

**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 15, 2017**

**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))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

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**Item 7.01. Regulation FD Disclosure.**

On May 15, 2017, Amicus Therapeutics, Inc. (the "Company") hosted a conference call and webcast to discuss positive functional data from initial patients in Pompe Phase 1/2 Study. A copy of the conference call presentation materials is attached hereto as Exhibit 99.1.

**Item 8.01. Other Events.**

On May 15, 2017, the Company issued a press release announcing positive functional data from initial patients in Pompe Phase 1/2 Study. 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.

Exhibit No.	Description
99.1	May 15, 2017 Conference Call Presentation Materials
99.2	Press Release dated May 15, 2017 titled "Amicus Therapeutics Announces Positive Functional Data from Initial Patients in Pompe Phase

**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 15, 2017

By: /s/ ELLEN S. ROSENBERG  
Ellen S. Rosenberg  
General Counsel and Corporate Secretary

**EXHIBIT INDEX**

<u>Exhibit No.</u>	<u>Description</u>
99.1	May 15, 2017 Conference Call Presentation Materials
99.2	Press Release dated May 15, 2017 titled “Amicus Therapeutics Announces Positive Functional Data from Initial Patients in Pompe Phase 1/2 Study.”



# Positive Pompe Phase 1/2 Functional Data in Initial Patients

## Conference Call & Webcast

May 15, 2017



## Safe Harbor

*This presentation contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including statements relating to encouraging preliminary data from a global Phase 1/2 study to investigate ATB200/AT2221 for the treatment of Pompe and the potential implications on these data for the future advancement and development of ATB200/AT2221. Words such as, but not limited to, "look forward to," "believe," "expect," "anticipate," "estimate," "intend," "confidence," "encouraged," "potential," "plan," "targets," "likely," "may," "will," "would," "should" and "could," and similar expressions or words identify forward-looking statements. The forward looking statements included in this presentation are based on management's current expectations and beliefs which are subject to a number of risks, uncertainties and factors, including that the preliminary data based on a small patient sample and reported before completion of the study will not be predictive of future results, that results of additional preliminary data or data from the completed study or any future study will not yield results that are consistent with the preliminary data presented, that the Company will not be able to demonstrate the safety and efficacy of ATB200/AT2221, that later study results will not support further development, or even if such later results are favorable, that the Company will not be able to successfully complete the development of, obtain regulatory approval for, or successfully commercialize ATB200/AT2221. In addition, all forward looking statements are subject to the other risks and uncertainties detailed in our Annual Report on Form 10-K for the year ended December 31, 2016 and Quarterly Report on 10-Q for the Quarter ended March 31, 2017. As a consequence, actual results may differ materially from those set forth in this press release. You are cautioned not to place undue reliance on these forward looking statements, which speak only of the date hereof. All forward looking statements are qualified in their entirety by this cautionary statement and we undertake no obligation to revise this press release to reflect events or circumstances after the date hereof.*

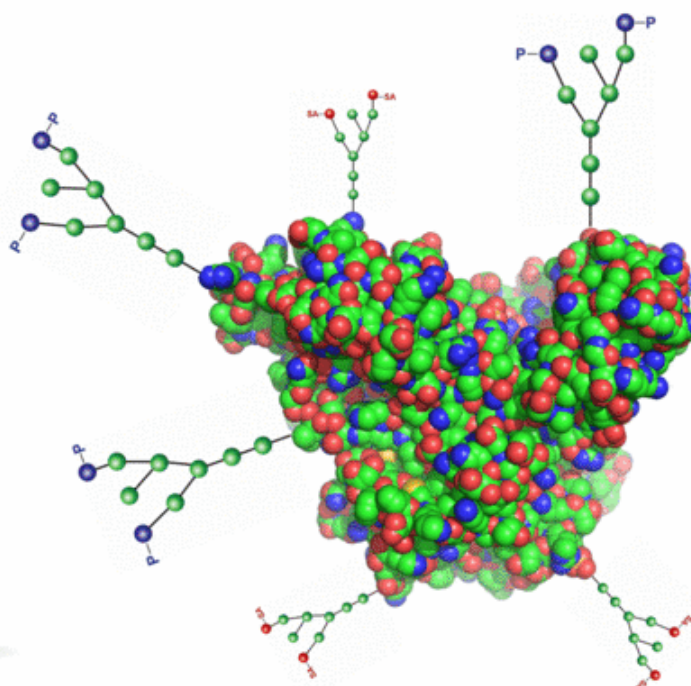
# ATB200 + Chaperone: A Highly Differentiated Approach

