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): **August 7, 2012**

AMICUS THERAPEUTICS, INC.
(Exact Name of Registrant as Specified in Its Charter)

Delaware	
(State or Other Jurisdiction of Incorporation)	
001-33497	**71-0869350**
(Commission File Number)	(IRS Employer Identification No.)
1 Cedar Brook Drive, Cranbury, NJ	**08512**
(Address of Principal Executive Offices)	(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:

o Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

o Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

o Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

o Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Item 2.02. Results of Operations and Financial Condition.

On August 7, 2012, Amicus Therapeutics, Inc. (the "Company") issued a press release announcing its financial results for the quarter ended June 30, 2012. A copy of this press release is attached hereto as Exhibit 99.1. The Company will also host a conference call and webcast on August 7, 2012 to discuss its second quarter results of operations. A copy of the conference call presentation materials is also attached hereto as Exhibit 99.2.

In accordance with General Instruction B.2. of Form 8-K, the information in this Current Report on Form 8-K and the Exhibits shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liability of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such a filing.

Item 9.01. Financial Statements and Exhibits.

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

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: August 7, 2012

By: /s/ Peter M. Macaluso
Peter M. Macaluso
Secretary

EXHIBIT INDEX

Exhibit No.	Description
99.1	Press Release dated August 7, 2012
99.2	August 7, 2012 Conference Call Presentation Materials

Amicus Therapeutics Announces
Second Quarter 2012 Financial Results & Corporate Updates

FDA to Consider Both Six-Month and 12-Month Efficacy and Safety Endpoints in Migalastat HCl Phase 3 Monotherapy Study 011 - Data Expected 4Q12

Chaperone-Enzyme Replacement Therapy (ERT) Combination Platform Advancing

Expects to End FY 2012 with at Least $90 Million Cash

CRANBURY, NJ, US, August 7, 2012 — Amicus Therapeutics (Nasdaq: FOLD), a biopharmaceutical company at the forefront of developing therapies for rare and orphan diseases, today announced financial results for the second quarter ended June 30, 2012. The Company also summarized recent and upcoming milestones and reiterated full-year 2012 operating expense guidance.

Key Highlights and Upcoming Milestones

- Primary six-month treatment period completed in Phase 3 Study 011 of migalastat HCl monotherapy for Fabry disease. 100% conversion of patients who completed the six-month primary treatment period into six-month open-label follow-up period (63 of 63 patients).
- Based on encouraging Type C guidance from the U.S. Food and Drug Administration (FDA) regarding planned new drug application (NDA) submission for migalastat HCl, Amicus and GSK intend to unblind Study 011 and release data in 4Q12 to allow for last patient to complete six-month follow-up period of the study and preserve the integrity and availability of the clinical data.
- Amicus and Glaxo Group Limited (GSK) expanded Fabry collaboration for development of migalastat HCl co-formulated with proprietary ERT - advancing preclinical studies
- Phase 2 Study 013 for Fabry disease ongoing in subjects receiving migalastat HCl 450 mg co-administered with enzyme replacement therapy (Fabrazyme® or Replagal®). Results expected to be presented at Fall 2012 scientific congress.
- Phase 2 Study 010 for Pompe disease demonstrated positive preliminary results in first two dose cohorts of AT2220 co-administered with ERT. Cohort 3 fully enrolled and reviewed by data safety monitoring board (DSMB), Cohort 4 now enrolling. Additional results to be presented at Fall 2012 scientific congress.

John F. Crowley, Chairman and Chief Executive Officer of Amicus stated, “During the second quarter we made excellent progress across all of our programs. In particular, we are very pleased with the interactions with FDA regarding our Phase 3 Fabry monotherapy Study 011. We believe that the FDA’s indication that it will consider efficacy as well as safety data at both six- and 12-month periods in this study further increases the likelihood of a successful outcome in this clinical program. The achievements in our Fabry program are also an added testament to the strong working collaboration between Amicus and GSK. We look forward to a continued busy and positive second half of 2012.”

