

**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 20, 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 August 20, 2014, Amicus Therapeutics, Inc. (the "Company") hosted a conference call and webcast to discuss the positive Phase 3 data from Fabry Monotherapy Study 012. A copy of conference call presentation materials is attached hereto as Exhibit 99.1.

**Item 8.01. Other Events.**

On August 20, 2014, the Company issued a press release announcing the positive Phase 3 data from Fabry Monotherapy Study 012. A copy of this press release is attached hereto as Exhibit 99.2.

**Item 9.01. Financial Statements and Exhibits.**

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

<b>Exhibit No.</b>	<b>Description</b>
99.1	August 20, 2014 Conference Call Presentation Materials
99.2	Press Release dated August 20, 2014 titled "Amicus Therapeutics Announces Positive Phase 3 Data from Fabry Monotherapy Study 012."

**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 20, 2014

By: /s/ WILLIAM D. BAIRD III

Name: William D. Baird III

Title: Chief Financial Officer

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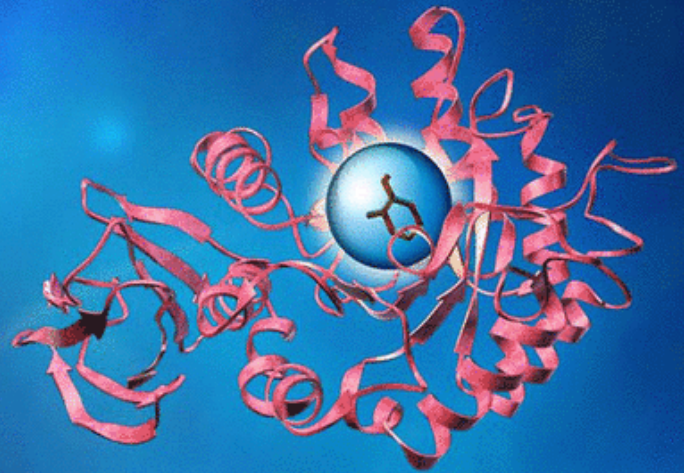
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**EXHIBIT INDEX**

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***Phase 3 Fabry Monotherapy Study  
(Study 012) Results Conference Call***

*August 20, 2014*

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

## Safe Harbor

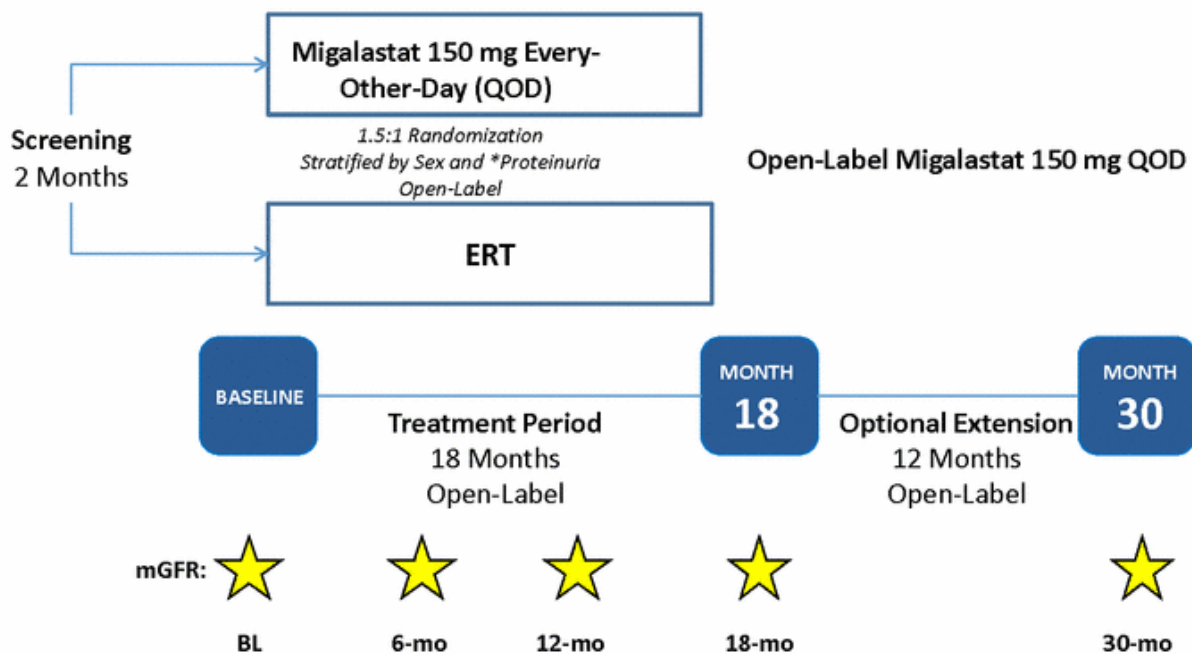
*This presentation contains “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995 relating to business, operations and financial conditions of Amicus including but not limited to preclinical and clinical development of Amicus’ candidate drug products, 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.*

