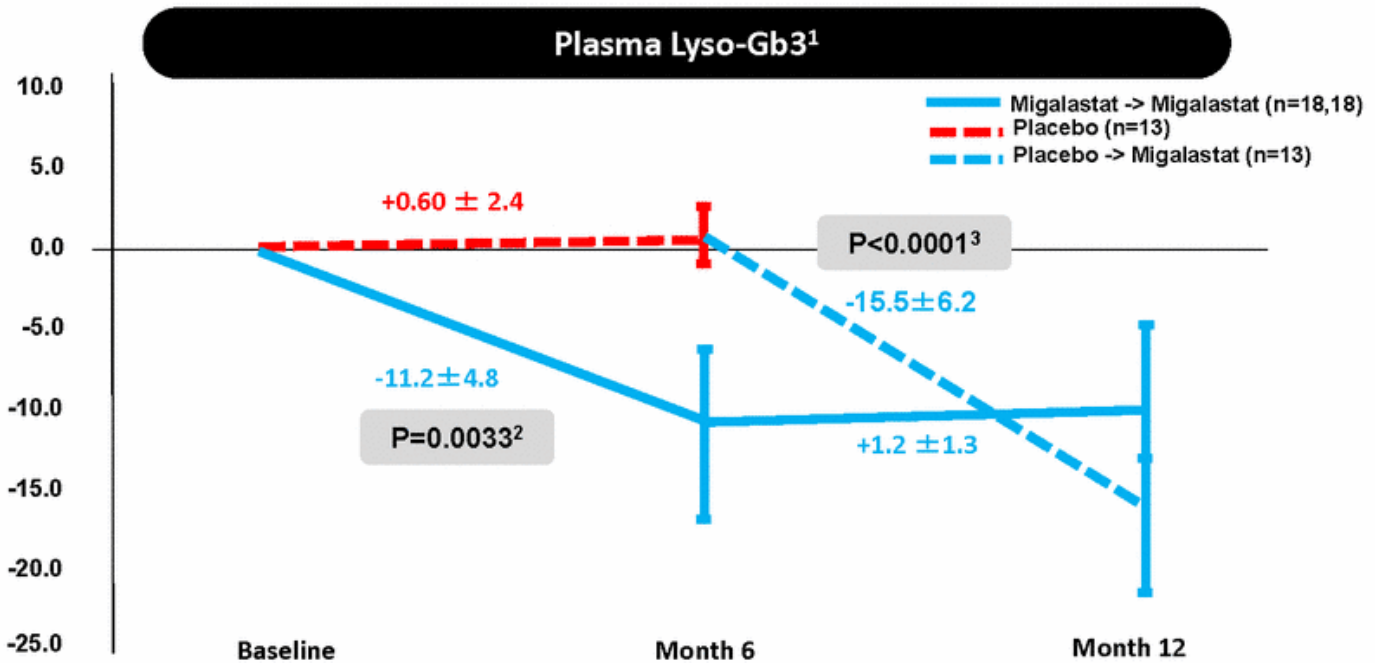
Statistically Significant Mean Change in Kidney Interstitial Capillary GL-3 in Patients Switching from Placebo to Migalastat HCl (GLP HEK Amenable)*



¹All patients with evaluable paired biopsies and amenable GLA mutations in GLP-validated HEK assay – post hoc at month 6 and pre-specified at month 12 ²Data points are baseline corrected; represent mean ± standard error (SEM) change from baseline in the mean number of GL-3 inclusions per capillary after 6 months of treatment with migalastat or placebo. ³Analysis of covariance (ANCOVA) model with covariate adjustment for baseline value and factors for treatment group and treatment by baseline interaction, P-value corresponding to least-square mean difference between migalastat and placebo is displayed. ⁴MMRM Pbo change M6 to M12.

Disease Substrate in Plasma (Plasma Lyso-Gb3)

Statistically Significant Reduction in Plasma Lyso-Gb3 at Month 6 and Month 12 Following Treatment with Migalastat (GLP HEK Amenable)*



*Patients with amenable GLA mutations in GLP-validated HEK assay ¹Baseline corrected. Error bars are SEM ²ANCOVA comparing migalastat to placebo in Stage 1 ³ANCOVA comparing change from month 6 to month 12 in subjects switching from placebo to migalastat

Kidney Function: Annualized Glomerular Filtration Rate (GFR)

GFR Remained Stable Over 18-24 Months (GLP HEK Amenable)*

Annualized GFR (ml/min/m²/yr) at Month 18 or 24¹

GFR Measure	N*	Mean	(SEM)
eGFR (CKD-EPI)	41	-0.30	(0.66)
eGFR (MDRD)	41	0.79	(1.03)
mGFR (iohexol)	37	-1.51	(1.33)