## Novel Pompe Treatment Paradigm with Three Key Differentiators

**ATB200  
(Novel ERT)**



**Chaperone  
addition**



**Optimized  
mixture of  
glycans**

**High levels of  
M6P and bis  
M6P**

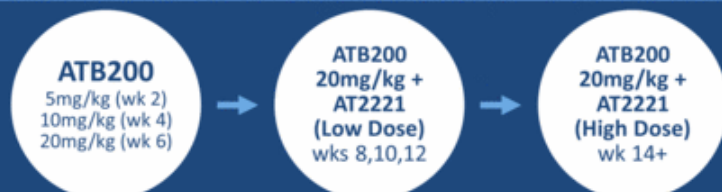
\*Artist rendering, not actual product image

# Phase 1/2 ATB200-02 Study Design

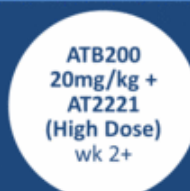
Phase 1/2 Clinical Study to Evaluate Safety, Tolerability, Pharmacokinetics (PK), and Pharmacodynamics (PD) of ATB200 + Chaperone (ATB200/AT2221)

## 18-Week Primary Treatment Period with Long-Term Extension (n=20)

Cohort 1 (Ambulatory ERT-Switch, n=11)



Cohort 2 (Non-Ambulatory ERT-Switch, n=4) & Cohort 3 (ERT-Naïve, n=5)



### Assessments:

- Safety/Tolerability
- Plasma PK
- Infusion-Associated Reactions
- Antibody & Cytokine Levels
- Pharmacodynamics
- Efficacy (Long-Term Extension)

## Baseline Characteristics of Patients in Phase 1/2 ATB200-02 Study (n=20)

### Patients Enrolled Across Three Cohorts Representative of Overall Late-Onset Pompe Population with Impairment at Baseline

Baseline Characteristics (N=20)	Cohort 1: Ambulatory ERT-Switch* (N=11)	Cohort 2: Non-Ambulatory ERT-Switch (N=4)	Cohort 3: ERT-Naïve (N=5)
Time on Lumizyme® – mean years (SD)	4.77 (1.42)*	8.9 (3.8)	N/A
Age – mean years (range)	49.4 (28, 66)	36.0 (18, 56)	49.4 (24, 65)
Sex M:F	9:2	3:1	1:4
6MWT – mean meters (SD)	392.0 (93.4)	N/A	399.5 (83.5)
FVC Upright – mean % predicted (SD)	52.3 (13.2)	N/A	53.4 (20.3)

\*Cohort 1 patients required to have been on Lumizyme for 2-6 years at baseline

## Safety Summary (n=20)\*

**Preliminary Safety Data for ATB200/AT2221 Show AEs Have Been Generally Mild and Transient with No Infusion-Associated Reactions After 200+ Total Infusions Across All Cohorts**

- AEs were generally mild and transient
- No infusion-associated reactions reported after 200+ total infusions across all patients
- Longest duration of treatment is 48 weeks

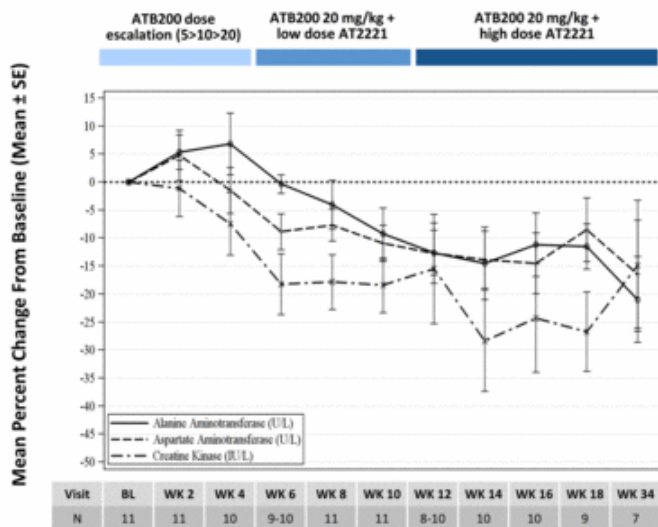
\*Reported through interim data analysis (maximum 48 weeks)