# Study 012 Top-Line 18-Month Data: *Key Findings*

## Migalastat Successfully Met Co-Primary Endpoints of Comparability to ERT on Key Measures of Kidney Function

- Migalastat had a comparable effect to ERT on patients' kidney function as measured by the change in both eGFR\* and mGFR\* with 100% overlap in the 95% confidence intervals for both measures
- Levels of plasma lyso-Gb3, an important biomarker of disease, remained low and stable in patients with amenable mutations who switched from ERT to migalastat
- Migalastat was generally safe and well-tolerated
- Of 48 subjects with GLP HEK-amenable mutations who completed Study 012, 46 (96%) elected to continue with the 12-month treatment extension and 45 remain on migalastat today as their only treatment for Fabry disease

# Phase 3 ATTRACT Study (Study 012)



**\*Proteinuria Stratification:**

High ( $\geq 0.1$  g/24h)

Low ( $< 0.1$  g/24h)

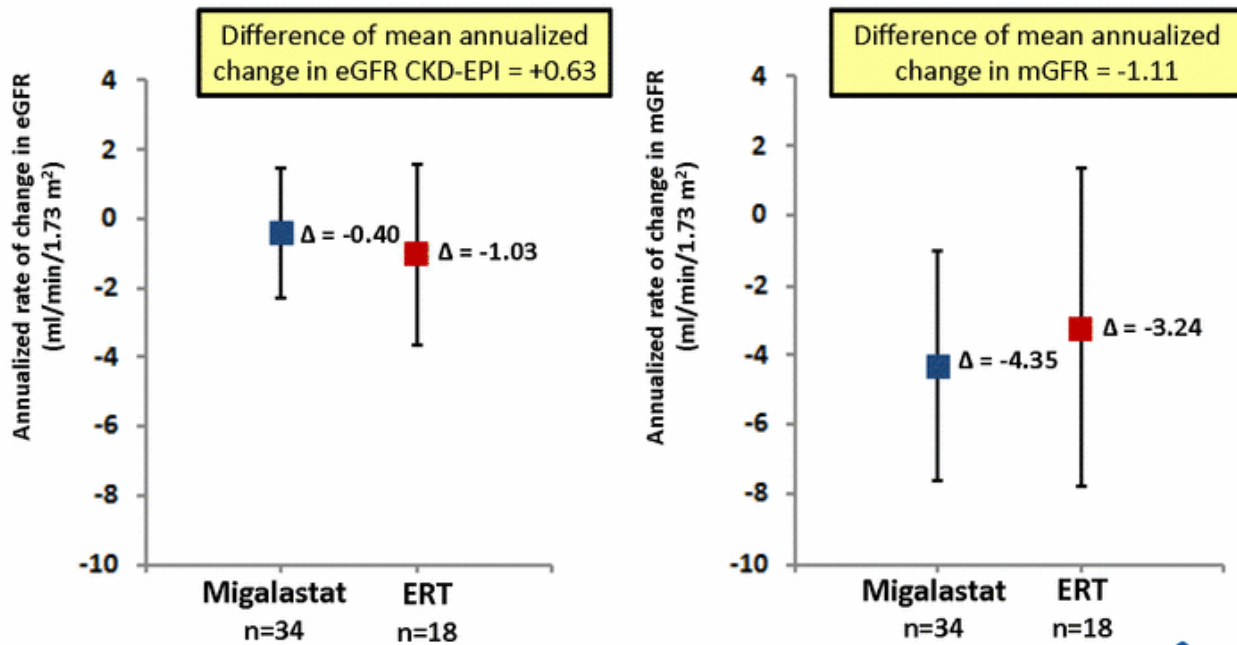
# Study 012 Statistical Analysis Plan

## Co-Primary Outcome Measures Assessing Comparability Between Migalastat and ERT on Key Measures of Kidney Function

- Comparability between migalastat and ERT in eGFR and mGFR assessed by:
  - Overlap of 95% CI >50%
  - Means within 2.2 mL/min/1.73 m<sup>2</sup>/yr
- Statistical model:
