

**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, 2013**

**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))

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**Item 2.02. Results of Operations and Financial Condition.**

On May 9, 2013, Amicus Therapeutics, Inc. (the "Company") issued a press release announcing its financial results for the quarter ended March 31, 2013. A copy of this press release is attached hereto as Exhibit 99.1. The Company will also host a conference call and webcast on May 9, 2013 to discuss its first 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.

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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.

Date: May 9, 2013

By: /s/ Peter M. Macaluso

Peter M. Macaluso

Secretary

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**EXHIBIT INDEX**

<u>Exhibit No.</u>	<u>Description</u>
99.1	Press Release dated May 9, 2013
99.2	May 9, 2013 Conference Call Presentation Materials

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## Amicus Therapeutics Announces First Quarter 2013 Financial Results and Corporate Updates

### 12-Month Results from Ongoing Phase 3 Fabry Disease Monotherapy Study Anticipated 3Q13

#### Phase 2b Pompe Co-Administration Study on Track to Begin 3Q13

CRANBURY, NJ, US, May 9, 2013 — Amicus Therapeutics (Nasdaq: FOLD), a biopharmaceutical company at the forefront of therapies for rare and orphan diseases, today announced financial results for the first quarter ended March 31, 2013. The Company also summarized recent and upcoming milestones and reiterated full-year 2013 operating expense guidance.

#### Key Highlights and Upcoming Milestones:

- 12-month results from first ongoing Phase 3 Fabry monotherapy study (Study 011) anticipated 3Q13. FDA will consider entirety of 6- and 12-month data for potential U.S. approval of migalastat HCl monotherapy.
- Phase 2b repeat-dose clinical study of AT2220 (duvoglustat HCl) co-administered with ERT (Myozyme®/Lumizyme®) for Pompe disease expected to begin 3Q13.
- IND submission for Fabry chaperone-ERT co-formulated product planned by year-end 2013 for entry into clinic in early 2014.
- Next-generation ERTs for Pompe disease and other LSDs advancing in preclinical studies.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics stated, “Amicus had a productive first quarter that was largely focused on the execution of clinical and regulatory activities for migalastat HCl monotherapy for Fabry disease, our upcoming clinical studies in Fabry and Pompe, and the build out of our CHART platform. A Type C meeting with the FDA will take place during the second quarter to discuss the statistical analysis plan for Study 011 while the 12-month data are still blinded. We remain on track to unblind and announce these 12-month results in the third quarter. We are also finalizing the design of our repeat-dose Phase 2b co-administration study for Pompe disease, which we expect to begin in the third quarter. Our strong financial position will continue to support the advancement of these programs as we approach several key milestones throughout the remainder of 2013.”

#### Financial Highlights for First Quarter Ended March 31, 2013

- Cash, cash equivalents, and marketable securities totaled \$84.8 million at March 31, 2013 compared to \$99.1 million at December 31, 2012.
- Cash reimbursements received from GlaxoSmithKline (GSK) for shared development of migalastat HCl totaled \$3.2 million compared to \$5.0 million in the first quarter 2012.
- No revenue was reported due to a change in revenue recognition accounting under the expanded GSK collaboration. Total revenue of \$7.8 million was recognized in the first quarter 2012.
- Total operating expenses decreased to \$17.3 million from \$18.5 million in the first quarter 2012 due to lower research and development expenses as well as a decrease in personnel-related costs.
- Net loss was \$17.5 million, or \$0.35 per share, compared to a net loss of \$13.1 million, or \$0.35 per share, for the first quarter 2012.

#### 2013 Financial Guidance

As previously announced, Amicus expects full-year 2013 net cash spend to total between \$52 million and \$58 million, including cash reimbursements received from GSK. Amicus and GSK are responsible for 40% and 60% of global development costs for migalastat HCl, respectively, in 2013 and beyond. The Company continues to project that the current cash position and anticipated Fabry program reimbursements from GSK are sufficient to fund operations into the second half of 2014.

