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Amicus Therapeutics Announces Positive Functional Data from 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, N.J., May 15, 2017 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD) today announced 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.

- 1 **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.
- 1 **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
Baseline Mean (SD)	383 (103)	4.4 (3.1)	11.0 (7.7)	7.5 (3.5)	51 (17)	35.4 (11.3)	69.5 (21.2)
Change from Baseline (SD)	+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
Baseline Mean (SD)	432 (68)	3.9 (0.6)	8.9 (0.9)	6.9 (0.8)	51 (27)	45.5 (27.6)	57.5 (9.2)
Change from Baseline (SD)	+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 biologic products for Fabry disease, Pompe disease, and other rare and devastating diseases.

¹[Johnson, et. al. *WORLDSymposium 2017*. First-in-human preliminary pharmacokinetic and safety data on a novel recombinant acid-α-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 "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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