  - Primary efficacy analysis using ANCOVA model accounting for treatment group, sex, age, baseline GFR, and baseline proteinuria

# Study 012 Kidney Function: Annualized GFR at Month 18 – ANCOVA model [mITT]

100% Overlap of Migalastat Confidence Intervals with ERT Confidence Intervals



6 Data represent LS means and 95% confidence intervals



# Study 012 Kidney Function:

Annualized Change in GFR at Month 18 – ANCOVA model [mITT]

Migalastat Successfully Met Both Co-Primary Endpoints of Comparability to ERT on Key Measures of Kidney Function

ANCOVA [mITT]	95% CI overlap >50%?	Difference of means within 2.2?	Migalastat group (mean ± SEM) n = 34	ERT group (mean ± SEM) n = 18	Difference in means
eGFR (CKD-EPI)	✓	✓	-0.40 ± 0.93	-1.03 ± 1.29	+0.63
mGFR (iohexol)	✓	✓	-4.35 ± 1.64	-3.24 ± 2.27	-1.11

<sup>7</sup> Terms in the model included treatment, sex, age, baseline mGFR, baseline proteinuria; LS means are presented



# AT1001-012 Kidney Function:

*Annualized Change in GFR at Month 18 – Median ± IQR [mITT]*

**Results Demonstrate Comparability of Median Annualized Changes in eGFR and mGFR**

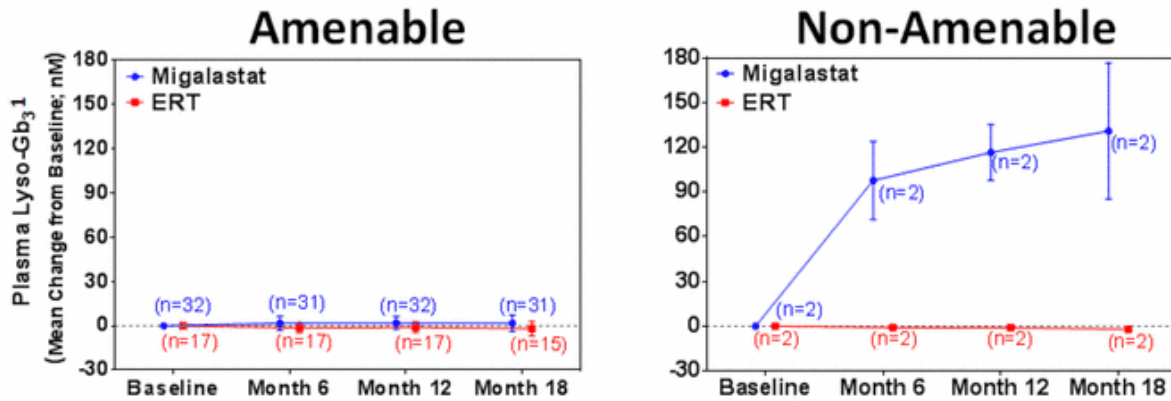
ANCOVA [Per Protocol]	Migalastat group (median, IQR) n = 34	ERT group (median, IQR) n = 18	Difference in medians
eGFR (CKD-EPI)	-1.29 [-3.33, 1.32]	-0.87 [-6.91, 1.77]	-0.42
mGFR (iohexol)	-3.23 [-7.57, 2.84]	-3.57 [-8.57, 0.01]	+0.34