#### Program Updates

##### Migalastat HCl for Fabry Disease

Amicus in collaboration with GSK is developing the investigational pharmacological chaperone migalastat HCl for the treatment of Fabry disease. Amicus has commercial rights to all Fabry products in the United States and GSK has commercial rights to all of these products in the rest of world.

##### Migalastat HCl Monotherapy

Migalastat HCl monotherapy (150 mg, every-other-day) is being investigated in two ongoing randomized Phase 3 studies for Fabry disease (Study 011 and Study 012) in patients with genetic mutations identified as amenable to this pharmacological chaperone in a cell-based assay.

- Study 011 is comparing migalastat HCl to placebo to support a potential U.S. marketing application as well as global registration. The FDA has indicated that it will consider the entirety of the efficacy and safety data from the 6-month double-blind treatment period (Stage 1) and 6-month follow-up period (Stage 2) of Study 011. The Stage 1 results have been reported while Stage 2 data are anticipated in the third quarter of 2013. A Type C meeting with the FDA has been scheduled during the second quarter to review the statistical analysis plan. Both Amicus and GSK will remain blinded to the Stage 2 results until after this Type C meeting has occurred. Following the announcement of 12-month results, an additional meeting is anticipated with the FDA to discuss a U.S. marketing application for migalastat HCl monotherapy.
- Study 012 is comparing open-label migalastat HCl to current standard of care ERTs (Fabrazyme and Replagal) to support global registration. A total of 60 patients were randomized 1.5:1 to switch from ERT to migalastat HCl or remain on ERT. Data are anticipated in the second half of 2014 on the primary outcome measure, which is renal function assessed by iohexol Glomerular Filtration Rate (GFR) at 18 months.

## ***Migalastat HCl in Combination with ERT***

In combination with ERT, migalastat HCl is designed to bind to and stabilize infused alpha-Gal A enzyme, independent of a patient's genetic mutation. Therefore Amicus believes this approach has the potential to benefit any patient with Fabry disease. Amicus and GSK, in collaboration with JCR Pharmaceutical Co. Ltd, are currently developing intravenous migalastat HCl co-formulated with a proprietary recombinant human alpha-Gal A enzyme (JCR's JR-051). An IND submission is planned by year-end 2013 for potential entry into the clinic in early 2014. The results from an open-label Phase 2 study (Study 013) of migalastat HCl co-administered with ERT (Fabrazyme® or Replagal®) have built confidence around the concept that migalastat HCl in combination with ERT can increase levels of active enzyme in plasma and tissues and support the rationale for the first repeat-dose clinical study with the chaperone ERT co-formulated product.

## **CHART Programs for Pompe Disease**

Outside the collaboration agreement with GSK, Amicus owns exclusive rights to the rest of its pipeline and applications of its CHART platform technology. The current CHART programs for Pompe disease are investigating the pharmacological chaperone AT2220 in combination with human recombinant GAA (rhGAA) enzymes. These next-generation therapies have the potential to increase enzyme activity in muscle and other disease-relevant tissues, improve glycogen reduction, and mitigate immunogenicity compared to rhGAA alone.

- **AT2220-IV Co-Administered with Marketed ERTs:** Amicus plans to initiate a repeat-dose Phase 2b clinical study in the third quarter of 2013 to evaluate a novel intravenous formulation of AT2220 (AT2220-IV) co-administered with currently marketed rhGAA (Myozyme/Lumizyme) in Pompe patients. To build upon positive results from a Phase 2 co-administration study (Study 010), Amicus plans to evaluate AT2220-IV co-administered with Myozyme/Lumizyme every 2 weeks to characterize safety, PK, and anti-rhGAA antibody titers. The study is expected to include both treatment-naïve and ERT-experienced individuals at leading Pompe centers.
- **Next-Generation ERT (AT2220 Co-Formulated with a Proprietary rhGAA Enzyme):** Amicus is also developing a proprietary rhGAA enzyme co-formulated with AT2220 as a next-generation therapy for Pompe disease. Amicus believes it has the potential to improve the properties of the rhGAA enzyme itself while incorporating AT2220 as a small molecule stabilizer to increase exposure and tissue uptake of active enzyme, reduce immunogenicity and potentially enable novel routes of delivery such as subcutaneous administration.