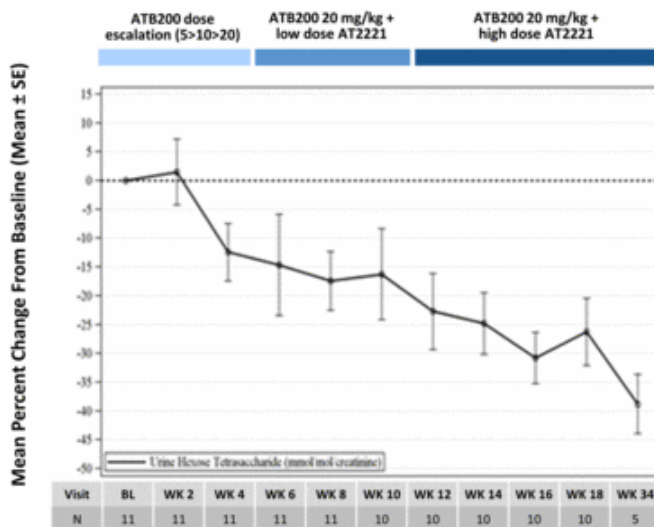
# Cohort 1: Biomarkers up to Week 34 (N=11)\*

After Switching from SOC to ATB200/A2221 Patients Demonstrated an Improvement in Biomarkers of Muscle Damage (CK, ALT, AST) and Biomarker of Disease Substrate (Hex4) for up to 34 Weeks

## Percent Change from Baseline for CK, ALT, AST



## Percent Change from Baseline for Hex 4

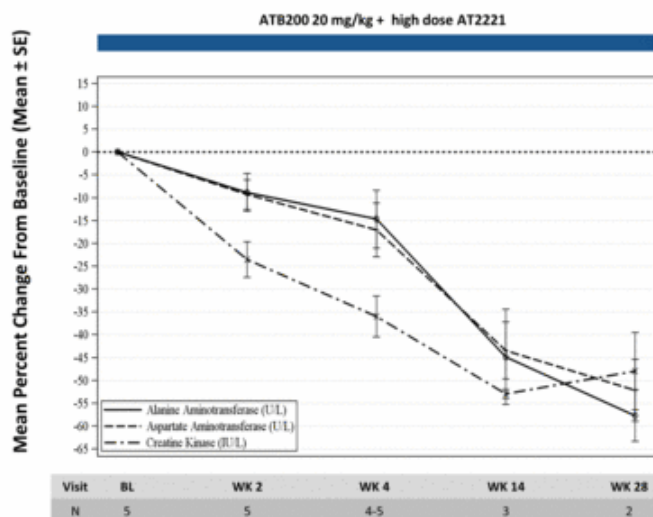


\*Reported through interim data analysis (maximum 34 weeks); Missing values either unable to be analyzed or not yet analyzed

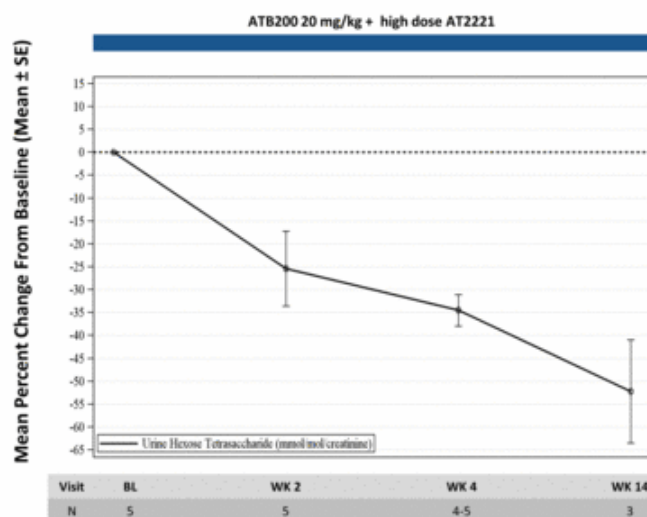
# Cohort 3: Biomarkers up to Week 14 (N=5)\*

Naïve Patients Treated with ATB200/AT2221 Demonstrated Robust Reduction in Biomarkers of Muscle Damage (CK, ALT, AST) and Biomarker of Disease Substrate (Hex4) for up to 14-28 Weeks

Percent Change from Baseline for CK, ALT, AST



Percent Change from Baseline for Hex 4



\*Reported through interim data analysis (maximum 28 weeks); Missing values either unable to be analyzed or not yet analyzed