▪ IQR = interquartile range [Q1-Q3]



# Disease Substrate in Plasma (Plasma Lyso-Gb3)

No Change in Plasma Lyso-Gb3 over 18 months Following Switch From ERT to Migalastat in Subjects with Amenable Mutations



- In subjects with amenable mutations the plasma lyso-Gb3 levels were comparable for migalastat and ERT
- In two male subjects with non-amenable mutations plasma lyso-Gb3 increased following switch from ERT as compared to two (1M, 1F) who remained on ERT

Data points represent the mean, Error bars are SD; Based on subjects with available samples for this analysis

# Safety Summary

## Common AEs ( $\geq 10\%$ )

### Migalastat Was Generally Safe and Well-Tolerated

	Migalastat	ERT
N subjects	36	21
n subjects with TEAEs (%)	34 (94%)	20 (95%)
Nasopharyngitis	33%	33%
Headache	25%	24%
Dizziness	17%	10%
Influenza	14%	19%
Abdominal Pain	14%	10%
Diarrhea	14%	10%
Nausea	14%	10%
Back Pain	11%	14%
Upper Respiratory Tract Infection	11%	5%
Urinary Tract Infection	11%	5%
Cough	8%	24%
Vomiting	8%	14%
Sinusitis	8%	14%
Arthralgia	8%	10%
Bronchitis	6%	14%
Edema Peripheral	6%	10%
Vertigo	3%	10%
Dry Mouth	3%	10%
Gastritis	3%	10%
Pain In Extremity	3%	10%
Dyspnea	3%	10%
Procedural Pain	-	10%

# Migalastat Monotherapy Experience

97 Patients Today Take Migalastat HCl as Only Therapy for Fabry Disease<sup>1</sup>



<sup>1</sup> All patients are receiving investigational drug, migalastat HCl, as part of ongoing clinical trials

\*Retention defined as # of patients who complete a study and chose to enter extension, e.g., 011 12-mo into 12-mo extension or 011 into 041

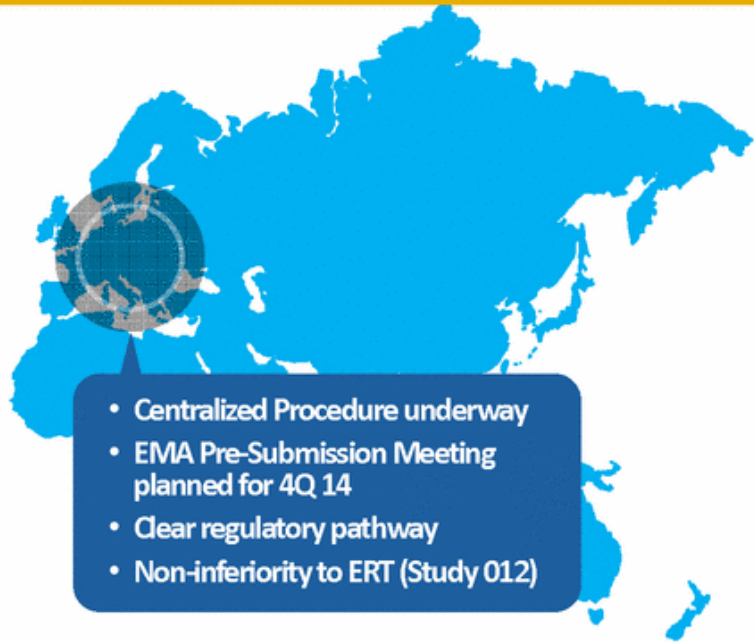
<sup>11</sup> As of Aug 4 2014



# Global Regulatory Strategy

## Pursuing Fastest Path to Approval for Migalastat

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



- Centralized Procedure underway
- EMA Pre-Submission Meeting planned for 4Q 14
- Clear regulatory pathway
- Non-inferiority to ERT (Study 012)