Second Quarter 2012 Financial Highlights (3 Months Ended June 30, 2012)

- Total revenue of $10.6 million compared to $4.0 million in 2Q11 on higher research, collaboration and milestone revenue.
- Research revenue of $5.5 million compared to $2.4 million in 2Q11. Research revenue reflects reimbursement received from GlaxoSmithKline (GSK) for shared development costs for migalastat HCl for Fabry disease. Amicus and GSK funded 25% and 75%, respectively, of these global development costs in 2Q12.
- Collaboration and milestone revenue of $5.2 million compared to collaboration revenue of $1.7 million in 2Q11. This amount includes the recognized portion of the $33.2 million upfront cash payment received from GSK upon entering the Fabry collaboration; as well as a $3.5 million clinical development milestone payment earned during 2Q12.
- Total operating expenses of $20.0 million compared to $18.8 million in 2Q11 on higher research and development expenses.
- Net loss attributable to common stockholders of $9.3 million, or $0.20 per share, compared to a net loss of $12.6 million, or $0.37 per share, in 2Q11.

Financial Guidance

Cash, cash equivalents, and marketable securities totaled $95.8 million at June 30, 2012 compared to $108.2 million at March 31, 2012 and $56.0 million at December 31, 2011. Amicus expects to end 2012 with at least $90 million in cash, cash equivalents and marketable securities which is expected to fund its current operating plan beyond 2013. This projection includes the receipt of the $18.6 million equity investment from GSK and the $3.5 million cash milestone payment from GSK in the third quarter 2012, and quarterly reimbursement from GSK for shared development costs for migalastat HCl.

Amicus expects full-year 2012 operating expenses within the higher end of the previously disclosed guidance range of $37 million to $43 million, net of anticipated cost sharing under the expanded Fabry disease collaboration with GSK. Amicus and GSK are funding 25% and 75% of the development costs, respectively, for migalastat HCl monotherapy and co-administration for full-year 2012. During the second half of 2012, Amicus and GSK will be responsible

for 40% and 60% of the preclinical development costs, respectively, for the co-formulated chaperone-ERT product. Amicus will be responsible for all U.S. commercial activities for migalastat HCl upon approval, including pricing, marketing, patient access and reimbursement.

Program Updates

Chaperone Monotherapy and Chaperone-ERT Combinations for Fabry Disease

Migalastat HCl Monotherapy

Migalastat HCl monotherapy is in Phase 3 development for Fabry disease in patients with genetic mutations that are amenable to chaperone monotherapy. Amicus and GSK are currently conducting Phase 3 global registration studies (Study 011 and Study 012) of migalastat HCl monotherapy.

Study 011 is a randomized, placebo-controlled study with a six-month, double-blind primary treatment period and a six-month, open-label follow-up period. The primary endpoint is interstitial capillary globotriaosylceramide (GL-3) as measured in kidney biopsy. The six-month primary treatment period was completed in a total of 63 patients during the second quarter 2012. These patients received kidney biopsies at baseline and month six. All 63 of these patients are continuing in the six-month follow-up period, and all of these patients are expected to have 12-month kidney biopsies by year-end 2012.

Amicus and GSK have recently engaged in encouraging interactions with the FDA regarding the planned NDA for migalastat HCl. The agency indicated it would consider safety and efficacy data from both the six- and 12-month kidney biopsies to support conditional approval under subpart H. In order to preserve the integrity and availability of clinical data for the open-label follow-up period, Amicus and GSK have jointly determined that the unblinding and analysis of the data from the primary six-month treatment arm will not occur prior to the fourth quarter 2012. Both companies remain blinded to the results at this time.

Study 012 is a randomized, open-label, Phase 3 study targeting approximately 50 total patients (30 to switch to migalastat HCl and 20 to remain on ERT). Final enrollment continues to be expected by year-end 2012.

Patients also continue to receive migalastat HCl monotherapy in Phase 2 and Phase 3 extension studies. As of July 31, 2012, 38 of 40 patients who have completed the treatment and follow-up periods in Study 011 are currently enrolled in a Phase 3 extension study. An additional 17 subjects continue in the ongoing Phase 2 extension study and have been receiving migalastat HCl for up to six years.