## **Conference Call and Webcast**

Amicus Therapeutics will host a conference call and audio/visual webcast today, May 9, 2013 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.

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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 64638686.

## **About Amicus Therapeutics**

Amicus Therapeutics (Nasdaq:FOLD) is a biopharmaceutical company at the forefront of therapies for rare and orphan diseases. The Company is developing novel, first-in-class treatments for a broad range of human genetic diseases, with a focus on delivering new benefits to individuals with lysosomal storage diseases. Amicus' lead programs include the small molecule pharmacological chaperones migalastat HCl as a monotherapy and in combination with enzyme replacement therapy (ERT) for Fabry disease; and AT2220 (duvoglustat HCl) in combination with ERT for Pompe disease.

## **About Chaperone-Advanced Replacement Therapy (CHART)**

The Chaperone-Advanced Replacement Therapy (CHART™) platform combines unique pharmacological chaperones with enzyme replacement therapies (ERTs) for lysosomal storage diseases (LSDs). In a chaperone-advanced replacement therapy, a unique pharmacological chaperone is designed to bind to and stabilize a specific therapeutic enzyme in its properly folded and active form. This proposed CHART mechanism may allow for enhanced tissue uptake of active enzyme, greater lysosomal activity, more reduction of substrate, and lower immunogenicity compared to ERT alone. Improvements in enzyme stability may also enable more convenient delivery of next-generation therapies. Amicus is leveraging the CHART platform to develop pharmacological chaperones co-administered with currently marketed ERTs as well as proprietary next-generation therapies that consist of lysosomal enzymes co-formulated with pharmacological chaperones.

## **About Migalastat HCl for Fabry Disease**

Migalastat HCl is an investigational pharmacological chaperone in development as a monotherapy and in combination with enzyme replacement therapy (ERT) for the treatment of Fabry disease. As a monotherapy, migalastat HCl is designed to bind to and stabilize, or "chaperone" a patient's own alpha-galactosidase A (alpha-Gal A) enzyme in those with genetic mutations that are amenable to this chaperone in a cell-based assay. For patients currently receiving ERT for Fabry disease, migalastat HCl in combination with ERT may improve ERT outcomes by keeping the infused alpha-Gal A enzyme in its properly folded and active form.

Fabry disease is an inherited lysosomal storage disorder caused by deficiency of the alpha-Gal A enzyme. The role of alpha-Gal A within the body is to break down specific lipids in lysosomes, including globotriaosylceramide (GL-3, also known as Gb3). Lipids that can be degraded by the action of alpha-Gal A are called "substrates" of the enzyme. Reduced or absent levels of alpha-Gal A activity leads to the accumulation of GL-3 in the affected tissues, including the kidneys, heart, central nervous system, and skin. This accumulation of GL-3 is believed to cause the various manifestations of Fabry disease, including pain, kidney failure, and increased risk of heart attack and stroke. It is currently estimated that Fabry disease affects approximately 5,000 to 10,000 people

worldwide. However, several literature reports suggest that Fabry disease may be significantly under-diagnosed, and the prevalence of the disease may be much higher.

### **About CHART for Pompe Disease**

In chaperone-advanced replacement therapy programs for Pompe disease, the small molecule pharmacological chaperone AT2220 is designed to bind to and stabilize human recombinant GAA (rhGAA) enzyme. Amicus is developing AT2220 co-administered with currently marketed ERTs (rhGAA enzymes, Myozyme/Lumizyme) in parallel with the development of a next-generation ERT (AT2220 co-formulated with a proprietary rhGAA enzyme). Positive results from a Phase 2 study (Study 010) established human proof-of-concept that oral administration of AT2220 just prior to infusing Myozyme/Lumizyme increases enzyme activity in muscle compared to ERT alone. In preclinical studies of AT2220 co-administered and co-formulated with Myozyme/Lumizyme, greater enzyme uptake in disease-relevant tissues led to greater glycogen reduction compared to Myozyme/Lumizyme alone. These chaperone-advanced replacement therapies also have the potential to mitigate Pompe ERT-related immunogenicity because properly folded proteins are generally less prone to aggregation and less immunogenic.