## Amicus Therapeutics Announces Positive Phase 3 Data from Fabry Monotherapy Study 012

### *Migalastat Successfully Meets Both Co-Primary Endpoints of Comparability to Enzyme Replacement Therapy (ERT) on Both Key Measures of Kidney Function*

#### *Comparability to ERT Also Demonstrated in Important Fabry Disease Biomarker, Plasma Lyso-Gb3*

#### *Proceeding with Centralized Procedure for European MAA Submission*

#### *Conference Call and Webcast Today at 8:00 a.m. ET*

**CRANBURY, NJ, August 20, 2014** — Amicus Therapeutics (Nasdaq: FOLD), a biopharmaceutical company at the forefront of therapies for rare and orphan diseases, today announced positive 18-month data from its second Phase 3 study (Study 012) of the oral small molecule chaperone migalastat HCl (“migalastat”) in Fabry patients with amenable mutations. Detailed results of this second Phase 3 monotherapy study are available in a slide presentation that will be shared by the Amicus management team on a conference call today at 8:00 a.m. ET. Please visit <http://ir.amicustherapeutics.com/events.cfm>.

Study 012 compared oral migalastat to standard-of-care enzyme replacement therapies (ERTs) for Fabry disease (Fabrazyme® and Replagal®). The co-primary outcome measures were the mean annualized changes in estimated glomerular filtration rate (eGFR) and measured (iohexol) GFR (mGFR) assessed by descriptive comparisons of migalastat and ERT over 18 months. The study enrolled 60 patients (26 males and 34 females) with Fabry disease with amenable mutations in a clinical trial assay who had been treated with ERT for a minimum of 12 months prior to study entry. These patients were randomized 1.5:1 to switch to migalastat (36 patients) or remain on ERT (24 patients) for the primary 18-month treatment period, after which they were eligible to receive migalastat in a 12-month extension phase. Among the 60 patients enrolled, 56 (34 in the migalastat group and 22 in the ERT group) had amenable mutations in a GLP-validated human embryonic kidney (HEK) cell-based *in vitro* assay (“GLP HEK-amenable”). The statistical analysis plan pre-specified that all efficacy measures would be based on the results from patients with GLP HEK-amenable mutations.

#### Summary of Study 012 18-Month Results

- Migalastat had a comparable effect to ERT on patients’ kidney function as measured by the change in eGFR and mGFR.
- Levels of plasma lyso-Gb3, an important biomarker of disease, remained low and stable in patients with amenable mutations who switched from ERT to migalastat.
- Migalastat was generally safe and well-tolerated.
- Of 48 patients with GLP HEK-amenable mutations who completed Study 012, 46 (96%) elected to continue with the 12-month treatment extension and 45 remain on migalastat today as their only treatment for Fabry disease.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc., stated, “We believe that this multi-year study unequivocally demonstrates that a Fabry patient on ERT with an amenable mutation can switch

safely and effectively from ERT to migalastat to treat their Fabry disease. Today is a great day for the Fabry community and for Amicus. This study was resoundingly positive and met our pre-defined criteria for success in terms of the co-primary endpoints of kidney function. These results clearly show that migalastat is comparable to ERT in slowing the progression of Fabry disease and continues to demonstrate a favorable safety profile. With every-other-day oral administration and a differentiated mechanism of action, migalastat may offer significant advantages for patients without the need for bi-weekly infusions with ERT. Combined with our previous Phase 3 results from Study 011, we have a compelling and consistent data set from both treatment-naïve and ERT-experienced patients. Given these results and the great need for new and effective medicines, we plan to work with European and U.S. regulators to determine the fastest way to get migalastat approved for all amenable Fabry patients.”

Raphael Schiffmann, M.D., M.H.Sc., an investigator with the Institute of Metabolic Disease, Baylor Research Institute, Dallas, TX stated, “I believe the results from Study 012 show a positive treatment effect of migalastat in Fabry patients with amenable mutations. The stabilization of renal function and the maintenance of substrate levels as measured by lyso-Gb3 provide further clinical evidence that supports my experience over the last eight years in treating Fabry patients with migalastat in various clinical studies. When combined with the favorable safety profile, the totality of the data from Study 012 and Study 011 indicate that migalastat should become an important new oral treatment option for Fabry patients.”