Migalastat HCl Co-Administered with ERT

When co-administered with ERT, migalastat HCl is designed to bind to and stabilize the infused enzyme, independent of alpha-Gal A mutation type. An open-label Phase 2 study (Study 013) is currently underway to investigate the effects of a single oral dose of migalastat HCl (150 mg or 450 mg) co-administered prior to ERT (Fabrazyme or Replagal) in males with Fabry disease. Results are expected to be presented at a Fall 2012 scientific congress.

Migalastat HCl Co-Formulated with Preclinical Proprietary ERT

Under the expanded Fabry collaboration, Amicus and GSK in collaboration with JCR Pharmaceutical Co., Ltd. are developing migalastat HCl co-formulated with a proprietary recombinant human alpha-Gal A enzyme (JR-051). This co-formulated chaperone-ERT product has been evaluated in preclinical studies and has the potential to enter the clinic in 2013.

Chaperone-ERT Combinations Programs for Additional Lysosomal Storage Diseases

Amicus and GSK are co-developing all formulations of migalastat HCl for Fabry disease. Outside the GSK collaboration, Amicus owns exclusive rights to the rest of its pipeline and applications of its platform technology.

Preclinical chaperone-ERT co-administration studies in animal models of Fabry, Pompe and Gaucher have shown that a pharmacological chaperone can selectively bind to and stabilize the enzyme, prevent deactivation in the circulation, and increase uptake of active enzyme into key tissues of disease. In published studies in Fabry(1) and Pompe(2) animal models, chaperone-ERT co-administration has also led to greater substrate reduction compared to ERT alone.

Pompe Disease: AT2220-ERT Co-Administration

During the second quarter the Company announced positive preliminary results from the two lowest dose cohorts in a Phase 2 open-label study (Study 010) to investigate four ascending dose cohorts of the pharmacological chaperone AT2220 co-administered with ERT for Pompe disease. Additional results are anticipated at a Fall 2012 scientific congress.

In parallel with Study 010, Amicus is conducting *in vitro* studies using Antitope Ltd.'s *EpiScreen*™ assay to evaluate the immunogenicity of the Pompe ERT alglucosidase alfa, with and without AT2220. Results from these studies may help guide further investigation of the effects of AT2220 on immune response to ERT in future clinical studies.

Gaucher Disease: Preclinical Chaperone-ERT Combinations

In Gaucher disease, Amicus is continuing preclinical studies to evaluate two pharmacological chaperones, AT2101 (afegostat tartrate) and AT3375, in combination with ERT (beta-glucosidase). Both of these chaperones target the glucocerobrosidase (GCase) enzyme. Inherited genetic mutations in the GBA1 gene, which encodes for the GCase enzyme, are the cause of Gaucher disease.

Parkinson's Disease in Gaucher Carriers: Preclinical Chaperone Monotherapy

Over the last decade, GBA1 mutations have been identified as the most common genetic risk factor for Parkinson's. By targeting GCase in the brain, AT3375 could potentially treat Gaucher, Parkinson's disease in Gaucher carriers, and possibly the general Parkinson's population. By year-end 2012, Amicus expects to complete additional preclinical and IND-enabling studies of AT3375, which are supported in part by a grant from the Michael J. Fox Foundation.

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(1) Benjamin E, Khanna R, Schilling A, Flanagan J, Pellegrino L, Brignol N, Lun Y, Guillen D, Ranes B, Frascella M,Soska R, Feng J, Dungan L, Khanna R, Young B, Lockhart D, Valenzano K, **Molecular Therapy**: April 2012, Vol. 20, No. 4, pp. 717—726

(2) Khanna R, Flanagan JJ, Feng J, Soska R, Frascella M, et al. **PLoS ONE** (2012) 7(7): e40776. doi:10.1371/journal.pone.0040776

Conference Call and Webcast

Amicus Therapeutics will host a conference call and audio/visual webcast today, August 7, 2012 at 5:00 p.m. ET to review financial results and provide a corporate update. Interested participants and investors may access the conference call at 5:00 p.m. ET by dialing 877-303-5859 (U.S./Canada) or 678-224-7784 (international).

