



February 15, 2017

Amicus Therapeutics Presents Important New Scientific Findings and Preclinical Data for Pompe Program at WORLDSymposium™ 2017

Scientific Findings Reveal that Cellular Damage Alters Trafficking for Key Proteins Involved in Muscle Membrane Integrity and Muscle Repair

Preclinical Studies Demonstrate Reversal of Cellular Damage and Significant Improvements in Muscle Strength in GAA Knock-out Mice After Treatment with Amicus Novel Pompe Treatment Paradigm

Additional Phase 1/2 Clinical Data to be Presented at Wednesday Afternoon Poster Session (4:30pm PT)

CRANBURY, N.J. and SAN DIEGO, Feb. 15, 2017 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD), a global biotechnology company at the forefront of rare and orphan diseases, today presented new scientific findings and preclinical data on functional outcomes in an oral presentation and [poster¹](#) at the 13th Annual [WORLDSymposium™](#) in San Diego, CA. 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 in muscles, co-administered with AT2221, a pharmacological chaperone designed to stabilize ERT in circulation.

In previously reported 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. For the first time at *WORLDSymposium*, Hung Do, PhD, Chief Science Officer of Amicus Therapeutics, presented results from preclinical studies that showed ATB200/AT2221 reversed cellular dysfunction and progressively increased muscle strength following every-other-week administration at 20 mg/kg over a five month period in an animal model of Pompe disease (GAA knock-out mice).

Key Scientific Findings

- | Impaired autophagy and other cellular defects lead to accumulation of intracellular vesicles that hinders protein trafficking for many proteins including key proteins that are required for muscle membrane integrity (various members of dystrophin-associated protein complex) and muscle repair (dysferlin) in muscles of *Gaa* knock-out mouse model of Pompe disease
- | Membrane and protein mistrafficking appeared to alter muscle structure which likely contributes towards progressive muscle weakness and persistent disrepair
- | Protein mistrafficking of these key proteins also observed in Pompe patient muscle biopsies

Key Highlights on New Preclinical Data for ATB200/AT2221 in GAA Knock-Out Mice

- | ATB200/AT2221 is well-targeted to lysosomes of muscles which led to significant clearance of accumulated glycogen substrate in muscles of *Gaa* knock-out mice
- | ATB200/AT2221 also appeared to restore important cellular pathways and muscle structure that were significantly altered
- | ATB200/AT2221 continuously improved functional muscle strength over 5-month study period (as measured by wire hang and grip strength tests)
- | Collectively, the data suggest that improved targeting and stabilization of ATB200 resulted in superior glycogen clearance, as well as continual reversal of muscle damage leading to improved muscle function

Dr. Do stated, "Our latest preclinical studies give us further confidence that ATB200/AT2221 has been optimally designed for efficient targeting to muscles. The results build upon our prior preclinical studies showing that ATB200/AT2221 not only clears accumulated glycogen but importantly, significantly reversed muscle damage and increased muscle function. These studies have also been invaluable for gaining important insights into the role of protein mistrafficking as contributing factors towards muscle weakness and disrepair in Pompe disease, and the potential for ATB200/AT2221 to reverse cellular dysfunction to restore muscle structure and increase muscle strength."

Grace K. Pavlath, Ph.D., Senior Vice President, Scientific Program Director of the Muscular Dystrophy Association stated,

"The scientific findings and preclinical data presented today are profound and shed new light on questions about the underlying cause of muscle damage and weakness in Pompe patients. Furthermore, these results provide a window into a potential underlying link among key muscular dystrophies, such as Pompe, Limb Girdle, and Duchenne. Amicus has been a pioneer in advancing the scientific understanding of Pompe disease and in developing next-generation therapies for patients."

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.

[¹Do, et. al. **WORLD Symposium 2017**. ATB200/AT2221 Cleared Accumulated Glycogen and Reversed Cellular Dysfunction to Increase Functional Muscle Strength in Mouse Model of Pompe Disease](#)

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 pre-clinical data from a study to investigate ATB200/AT2221 in a knock-out mouse model and the potential implications on these data for the future advancement and development of ATB200/AT2221 in humans. 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 beliefs which are subject to a number of risks, uncertainties and factors, including that the pre-clinical data will not be predictive of future results in humans, and that preliminary human clinical 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 pre-clinical or 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, 2015 and Quarterly Report on 10-Q for the Quarter ended September 30, 2016. As a consequence, actual results may differ materially from those set forth in this press release or the accompanying conference call or webcast. 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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Source: Amicus Therapeutics, Inc

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