October 4, 2017

Amicus Therapeutics Announces Additional Positive Data in Pompe Disease Phase 1/2 Study at World Muscle Society

Mean Six-Minute Walk Distance Improved in ERT-Naive Patients (+42 Meters at 6 Months, +75 Meters at 9 Months) and ERT-Switch Patients (+35 Meters at 6 Months, +37 Meters at 9 Months)

Persistent & Durable Reductions in Key Disease Biomarkers

Pulmonary Function Generally Improved or Remained Stable at 6 and 9 Months

Very Low Number (< 1%) of Infusion Associated Reactions Observed After 400+ Infusions

Conference Call at 8:30am ET

CRANBURY, N.J., Oct. 04, 2017 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD) today announced additional positive results from a global Phase 1/2 clinical study (ATB200-02) to investigate ATB200/AT2221 in patients with Pompe disease, an inherited lysosomal storage disorder caused by an enzyme deficiency that leads to accumulation of glycogen (disease substrate) in cells. Consistent with previous results, patients who completed six months of treatment with ATB200/AT2221 showed improvements in six-minute walk test (6MWT) distance and other measures of motor function, stability or increases in forced vital capacity (FVC), and further reductions in biomarkers of muscle damage and disease substrate, with consistent results reported in initial patients who completed nine months of treatment. These clinical results were featured at the 22nd International Congress of the World Muscle Society in a late-breaker poster[1]. With these data, Amicus plans to continue a series of collaborative discussions with regulators in the US and EU, and expects to provide an update in the first half of 2018.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics stated, "These remarkable data from our Pompe clinical study of ATB200/AT2221 have once again exceeded our expectations. The consistency, durability and magnitude of the functional outcomes align with significant and continued reductions in key biomarkers of muscle damage and disease substrate, across patients, across cohorts and over significant periods of time. Taken together the strength of these results suggest the effect of ATB200/AT2221 may be very clinically meaningful for people living with Pompe disease. We are committed to working collaboratively with regulators to determine the fastest regulatory pathways that may be available to bring this new treatment paradigm to as many patients living with Pompe disease globally, as quickly as possible."

Mark Roberts, MD, Department of Neurology, Salford Royal NHS Foundation Trust and Principal Investigator in the ATB200-02 study stated, "I believe that the results from this Phase 1/2 clinical study show striking improvements in functional measures and key biomarkers during the first six months of treatment, in addition to continued, further benefit out to nine months. I am especially intrigued by the six-minute walk distance and other motor function tests in the ERT-switch patients who historically have declining motor function following two or more years of treatment. These clinical data are compelling and suggest that ATB200/AT2221 has the potential to shift the treatment paradigm for Pompe disease."

ATB200-02 Full Study Data - Highlights in ERT-Switch and ERT-Naive Patients

Safety, Tolerability & Pharmacokinetics (PK) (n=20)
Safety and tolerability data in all 20 patients reflect a maximum of 72 weeks of treatment. To date, adverse events have been generally mild and transient. Importantly, ATB200/AT2221 has resulted in a low rate of infusion-associated reactions (IARs) following 400+ infusions (three events of IARs in two patients; < 1% of all 400+ infusions with an IAR). As previously reported, the clinical pharmacokinetic profile has been consistent with previously reported preclinical data.

Pharmacodynamic (PD) Data on Muscle Damage and Disease Substrate Biomarkers (n=20)
Treatment with ATB200/AT2221 resulted in reductions in key disease biomarkers across all patient cohorts after up to 58 weeks and continue to suggest a positive effect on muscle cells.

- **Muscle damage biomarkers**: Creatine kinase (CK) enzyme, alanine aminotransferase (ALT), and aspartate aminotransferase (AST), are biomarkers indicative of muscle damage in Pompe disease. Mean reductions from baseline were approximately 25-35%, 5-25% and 40-55% for the ambulatory ERT-switch (n=11), non-ambulatory ERT-switch (n=4) and ERT-naïve (n=5) patients, respectively.

- **Disease substrate biomarker**: Urine hexose tetrasaccharide (Hex4) is a biomarker of glycogen build-up. Mean reductions from baseline were approximately 40%, 35% and 55% for the ambulatory ERT-switch (n=11), non-ambulatory ERT-switch (n=4) and ERT-naïve (n=5) patients, respectively.

Functional Outcomes (n=18)
Data on 6-month functional outcomes are available for 18 of the 20 patients enrolled (one patient dropped out of the extension study due to travel burden and family considerations, while month 6 assessments are pending in one patient due to an incomplete visit). Muscle function improved in 16 patients and was stable in two patients at month 6. Muscle function improved in 10 out of 10 patients with available data at month 9.

- **Muscle Function:**
  - **Motor function (n=14):** Six-minute walk test (6MWT) distance, a primary measure of motor function in Pompe disease patients, improved in both ERT-naive and ERT-switch patients and was durable to month 9. ERT-naive patients showed mean increases of 42 meters at month 6 (n=5) and 75 meters at month 9 (n=2). ERT-switch patients showed mean increases of 35 meters at month 6 (n=9) and 37 meters at month 9 (n=8). Other motor function tests showed mean improvements consistent with 6MWT distance.