An audio/visual webcast can also be accessed via the Investors section of the Amicus Therapeutics corporate web site at http://www.amicusrx.com, and will be archived for 30 days. Web participants are encouraged to go to the Web site 15 minutes prior to the start of the call to register, download and install any necessary software.

The slide presentation for today's conference call and webcast is also available in the Investors section of the Amicus Therapeutics corporate web site at http://www.amicusrx.com.

A telephonic replay of the call will be available for seven days beginning at 8:00 p.m. ET today. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); participant code 16856524.

About Amicus Therapeutics

Amicus Therapeutics (Nasdaq:FOLD) is a biopharmaceutical company at the forefront of developing therapies for rare diseases. The Company is developing orally-administered, small molecule drugs called pharmacological chaperones, a novel, first-in-class approach to treating a broad range of human genetic diseases. Amicus' late-stage programs for lysosomal storage disorders include migalastat HCl monotherapy in Phase 3 for Fabry disease; migalastat HCl co-administered with enzyme replacement therapy (ERT) in Phase 2 for Fabry disease; and AT2220 co-administered with ERT in Phase 2 for Pompe disease.

About Fabry Disease

Fabry disease is an inherited lysosomal storage disease that is currently estimated to affect approximately 5,000 to 10,000 people worldwide. Fabry Disease is caused by deficiency of an enzyme called alpha-galactosidase A (alpha-Gal A). The role of alpha-Gal A within the body is to break down a complex lipid called globotriaosylceramide (GL-3). Reduced or absent levels of alpha-Gal A activity leads to the accumulation of GL-3 in the affected tissues, including the central nervous system, heart, kidneys, and skin. This accumulation of GL-3 is believed to cause the various symptoms of Fabry disease, including pain, kidney failure, and increased risk of heart disorders and stroke.

About Pompe Disease

Pompe disease is a lysosomal storage disease characterized by progressive skeletal muscle weakness and respiratory insufficiency. It is caused by a deficiency in lysosomal alpha-glucosidase (GAA) activity, which leads to accumulation of glycogen in tissues affected by the disease (primarily muscle). Pompe disease affects an estimated 5,000 to 10,000 individuals worldwide and is clinically heterogeneous in the age of onset, the extent of organ involvement, and the rate of progression.

Forward-Looking Statements

This press release contains, and the accompanying conference call will contain, "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating 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, and the projected cash position for the Company. Words such as, but not limited to, "look forward to," "believe," "expect," "anticipate," "estimate," "intend," "plan," "targets," "likely," "will," "would," "should" and "could," and similar expressions or words identify forward-looking statements. Such forward-looking statements are based upon current expectations that involve risks, changes in circumstances, assumptions and uncertainties. The inclusion of forward-looking statements should not be regarded as a representation by Amicus that any of its plans will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong. They can be affected by inaccurate assumptions Amicus might make or by known or unknown risks and uncertainties. For example, with respect to statements regarding the goals, progress, timing and outcomes of discussions with regulatory authorities and the potential goals, progress, timing and results of preclinical studies and clinical trials, actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in the business of Amicus, including, without limitation: the potential that results of clinical or pre-clinical studies indicate that the product candidates are unsafe or ineffective; the potential that it may be difficult to enroll patients in our clinical trials; the potential that regulatory authorities may not grant or may delay approval for our product candidates; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; the potential that we will need additional funding to complete all of our studies and, our dependence on third parties in the conduct of our clinical studies. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. With respect to statements regarding projections of the Company's cash position, actual results may differ based on market factors and the Company's ability to execute its operational and budget plans. In addition, all forward looking statements are subject to other risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2011. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. 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. This caution is made under the safe harbor provisions of Section 21E of the Private Securities Litigation Reform Act of 1995.