## 6-Minute Walk Test (6MWT) Summary at Month 6 (n=9)

6MWT Distance Improved for Both ERT-Naïve Patients (Mean +52 Meters)  
and ERT-Switch Patients (Mean +38 Meters) at Month 6

### 6-Minute Walk Test (m): Month 6

Cohort	Baseline Mean (SD)	Change at Month 6 Mean (SD)
Cohort 3 ERT Naïve (n=2)	432 (68)	+52 (15)
Cohort 1 ERT Switch (n=7)	383 (103)	+38 (43)

6MWT Increased in 2/2 ERT-Naïve Patients and 6/7 ERT-Switch Patients

## Other Motor Function Tests at Month 6 (n=9)

Other Motor Function Tests Show Improvements for Both ERT-Naïve and ERT-Switch Patients, Consistent With 6MWT

### Other Motor Function Tests: Month 6

Patients	Timepoint	4 Stair Climb Mean (SD) (sec)	Timed Up and Go Mean (SD) (sec)	10M walk Mean (SD) (sec)
Cohort 3: ERT Naïve (n=2)	Baseline	3.9 (0.6)	8.9 (0.9)	6.9 (0.8)
	Change at Month 6	-0.3 (0.0)	-1.4 (0.4)	-0.5 (0.2)
Cohort 1: ERT Switch (n=7)	Baseline	4.4 (3.1)	11.0 (7.7)	7.5 (3.5)
	Change at Month 6	-1.1 (1.3)	-1.9 (2.8)	-0.04 (1.6)

## Cohort 2 Muscle Strength Testing at Month 6 (n=1)

Substantial Improvement Observed in Shoulder and Elbow Strength in First Non-Ambulatory ERT-Switch Patient with Available Data at Month 6

### Quantitative Muscle Testing (QMT) - Dynamometer

Assessment	Elbow Flex		Elbow Extension		Shoulder Adduction		Shoulder Abduction		<b>Scoring</b> Measurement of force production in pounds as measured by dynamometer
	Right	Left	Right	Left	Right	Left	Right	Left	
Baseline	1.0	0.9	1.2	1.1	0.8	0.5	1.3	0.9	
Month 6	4.1	3.3	3.5	3.2	2.8	0.0	3.3	3.6	
CFBL	+3.1	+2.4	+2.3	+2.1	+2.0	-0.5	+2.0	+2.7	

### Manual Muscle Testing (MMT)\*

Assessment	Elbow Flex		Elbow Extension		Shoulder Adduction		<b>Scoring</b> 1. Visible muscle movement, but no movement at the joint 2. Movement at the joint, but not against gravity 3. Movement against gravity, but not against added resistance 4. Movement against resistance, but less than normal 5. Normal strength
	Right	Left	Right	Left	Right	Left	
Baseline	2	2	2	2	2	2	
Month 6	4	3	4	3	2	2	
CFBL	+2	+1	+2	+1	0	0	

\*R/L shoulder abduction by MMT not assessed at M6

## Forced Vital Capacity (FVC) Summary at Month 6 (n=8)\*

FVC Results Show Improvement in ERT-Naïve Patients (Mean +3.0%) and Stability in ERT-Switch Patients (Mean +0.3%) at Month 6

### FVC (% Predicted): Month 6

Cohort	Baseline Mean (SD)	Absolute Change at Month 6 Mean (SD)
Cohort 3 ERT Naïve (n=2)	51 (27)	+3 (0)
Cohort 1 ERT Switch (n=6)*	51 (17)	+0.3 (3)

**FVC increased in 2/2 ERT-Naïve patients and 3/6 ERT-Switch patients**

\*FVC results not available for 1 subject at month 6

## Other Pulmonary Function Tests at Month 6 (n=8-9)\*

MIP increased and MEP decreased in ERT-naïve patients,  
MIP and MEP both increased in ERT-switch patients

### Other Pulmonary Function Tests: Month 6

Patients	Timepoint	MIP Mean (SD)	MEP Mean (SD)
Cohort 3: ERT Naïve (n=2)	Baseline	45.5 (27.6)	57.5 (9.2)
	Change at Month 6	+8.5 (3.5)	-4.5 (17.7)
Cohort 1: ERT Switch (n=6-7)*	Baseline	35.4 (11.3)	69.5 (21.2)
	Change at Month 6	+1.0 (5.2)	+15.5 (25.4)