  - **Muscle Strength (n=4):** All four non-ambulatory ERT-switch patients showed improvements in upper extremity strength (elbow and shoulder) from baseline to month 6, as measured by quantitative muscle testing (QMT) and manual muscle testing (MMT).

- **Pulmonary Function:** Forced vital capacity (FVC), the primary measure of pulmonary function in Pompe disease, improved in ERT-naïve patients, with mean absolute change in percent predicted FVC of +4.2% at month 6 (n=5) and +5.0% at month 9 (n=2). FVC was generally stable in ERT-switch patients with mean absolute change in percent predicted FVC of -1.0% at month 6 (n=8) and -2.0% at month 9 (n=7). Other pulmonary tests included maximal inspiratory pressure (MIP), a measure of inhalation, and maximal expiratory pressure (MEP), a measure of exhalation. MIP and MEP were generally stable or increased in both ERT-naïve and ERT-switch patients.

Summary of Functional Outcomes from Baseline to Month 6

Cohort 1 ERT-Switch Patients: Functional Outcomes on ATB200/AT2221 from Baseline to Month 6 and 9

	Motor Function Tests						Pulmonary Function Tests		
	6MWT (m)	**4 Stair Climb (sec)**	**Timed up and go (sec)**	**10m walk (sec)**	**Gowers#**	**GSGC Score**	**FVC (%)**	**MIP**	**MEP**
Baseline Mean (SD) (n=10)	**397.2 (96.8)**	**4.1** (2.7)	**10.5** (6.6)	**7.4** (3.0)	**7.9** (2.8)	**12.6** (4.8)	**52.6** (14.7)	**35.7** (11.0)	**72.6** (32.6)
Change at Month 6 (SD) (n=9)	**+35.3** (40.1)	**-1.0** (1.2)	**-2.2** (3.4)	**-0.3** (1.6)	**-2.2** (2.0)	**-0.8** (3.0)	**-1.0** (4.2)	**+0.9** (4.5)	**+20.3** (42.4)
Change at Month 9 (SD) (n=8)	**+37.2** (33.8)	**-0.9** (1.3)	**-0.6** (2.5)	**0.1** (1.3)	**-2.1** (1.3)	**-0.9** (3.5)	**-2.0** (3.6)	**-1.4** (2.7)	**+31.1** (39.3)

Cohort 3 ERT-Naïve Patients: Functional Outcomes on ATB200/AT2221 from Baseline to Month 6 and 9

	Motor Function Tests						Pulmonary Function Tests		
	6MWT (m)	**4 Stair Climb (sec)**	**Timed up and go (sec)**	**10m walk (sec)**	**Gowers#**	**GSGC Score**	**FVC (%)**	**MIP**	**MEP**
Baseline Mean (SD) (n=5)	**399.5 (83.5)**	**4.2** (1.5)	**9.4** (2.9)	**7.9** (3.0)	**13.9** (11.0)	**12.2** (3.6)	**53.4** (20.3)	**32.6** (18.5)	**60.6** (8.3)
Change at	**+41.8**	**-0.6**	**-1.0**	**-0.7**	**7.9***	**-1.8**	**+4.2**	**+11.0**	**-0.4**

Month 6 (SD) (n=5)	(29.4)	(0.3)	(1.1)	(1.1)	(21.0)	(3.8)	(5.6)	(5.0)	(12.4)
Change at Month 9 (SD) (n=2)	**+74.9** (4.0)	**-0.8** (0.3)	**-1.6** (1.0)	**-1.0** (0.1)	**-1.3** (0.0)	**-4.0** (1.4)	**+5.0** (1.4)	**+1.5** (0.7)	**-1.0** (19.8)

*Median change from baseline was -0.8 and 4/5 had decrease; # N=9 Missing values not obtained due to patient refusal to perform test

Conference Call and Webcast
Amicus Therapeutics will host a conference call and webcast today, October 4, 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 96220532. 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 96220532.

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. Sixteen clinical sites in five countries participated in the ATB200-02 clinical study. The study enrolled a total of 20 patients at 16 participating sites in five countries 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 the enzyme acid alpha-glucosidase (GAA). Reduced or absent levels of GAA leads to accumulation of glycogen in cells, which is believed to result in the clinical manifestations of Pompe disease. Pompe disease can be debilitating, and is characterized by severe muscle weakness that worsens over time. Pompe disease ranges from a rapidly fatal infantile form with significant impacts to heart function to a more slowly progressive, late-onset form primarily affecting skeletal muscle. It is estimated that Pompe disease affects approximately 5,000 to 10,000 people worldwide.

For more information, download our Pompe disease infographic.

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, as well as novel enzyme replacement therapy (ERT) and biologic products for Fabry disease, Pompe disease, and other rare and devastating diseases.

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 June 30, 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.

[1] Roberts, *et. al.,* **22nd International Congress of the World Muscle Society,** First-in-Human Study of ATB200/AT2221 in Patients With Pompe Disease: Interim Results From the ATB200-02 Trial

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