CONTACTS:

Investors/Media:

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(609) 662-5044

Table 1
Amicus Therapeutics, Inc.
(a development stage company)
Consolidated Statements of Operations
(Unaudited)
(In thousands, except share and per share amounts)

	Three Months Ended June 30,		Six Months Ended June 30,		Period from February 4, 2002 (inception) to June 30, 2012
	2011	2012	2011	2012	
Revenue:					
Research revenue	$ 2,380	$ 5,477	$ 6,686	$ 11,591	$ 57,493
Collaboration and milestone revenue	1,660	5,160	3,320	6,820	64,382
Total revenue	4,040	10,637	10,006	18,411	121,875
Operating Expenses:					
Research and development	11,618	13,723	22,743	27,727	293,347
General and administrative	6,720	5,819	11,122	9,914	123,163
Restructuring charges	—	—	—	—	1,522
Impairment of leasehold improvements	—	—	—	—	1,030
Depreciation and amortization	426	442	864	862	10,925
In-process research and development	—	—	—	—	418
Total operating expenses	18,764	19,984	34,729	38,503	430,405
Loss from operations	(14,724)	(9,347)	(24,723)	(20,092)	(308,530)
Other income (expenses):					
Interest income	46	116	105	143	14,216
Interest expense	(41)	(15)	(89)	(58)	(2,391)
Change in fair value of warrant liability	2,078	(118)	(1,354)	(2,494)	(1,594)
Other income	—	21	70	21	252
Loss before tax benefit	(12,641)	(9,343)	(25,991)	(22,480)	(298,047)
Benefit from income taxes	—	—	—	—	5,463
Net loss	(12,641)	(9,343)	(25,991)	(22,480)	(292,584)
Deemed dividend	—	—	—	—	(19,424)
Preferred stock accretion	—	—	—	—	(802)
Net loss attributable to common stockholders	$ (12,641)	$ (9,343)	$ (25,991)	$ (22,480)	$ (312,810)
Net loss attributable to common stockholders per common share — basic and diluted	$ (0.37)	$ (0.20)	$ (0.75)	$ (0.53)	
Weighted-average common shares outstanding — basic and diluted	34,530,693	46,870,067	34,514,947	42,103,642	

Exhibit 99.2

Amicus Therapeutics

2Q12 Financial Results Conference Call & Webcast

At the Forefront of Therapies for Rare and Orphan Diseases™

August 7, 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.

Agenda

Corporate Highlights	John F. Crowley, Chairman & CEO
Fabry Program – Phase 3 Updates	John F. Crowley, Chairman & CEO
Chaperone-ERT Program Highlights	Bradley L. Campbell, Chief Business Officer
2Q12 Financial Results & FY12 Guidance	Chip Baird, Chief Financial Officer
Upcoming Milestones/Concluding Remarks	John F. Crowley, Chairman & CEO
Q&A	John F. Crowley, Chairman & CEO Bradley L. Campbell, Chief Business Officer Chip Baird, Chief Financial Officer David J. Lockhart, PhD, Chief Scientific Officer

2Q12 Key Highlights

Phase 3 Fabry Program Migalastat HCl Monotherapy	✓ Study 011: primary 6-month treatment period completed – all patients to complete 6-month treatment extension in 4Q12 ✓ Encouraging feedback from FDA on NDA submission with 6- and 12-month data; unblinding in 4Q12
Pompe Program AT2220 Co-Administered with ERT	✓ Positive preliminary results in Phase 2 Study 010 (Cohorts 1-2)
Strong Financial Position	✓ $95.8M cash position on June 30, 2012 ✓ Flexibility to advance Fabry programs with GSK, rest of pipeline independently ✓ Full U.S. economics for all Fabry products upon approval
Entering Transformational 2H12	✓ Expanded Fabry collaboration with GSK in July 2012 ✓ Transitioning into fully-integrated biopharmaceutical company within U.S. ✓ Advancing technology platform along continuum of innovation

Amicus & GSK Rare Diseases Expanded Alliance

Maximizes Value Proposition to Deliver New Benefits to Fabry Patients

GSK increasing investment in Amicus and Fabry development
Amicus transforming into commercial-stage U.S. biopharmaceutical company