\*MEP results not available for 1 patient at month 6

## Preliminary Functional Data Summary

- **Muscle function at Month 6**

- Muscle function improved in 9/10 patients
- Mean 6MWT distance improved in both naïve (+52 Meters) and ERT-switch (+38 Meters) patients (8 out of 9)
- Other motor function tests in ambulatory patients consistent with 6MWT
- First non-ambulatory patient showed significant improvements in muscle strength tests

- **Pulmonary function at Month 6**

- FVC increased in ERT-naïve patients (mean +3.0%) and was stable in ERT-switch patients (mean +0.3%)
- MIP and MEP generally consistent with FVC



# Pompe Phase 1/2 Study ATB200-02 Data Cascade

On Track to Report Full Data Set in 3Q17

## Pompe Milestones in 2017



### 18-WEEK DATA

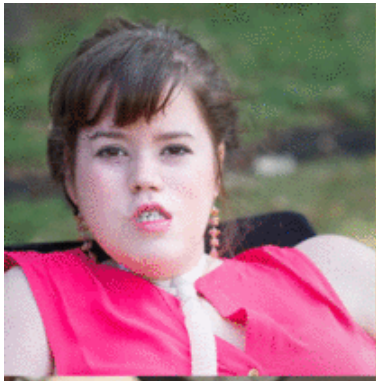
- Safety / tolerability
- Pharmacokinetics (PK)
- Biomarkers
- Immunogenicity

### EXTENSION DATA

- Motor/pulmonary function

Thank you





# Appendix

# Pompe Disease Overview

## Pompe Disease is Heterogeneous Across a Broad Spectrum of Patients

Deficiency of GAA leading to glycogen accumulation

Respiratory and cardiac failure are leading causes of morbidity and mortality

Age of onset ranges from infancy to adulthood

5,000 – 10,000 patients diagnosed WW<sup>1</sup>

Symptoms include muscle weakness, respiratory failure, and cardiomyopathy

~\$800M+ Global Pompe ERT sales in FY15<sup>2</sup>



1. National Institute of Neurological Disorders and Stroke (NIH). 2. Sanofi Press Release & 10-K

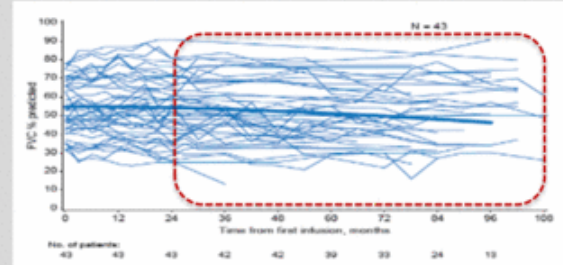
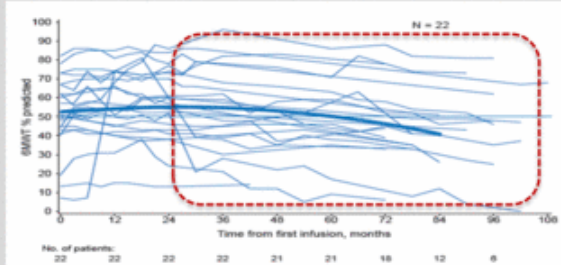
# Long-Term Motor and Pulmonary Function on Lumizyme Treatment

Patients Expected to Stabilize or Experience Progressive Decline in Motor and Pulmonary Function After 2-6 Years on Lumizyme Treatment

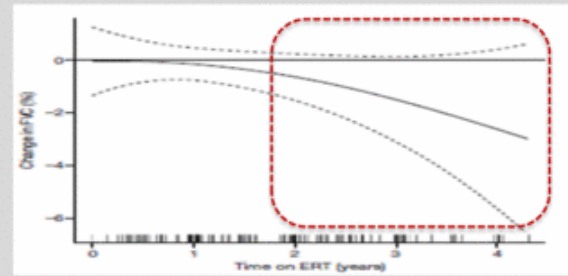
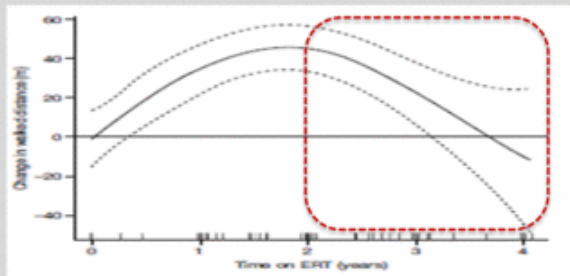
## Six Minute Walk Test (m)

## FVC (%)

LOTS Study Extension  
(van der Ploeg et al. 2017)