Dr. Schiffmann added, “Given the choice, I would use migalastat over ERT for the treatment of Fabry patients with amenable mutations.”

#### Study 012 Kidney Function Data in GLP HEK-Amenable Patients

Among patients with GLP HEK-amenable mutations in Study 012, kidney function was comparable to ERT over 18 months following treatment with migalastat. Decline in kidney function is a key cause of mortality in patients with Fabry disease.

	Overlap of 95% Confidence Intervals (means)	Difference Between Migalastat and ERT (means)	Mean Values ± SEM		Median Values	
			Migalastat group (n=34)	ERT group (n=18)	Migalastat group (n=34)	ERT group (n=18)
			<b>Estimated GFR (eGFR) (CKD-epi)</b>	100%	+0.63	-0.40 ± 0.93
<b>Measured GFR (mGFR) (iohexol)</b>	100%	-1.11	-4.35 ± 1.64	-3.24 ± 2.27	-3.23	-3.57

As pre-specified in the final statistical analysis plan, the co-primary outcome measures of efficacy in Study 012 were the descriptive assessments of comparability of the mean annualized change in eGFR and mGFR for migalastat and ERT. Both eGFR and mGFR are considered important measures of renal function. Comparability on eGFR and mGFR was defined in two ways:

- A  $\geq 50\%$  overlap in the confidence intervals between the migalastat and ERT treatment groups; and
- Whether the mean annualized changes for patients receiving migalastat are within 2.2 mL/min/1.73 m<sup>2</sup>/yr of patients receiving ERT.

“These data mark an exciting day for the Fabry community and validate our long-term commitment to work in partnership with industry toward our goal of multiple treatment options and improved medicines for all people living with Fabry disease,” said Jack Johnson, Founder and Executive Director, Fabry Support & Information Group. “We await the regulatory agencies’ review of these data, and we are grateful to the many people with Fabry disease, families and volunteers who have committed so much of themselves to help accelerate efforts to bring a more convenient and effective therapy to people living with the disease.”

### **About GLP HEK-Amenable Mutations**

Amenable mutations are defined as having an absolute increase of 3% of wild type alpha-Gal A enzyme activity and a relative increase of 20% when exposed to migalastat in a cell-based *in vitro* assay. All patients enrolled in Study 012 had amenable mutations in the clinical trial HEK assay available at study initiation (“clinical trial assay”). Following the completion of enrollment, a GLP HEK assay was developed with a third party to measure the criteria for amenability with more quality control and rigor. However, approximately 10% of mutations in the HEK database switched categorization between “amenable” and “non-amenable” when moving from the clinical trial assay to the GLP HEK assay. Therefore there were changes in categorization from amenable to non-amenable in 4 patients in Study 012.

Overall based on results from mutations tested in the GLP HEK assay, Amicus continues to believe that approximately 30% to 50% of the Fabry population have mutations that are amenable to migalastat.

### **Conference Call and Webcast**

Amicus Therapeutics will host a conference call and audio webcast today, August 20, 2014 at 8:00 a.m. ET to discuss positive results from Study 012. Interested participants and investors may access the conference call at 8:00 a.m. ET by dialing 877-303-5859 (U.S./Canada) or 678-224-7784 (international). The slide presentation for the conference call is available at <http://ir.amicustherapeutics.com/events.cfm>.

An audio webcast can also be accessed via the Investors section of the Amicus Therapeutics corporate web site at <http://ir.amicustherapeutics.com/events.cfm>, 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. A telephonic replay of the call will be available for seven days beginning at 11:00 a.m. ET today. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); participant code 90825426.

### **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 as a monotherapy and in combination with enzyme replacement therapy (ERT) for Fabry disease; and AT2220 (duvoglustat) in combination with ERT for Pompe disease.

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### **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,” “may,” “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, 2013. 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.

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