Pompe disease is a lysosomal storage disease characterized by progressive skeletal muscle weakness and respiratory insufficiency. It is caused by a deficiency in GAA activity, which leads to accumulation of glycogen in the heart, muscle, and other tissues affected by the disease. 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,” “potential,” “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, 2012. 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:

Sara Pellegrino  
 spellegrino@amicusrx.com  
 (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 March 31,		Period from February 4, 2002 (inception) To March 31, 2013
	2012	2013	
Revenue:			
Research revenue	\$ 6,114	\$ —	\$ 57,493
Collaboration and milestone revenue	1,660	—	64,382
Total revenue	7,774	—	121,875
Operating Expenses:			
Research and development	14,004	11,989	327,882

General and administrative	4,095	4,823	137,436
Restructuring charges	—	—	1,522
Impairment of leasehold improvements	—	—	1,030
Depreciation and amortization	420	439	12,207
In-process research and development	—	—	418
Total operating expenses	<u>18,519</u>	<u>17,251</u>	<u>480,495</u>
Loss from operations	(10,745)	(17,251)	(358,620)
Other income (expenses):			
Interest income	27	65	14,454
Interest expense	(43)	(10)	(2,432)
Change in fair value of warrant liability	(2,376)	(262)	1,291
Other income	—	—	252
Loss before tax benefit	(13,137)	(17,458)	(345,055)
Benefit from income taxes	—	—	8,708
Net loss	(13,137)	(17,458)	(336,347)
Deemed dividend	—	—	(19,424)
Preferred stock accretion	—	—	(802)
Net loss attributable to common stockholders	<u>\$ (13,137)</u>	<u>\$ (17,458)</u>	<u>\$ (356,573)</u>
Net loss attributable to common stockholders per common share — basic and diluted	<u>\$ (0.35)</u>	<u>\$ (0.35)</u>	
Weighted-average common shares outstanding — basic and diluted	<u>37,887,520</u>	<u>49,621,188</u>	

Table 2

**Amicus Therapeutics, Inc.**  
**(a development stage company)**  
**Consolidated Balance Sheets**  
**(Unaudited)**  
**(in thousands, except share and per share amounts)**

	December 31, 2012	March 31, 2013
<b>Assets:</b>		
Current assets:		
Cash and cash equivalents	\$ 33,971	\$ 26,736
Investments in marketable securities	65,151	58,015
Receivable due from GSK	3,225	1,308
Prepaid expenses and other current assets	2,270	1,753
Total current assets	<u>104,617</u>	<u>87,812</u>
Property and equipment, less accumulated depreciation and amortization of \$8,501 and \$8,872 at December 31, 2012 and March 31, 2013, respectively	5,029	4,962
Other non-current assets	442	442
<b>Total Assets</b>	<u>\$ 110,088</u>	<u>\$ 93,216</u>
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities:		
Accounts payable and accrued expenses	\$ 8,845	\$ 6,427
Current portion of secured loan	398	398
Warrant liability	—	1,170
Total current liabilities	<u>9,243</u>	<u>7,995</u>
Deferred reimbursements	30,418	31,685
Warrant liability, non-current	908	—
Secured loan, less current portion	299	199
Commitments and contingencies		
Stockholders' equity:		
Common stock, \$.01 par value, 125,000,000 shares authorized, 49,631,672 shares issued and outstanding at December 31, 2012, 49,631,672 shares issued and outstanding at March 31, 2013	556	556
Additional paid-in capital	387,539	389,113
Accumulated other comprehensive income	14	15
Deficit accumulated during the development stage	(318,889)	(336,347)
Total stockholders' equity	<u>69,220</u>	<u>53,337</u>
<b>Total Liabilities and Stockholders' Equity</b>	<u>\$ 110,088</u>	<u>\$ 93,216</u>



*1Q13 Financial Results  
Conference Call & Webcast*

***At the Forefront of Therapies for Rare and Orphan Diseases™***

May 9, 2013



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 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.