UK Health Technology Assessment\*  
(Wyatt et al. 2012)



\*Data show the age-adjusted association between time on ERT and 6MWT/FVC

## Summary of Motor (6MWT) and Pulmonary (FVC) Function with Standard of Care and Investigational ERTs

**General Improvement in ERT-Naïve Population During Initial Treatment on All ERTs –  
Data on Development Stage ERTs in Switch Population has Been Inconsistent**

Patients	Treatment	Study (Duration, n)	CFBL 6MWT (m)	CFBL FVC (%)
Untreated	Placebo	LOTS (72 wks, n=30)	-3	-2.2
ERT-Naïve	Lumizyme	LOTS (78 wks, n=60)	+25	+1.2
	Neo-GAA	Neo-GAA Phase 2 (24 wks, n=3)	+24	+6.2
	BMN701	BMN701 Phase 1/2 (24 wks, n=16)	+22	+1.2
ERT-Switch	Neo-GAA	Neo-GAA Phase 2 (24 wks, n=6)	-6	+1.4
	BMN701	BMN701 Phase 2 (24 wks, n=18)	+26	-3.7

Sources: van der Ploeg, *et al.* NEJM (2010); WORLDSymposium 2016 Poster and Sanofi press release March 2016; BioMarin press release March 2013; BioMarin J.P. Morgan 2016 presentation



## Amicus Therapeutics Announces Positive Functional Data Initial Patients in Pompe Phase 1/2 Study

*Mean Six-Minute Walk Distance at Month Six Improved in ERT-Naïve Patients (+52 Meters) and ERT-Switch Patients (+38 Meters)*

*Muscle Function Improved in 9 out of 10 Patients*

*Pulmonary Function Improved in a Majority of Patients*

*No Infusion-Associated Reactions Following 200+ Infusions*

*Conference Call at 8:30am ET*

**CRANBURY, NJ, May 15, 2017** — Amicus Therapeutics (Nasdaq: FOLD) today announced additional positive functional data from initial patients in a global Phase 1/2 study (ATB200-02) to investigate ATB200/AT2221 in patients with Pompe disease. Patients who completed six months of treatment with ATB200/AT2221 showed improvements in the six-minute walk test (6MWT) distance and other measures of motor function, in addition to stability or improvements in forced vital capacity (FVC). Consistent with previous results(1) presented at the 2017 *WORLD Symposium™*, patients treated with ATB200/AT2221 continue to show improvements in biomarkers of muscle damage and disease substrate.

“We are very pleased to see improvements in six minute walk distance and other measures of motor function in both naïve and ERT-switch patients, as well as stability or improvements in forced vital capacity. The consistency and magnitude of improvements exceeded our expectations and follow the initial improvements seen on key biomarkers of muscle damage and disease substrate,” said John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. “These preliminary functional results are very encouraging and suggest a clinically meaningful improvement for patients. We look forward to additional data from all patients in the third quarter as we continue in our mission to develop an improved treatment option for people living with Pompe disease.”

### ATB200-02 Study — Updated Data Highlights in Initial ERT-Switch and Naïve Patients

#### **Safety, Tolerability & Pharmacokinetics (PK)**

Safety and tolerability data are currently available for all 20 patients enrolled in the study (maximum 48 weeks). To date, adverse events have been generally mild and transient. Importantly, ATB200/AT2221 has also shown no infusion-associated reactions following 200+ infusions. As previously reported, the clinical PK profile has been consistent with previously reported preclinical data.

#### **Pharmacodynamic (PD) Data on Muscle Damage and Disease Substrate Biomarkers (n=16)**

PD data are currently available for 11 ERT-switch patients and five ERT-naïve patients. Improvements in key biomarkers of muscle damage and disease substrate continue to suggest a positive effect of ATB200/AT2221 on muscle cells after up to 34 weeks of treatment.

- **Muscle damage biomarkers:** Creatine kinase (CK) enzyme, alanine aminotransferase (ALT), and aspartate aminotransferase (AST) continue to show a decrease in a majority of patients. Across the three biomarkers, mean reductions from baseline were approximately 15-20% and 50-55% for the ERT-switch and ERT-naïve patients, respectively.
- **Disease substrate biomarker:** Urine hexose tetrasaccharide (Hex4) continues to show decreases in a majority of ERT-switch patients and all ERT-naïve patients, with mean reductions from baseline of approximately 40% and 50% for the ERT-switch and ERT-naïve patients, respectively.