- **Joint development of all Fabry products**
  - Migalastat monotherapy in Phase 3: Study 011 results anticipated 4Q12
  - Migalastat HCl co-administered with ERT in Phase 2: positive preliminary results
  - Migalastat HCl co-formulated with proprietary recombinant human α-Gal A enzyme (JR-051) developed by JCR/GSK
- **U.S. commercial rights to all formulations of migalastat HCl for Fabry disease**
  - Amicus-led U.S. marketing, pricing, access/reimbursement
  - Leverages strength in patient advocacy and medical affairs
- **GSK investing in Amicus and Fabry development programs**
  - $18.6M equity investment (19.9% ownership)
  - Funding development costs (75% in 2012, 60% in 2013 and beyond)
- **Further validation of Amicus' platform along continuum of innovation**
- **Enhances Amicus significance as strategic collaboration for GSK Rare Diseases**

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
- 150 mg migalastat, every-other-day (QOD)
- Placebo-controlled
- 67 patients
- 6-month surrogate endpoint – kidney GL-3
- Eligible for accelerated approval
- 6-month primary treatment period complete
- Data expected 4Q12

STUDY 012

- Global Registration Study
- 150 mg migalastat QOD
- Switch from ERT
- 50 patients
- 18-month clinical endpoint – kidney function
- Full enrollment targeted by YE12

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 (key biomarker) ▪ 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
Phase 3 Observations Study 011	▪ Low dropout rate ▪ High conversion to extension study

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

Study 011

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

Low drop out rate and high conversion to extension studies

63 completed 6-mo. double-blind treatment period (~6% drop-out 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

Continuum of Innovation

Multiple Paths Forward for Chaperone-ERT Combinations

Envisioning Constant New Product Advances Unique to Each LSD

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

Phase 2 Chaperone-ERT Co-Administration Studies

Positive Preliminary Results in Different LSDs with 2 Different Chaperones

FABRY STUDY 013

- Drug-drug interaction study
- Migalastat HCl 150 mg or 450 mg, prior to ERT (Fabrazyme® or Replagal®)
- **Positive preliminary results** (migalastat HCl 150 mg + Fabrazyme)
- Plasma PK & PD (skin biopsies)
- **3 cohorts completed** (migalastat HCl 150 mg + Fabrazyme; migalastat HCl 150 mg + Replagal; migalastat HCl 450 mg + Fabrazyme)
- Enrollment ongoing in final cohort (migalastat HCl 450 mg + Replagal)
- Additional results expected at Fall 2012 scientific congress

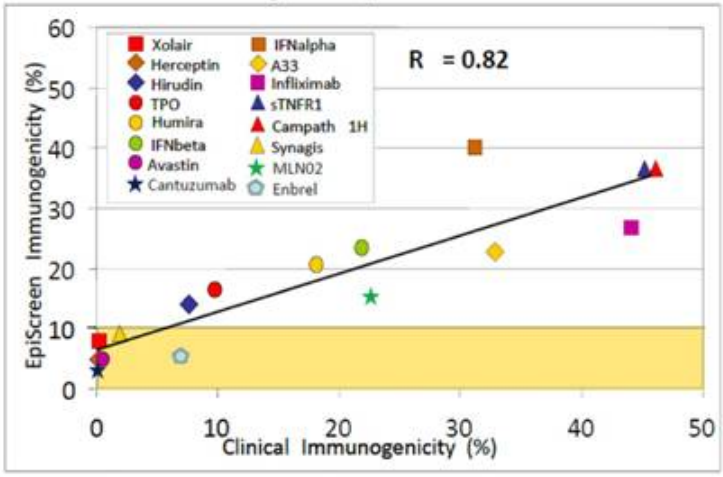
POMPE STUDY 010

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

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 PBMCs from 50 healthy volunteers (represent 90% of HLA haplotypes in general population)
- Evaluating T-cell response in patient-derived PBMCs from Study 010 (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

Chaperone-ERT Co-Formulation:

Strategic Relationship Leverages JCR's Biological Expertise

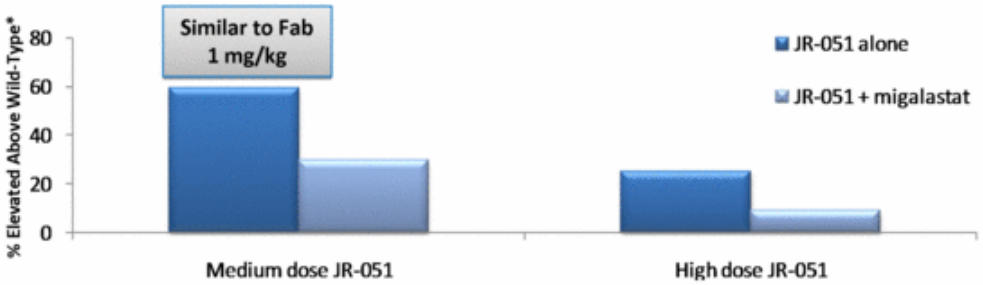
Formulation and Preclinical Studies Conducted Over 16+ Months

Chaperone-ERT Co-Formulation for Fabry Disease

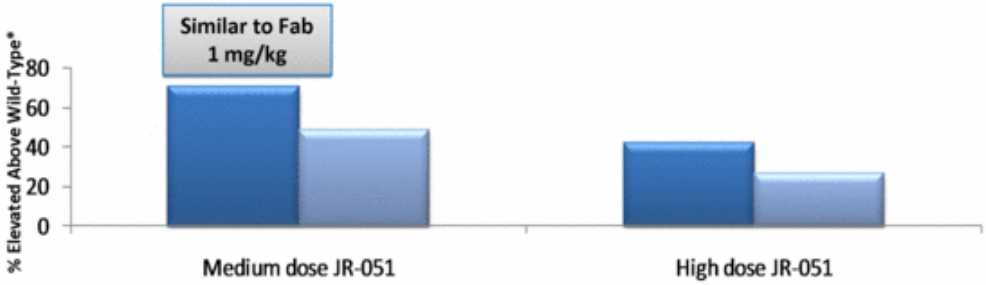
JR-051 +/- Migalastat HCl in GLA *Knock-Out Mice (IV Administration)*

Co-formulation with Migalastat Results in Significantly Greater GL-3 Reduction Than What Has Been Previously Observed

Heart GL-3



Kidney GL-3



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

2Q12 Financial Results

Consolidated Statement of Operations (Unaudited)
In thousands, except share and per share amounts

	3 Months Ended June 30, 2012	2011
Revenue:		
Research Revenue	$ 5,477	$ 2,380
Collaboration and milestone revenue	5,160	1,660
Total revenue	10,637	4,040
Operating Expenses:		
Research and development	13,723	11,618
General and administrative	5,819	6,720
Depreciation and amortization	442	426
Total operating expenses	19,984	18,764
Loss from operations	(9,347)	(14,724)
Non-operating income (expenses)	(4)	2,083
Net loss / net loss attributable to common stockholders	$ (9,343)	$ (12,641)
Net loss per common share – basic and diluted	$ (0.20)	$ (0.37)
Weighted-average common shares outstanding - basic and diluted	46,870,067	34,530,693

FY12 Financial Guidance

- **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

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 Study 011 – 6-month primary treatment complete	Q3
	▪ Phase 3 Study 011 – 6-month treatment extension complete	Q4
	▪ Phase 3 Study 011 Data	Q4
	▪ Phase 2 Study 013 Data	Fall 2012
	▪ Phase 3 Study 012 Complete 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 Co-Administration Data	Fall 2012
	▪ Final Phase 2 Study 010 Co-Administration Data	Q4
Parkinson's	▪ Completion of additional AT3375 IND-Enabling Studies	Q4

Q&A

John F. Crowley, Chairman & CEO

Bradley L. Campbell, Chief Business Officer

Chip Baird, Chief Financial Officer

David J. Lockhart, PhD, Chief Scientific Officer