*Patients with amenable GLA mutations in GLP-validated HEK assay

¹24 Months of Data in Subjects Treated with Migalastat from Baseline, 18 Months of Data in Subjects Switched from Placebo to Migalastat After 6 Months

Safety Summary

Migalastat Generally Safe and Well Tolerated

Most Common Treatment Emergent Adverse Events ($\geq 10\%$ of Subjects)

Adverse event	Baseline to Month 6		Months 7-12		Open-Label Extension (Months 13-24)	
	Placebo* (n=33)	Migalastat (n=34)	Placebo-Migalastat* (n=30)	Migalastat (n=33)	Placebo-Migalastat* (n=28)	Migalastat (n=29)
Any Event	91%	91%	80%	79%	86%	83%
Headache	21%	35%			11%	10%
Fatigue	12%	12%				
Nausea	9%	12%				
Nasopharyngitis	6%	15%				
Paresthesia	12%	9%				
Procedural Pain			10%	12%		
Proteinuria					18%	14%
Bronchitis					11%	10%

18 *Subjects Received Placebo from Baseline to Month 6, Switched to Migalastat After Month 6

Differentiated Approach

Oral Agent with Similar Benefit to ERT may be Important Option for Fabry patients with Amenable Mutations

	ERT	Migalastat
Enzyme Level	Transient elevation (short half-life)	Consistent elevation
Delivery	Bi-weekly, multi-hour IV infusions	Oral, every-other-day dosing; Broad tissue distribution
Manufacturing	Recombinant protein	Small molecule
Addressable Patients	Not specific to genotype	30%-50% of Fabry patients, based on genotype

Patient Experience

97 Patients Today Take Migalastat as Only Therapy for Fabry Disease¹



Global Regulatory Strategy

- Totality of clinical data
- 8+ years of data in extension studies
- Complete data from Phase 3 Studies (011 and 012)

- Clear regulatory pathway
- Non-inferiority to ERT (Study 012)

Key Milestones

Timing	Milestone	
3Q09	Phase 3 Study 011 initiation	✓
3Q11	Phase 3 Study 012 initiation	✓
4Q12	Interim 6-month data from first Phase 3 Study (011)	✓
2Q13	FDA meeting (Type C)	✓
2Q14	12-month Study 011 data (kidney biopsies)	✓
2Q14	24-month Study 011 data (clinical outcomes)	✓
3Q14	18-month Study 012 data (kidney function)	

Advanced Product Pipeline



INDICATION	PRE-CLINICAL	PHASE 1	PHASE 2	PHASE 3
Fabry Disease				
AT-B100 (JR-051) + Migalastat				
Pompe Disease				
AT-B200 (rhGAA) + Chaperone				
Mucopolysaccharidosis Type I				
Next-Generation ERT				
Fabry Disease				
Migalastat				
Parkinson's Disease				
Novel Small Molecules				

3 Next-Generation ERTs Expected to Enter Clinic in Next 3 Years

NEXT-GENERATION ERTs (co-formulation)

MONOTHERAPY

Current Financial Picture

Financial Position

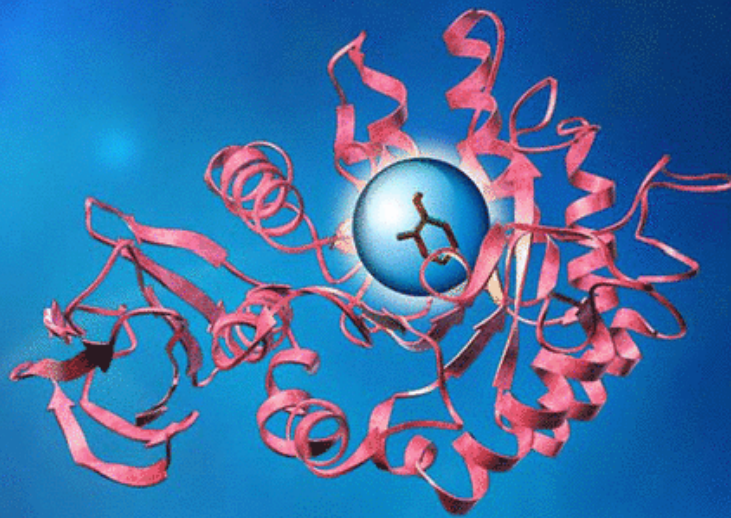
March 31 cash: \$71.6M

2014 net cash spend: \$54-59M

Cash runway: 2H15

Capitalization

Shares outstanding: 64,366,088



Corporate Overview

May 2014

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