#### **Functional Outcomes at Month 6 (n=10)**

Functional outcomes data from baseline to Month 6 are currently available for 10 patients (seven ambulatory ERT-switch, two ERT-naïve and one non-ambulatory ERT-switch). Motor function improved and pulmonary function was stable in ambulatory ERT-switch patients; motor and pulmonary function both improved in ERT-naïve patients. Muscle strength data are available from the first non-ambulatory ERT-switch patient and showed improvement.

- **Muscle Function:**
  - **Motor function:** Six-minute walk test (6MWT) distance, a primary measure of motor function in Pompe patients, increased in both ERT-switch patients (mean +38 meters; improvement in 6/7 patients) and ERT-naïve patients (mean +52 meters; improvement in 2/2 patients). Other motor function tests also showed mean improvements, consistent with 6MWT distance.
  - **Muscle Strength:** In the first non-ambulatory ERT-switch patient, improvements in four out of four muscle groups on the quantitative muscle testing (QMT) and two of three muscle groups on the manual muscle testing (MMT) were observed.
- **Pulmonary Function:** Forced vital capacity (FVC), the primary measure of pulmonary function, was stable in ERT-switch patients (mean absolute change in percent predicted FVC +0.3%) and improved in ERT-naïve patients (mean absolute change +3.0%). Other pulmonary tests included maximal inspiratory pressure (MIP), a measure of inhalation, and maximal expiratory pressure (MEP), a measure of exhalation. MIP and MEP both showed mean increases in ERT-switch patients. MIP showed a mean increase and MEP showed a mean decrease in ERT-naïve patients.

Prof. Dr. Benedikt Schoser of the Friedrich-Baur Institute in Munich, Germany stated, “These preliminary data from the first clinical study of ATB200/AT2221 are very positive and suggest that this could become a significant and different treatment paradigm for Pompe disease. There have been considerable improvements in functional measures, especially the six minute walk test, among both naïve patients and in ambulatory patients who switched from standard of care. To my knowledge, no other investigational agents show similar positive results across such a broad range of patients at this stage of development. If the full data set is according to these functional measures, then it could be very meaningful for our patients.”

### **Summary of Functional Outcomes from Baseline to Month 6**

#### **Cohort 1 ERT-Switch Patients (n=7): Functional Outcomes on ATB200/AT2221 from Baseline to Month 6**

	Motor Function Tests (n=7)			Pulmonary Function Tests (n=6-7)			
	6MWT (m)	4 Stair Climb (sec)	Timed up and go (sec)	10m walk (sec)	FVC (%)	MIP	MEP
<b>Baseline Mean (SD)</b>	383 (103)	4.4 (3.1)	11.0 (7.7)	7.5 (3.5)	51 (17)	35.4 (11.3)	69.5 (21.2)
<b>Change from Baseline (SD)</b>	+38 (43)	-1.1 (1.3)	-1.9 (2.8)	-0.04 (1.6)	+0.3 (3)	+1.0 (5.2)	+15.5 (25.4)

#### **Cohort 3 ERT-Naïve Patients (n=2): Functional Outcomes on ATB200/AT2221 from Baseline to Month 6**

	Motor Function Tests (n=2)			Pulmonary Function Tests (n=2)			
	6MWT (m)	4 Stair Climb (sec)	Timed up and go (sec)	10m walk (sec)	FVC (%)	MIP	MEP
<b>Baseline Mean (SD)</b>	432 (68)	3.9 (0.6)	8.9 (0.9)	6.9 (0.8)	51 (27)	45.5 (27.6)	57.5 (9.2)
<b>Change from Baseline (SD)</b>	+52 (15)	-0.3 (0.0)	-1.4 (0.4)	-0.5 (0.2)	+3 (0)	+8.5 (3.5)	-4.5 (17.7)

### **Conference Call and Webcast**

Amicus Therapeutics will host a conference call and webcast today, May 15, 2017 at 8:30 a.m. ET. Interested participants and investors may access the conference call by dialing 877-303-5859 (U.S./Canada) or 678-224-7784 (international) conference ID 23261527. The slide presentation to accompany this conference call and webcast will be available at <http://ir.amicusrx.com/events.cfm>.

An audio webcast can also be accessed via the Investors section of the Amicus Therapeutics corporate web site at <http://ir.amicusrx.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 11:30 a.m. ET today. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); conference ID 23261527.