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## Agenda

**Introduction**

**Migalastat HCl Monotherapy for Fabry Disease**

**Chaperone-ERT Combination Programs**

**FY12 Financial Results and FY13 Guidance**

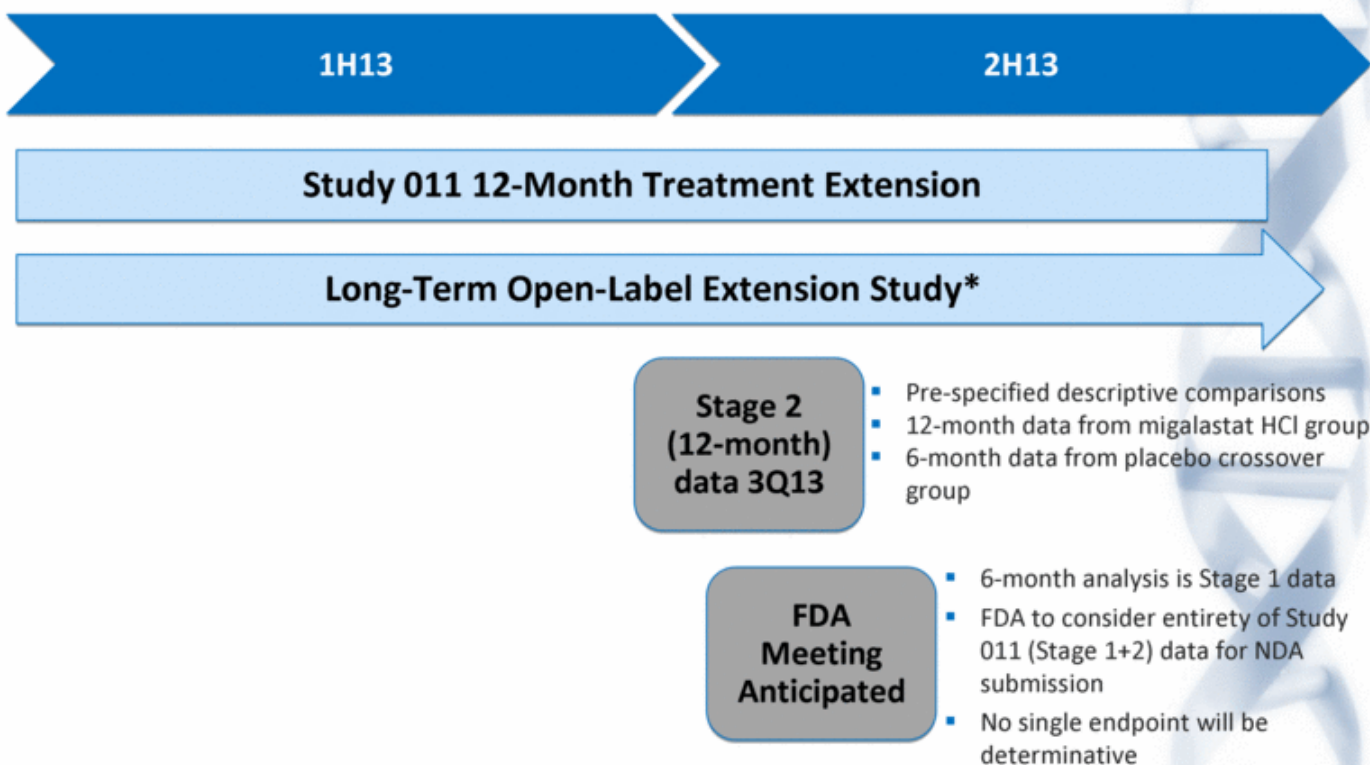
**Upcoming Milestones/Concluding Remarks**

**Q&A**

Slide 3



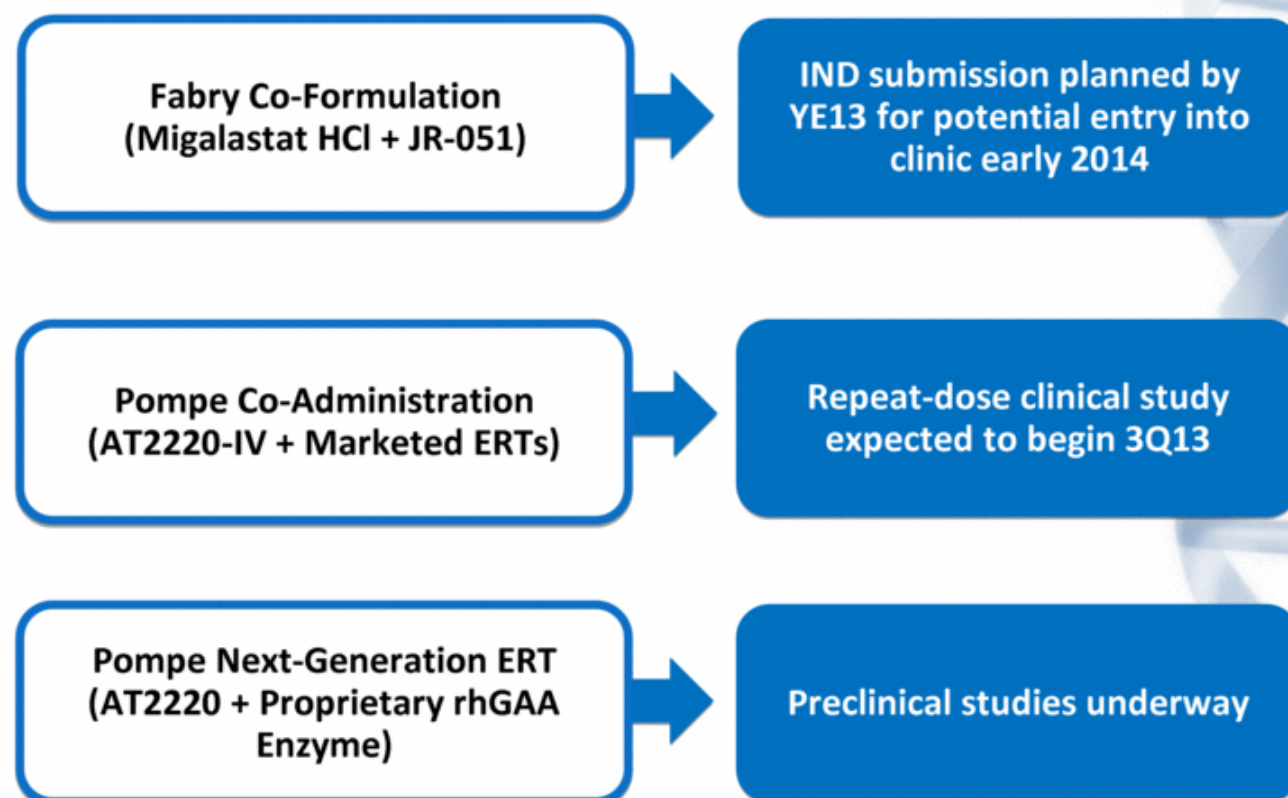
## Study 011 Status and Anticipated Upcoming Milestones



\*Patients rolled over following 12-month treatment extension

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## Chaperone-ERT Combination Programs



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Current cash and anticipated Fabry program reimbursements from GSK expected to fund operations into 2H14