### **About ATB200-02 Clinical Study**

The primary objectives of the open-label ATB200-02 clinical study are to evaluate the safety, tolerability, pharmacokinetics (PK), and pharmacodynamics (PD) of ATB200/AT2221 over an 18-week primary treatment period followed by a long-term extension. The study enrolled a total of 20 patients across three patient cohorts: ambulatory ERT-switch (Cohort 1, n=11), non-ambulatory ERT-switch (Cohort 2, n=4) and ERT-naïve (Cohort 3, n=5). Patients in Cohort 1 received escalating doses of ATB200 (5, 10, 20 mg/kg), followed by 3 doses of 20 mg/kg ATB200 plus low dose AT2221, followed by ongoing doses of 20 mg/kg ATB200 plus high dose AT2221. Patients in Cohort 2 and 3 patients have all received 20 mg/kg ATB200 plus high dose AT2221.

### **About ATB200/AT2221**

ATB200/AT2221 is a novel treatment paradigm that consists of ATB200, a unique recombinant human acid alpha-glucosidase (rhGAA) enzyme with optimized carbohydrate structures, particularly mannose-6 phosphate (M6P), to enhance uptake, co-administered with AT2221, a pharmacological chaperone. In preclinical studies, ATB200 was associated with increased tissue enzyme levels and reduced glycogen levels in muscle, which was further improved when AT2221 was co-administered with ATB200. Amicus Therapeutics is currently conducting a global Phase 1/2 study (ATB200-02) to evaluate the safety, tolerability, pharmacokinetics (PK) and pharmacodynamics of ATB200/AT2221.

### **About Pompe Disease**

Pompe disease is an inherited lysosomal storage disorder caused by deficiency of an enzyme called acid alpha-glucosidase (GAA). Reduced or absent levels of GAA lead to the accumulation of the substrate glycogen in the lysosomes of muscles and other tissues. Progressive accumulation of glycogen is believed to lead to the morbidity and mortality associated with Pompe disease, including muscle weakness and respiratory insufficiency.

### **About Amicus Therapeutics**

Amicus Therapeutics (Nasdaq: FOLD) is a global biotechnology company at the forefront of therapies for rare and orphan diseases. The Company has a robust pipeline of advanced therapies for a broad range of human genetic diseases. Amicus' lead programs in development include the small molecule pharmacological chaperone migalastat as a monotherapy for Fabry disease, SD-101 for Epidermolysis Bullosa (EB), as well as novel enzyme replacement therapy (ERT) and biological products for Fabry disease, Pompe disease, and other rare and devastating diseases.

(1)Johnson, *et. al*, **WORLD Symposium 2017**, First-in-human preliminary pharmacokinetic and safety data on a novel recombinant acid- $\alpha$ -glucosidase, ATB200, co-administered with the pharmacological chaperone, AT2221, in ERT-experienced Pompe patients

### **Forward-Looking Statements**

This press release contains “forward- looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995, including statements relating to encouraging preliminary data from a global Phase 1/2 study to investigate ATB200/AT2221 for the treatment of Pompe and the potential



implications on these data for the future advancement and development of ATB200/AT2221. Words such as, but not limited to, “look forward to,” “believe,” “expect,” “anticipate,” “estimate,” “intend,” “confidence,” “encouraged,” “potential,” “plan,” “targets,” “likely,” “may,” “will,” “would,” “should” and

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“could,” and similar expressions or words identify forward-looking statements. The forward looking statements included in this press release are based on management’s current expectations and belief’s which are subject to a number of risks, uncertainties and factors, including that the preliminary data based on a small patient sample and reported before completion of the study will not be predictive of future results, that results of additional preliminary data or data from the completed study or any future study will not yield results that are consistent with the preliminary data presented, that the Company will be not able to demonstrate the safety and efficacy of ATB200/AT2221, that later study results will not support further development, or even if such later results are favorable, that the Company will not be able to successfully complete the development of, obtain regulatory approval for, or successfully commercialize ATB200/AT2221. In addition, all forward looking statements are subject to the other risks and uncertainties detailed in our Annual Report on Form 10-K for the year ended December 31, 2016 and Quarterly Report on 10-Q for the Quarter ended March 31, 2017. As a consequence, actual results may differ materially from those set forth in this press release. You are cautioned not to place undue reliance on these forward looking statements, which speak only of the date hereof. All forward looking statements are qualified in their entirety by this cautionary statement and we undertake no obligation to revise this press release to reflect events or circumstances after the date hereof.

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