- **Cash position: \$84.8M at March 31, 2013 vs. \$99.1M at December 31, 2012**
- **FY13 net cash spend guidance: \$52M-\$58M**

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

	3 Months Ended March 31,	
	2013	2012
<b>Revenue:</b>		
Research Revenue*	\$ -	\$ 6,114
Collaboration and milestone revenue**	-	1,660
<b>Total revenue</b>	<b>-</b>	<b>7,774</b>
<b>Operating Expenses:</b>		
Research and development	11,989	14,004
General and administrative	4,823	4,095
Depreciation and amortization	439	420
<b>Total operating expenses</b>	<b>17,251</b>	<b>18,519</b>
<b>Loss from operations</b>	<b>(17,251)</b>	<b>(10,745)</b>
<b>Non-operating income</b>	<b>(207)</b>	<b>(2,392)</b>
<b>Net loss / net loss attributable to common stockholders</b>	<b>\$ (17,458)</b>	<b>\$ (13,137)</b>
<b>Net loss per common share – basic and diluted</b>	<b>\$ (0.35)</b>	<b>\$ (0.35)</b>
<b>Weighted-average common shares outstanding - basic and diluted</b>	<b>49,621,188</b>	<b>37,887,520</b>

\*Cash payments from GSK as part of cost sharing arrangement began being recorded on balance sheet as deferred reimbursements beginning in 3Q12

\*\*Upfront GSK license payment previously amortized in collaboration revenue – unrecognized balance now in deferred reimbursements

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## Revenue Recognition Under Expanded GSK Deal

### Impact from Contingent Future Milestones

Impacts Research and Collaboration Revenue Recognition, Effective 3Q12  
No Impact on Cash or Deal Economics

Fabry cost sharing (~\$1.2M in 1Q13 and ~\$7.8M in 2H12)

Balance of unrecognized upfront license payment (~\$22.7M on 3/31/13)

Consolidated Balance Sheets (Unaudited)*	Mar. 31, 2013	Dec. 31, 2012
<b>Assets:</b>		
Total Current Assets	87,812	104,617
Total Non-Current Assets	5,404	5,471
<b>Total Assets</b>	<b>93,216</b>	<b>110,088</b>
<b>Liabilities &amp; Stockholders' Equity</b>		
Total current liabilities:	7,995	9,243
Deferred reimbursements less current portion	31,685	30,418
Warrant Liability	-	908
Secured loan, less current portion	199	299
<b>Total liabilities</b>	<b>39,879</b>	<b>40,868</b>
Commitments and contingencies		
<b>Total stockholders' equity</b>	<b>53,337</b>	<b>69,220</b>
<b>Total Liabilities &amp; Stockholder's Equity</b>	<b>\$ 93,216</b>	<b>\$ 110,088</b>



## Building Shareholder Value

### Migalastat HCl Monotherapy for Fabry Disease

- |   |           |
|---|-----------|
| ✓ Study 011 6-Month data (Stage 1) at LDN WORLD | Feb. 2013 |
| ▪ Top-line Study 011 12-month data (Stage 2)    | 3Q13      |
| ▪ FDA meeting to discuss U.S. approval pathway  | 2H13      |

### Pompe Chaperone-ERT Co-Administration

- |   |           |
|---|-----------|
| ✓ Phase 2 Study 010 data at LDN WORLD (all 4 cohorts) | Feb. 2013 |
| ▪ Initiation of repeat-dose clinical study            | 3Q13      |

### Fabry Chaperone-ERT Co-Formulation (Migalastat HCl + JR-051)

- |                     |      |
|---------------------|------|
| ▪ IND submission    | 4Q13 |
| ▪ Entry into clinic | 1Q14 |

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## Q&A

**John F. Crowley, Chairman & CEO**  
**Chip D. Baird, Chief Financial Officer**  
**Bradley L. Campbell, Chief Business Officer**  
**David J. Lockhart, PhD, Chief Scientific Officer**

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