



February 10, 2015

Amicus Therapeutics Presents Preclinical Data on Next-Generation Pompe ERT at WORLDSymposium(TM) 2015

Optimized for Efficient Targeting to Multiple Key Muscle Tissues of Disease

Significantly Better Than Approved ERT in Reducing Disease Substrate (Glycogen)

Addition of Chaperone Further Improves Glycogen Reduction

ORLANDO, Fla. and CRANBURY, N.J., Feb. 10, 2015 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD), a biopharmaceutical company at the forefront of therapies for rare and orphan diseases, today highlighted results from preclinical studies of its next-generation Pompe ERT at [WORLDSymposium™2015](#) in Orlando, Florida.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc., stated, "Our goal is to develop a fundamentally better therapy for patients living with Pompe disease. The results presented at WORLDSymposium suggest that we have made significant progress with our Pompe program as we look toward addressing some of the major challenges with the current approved therapy including enzyme activity and stability; targeting and uptake; and tolerability and immunogenicity. These results also validate our internal biologics capabilities to develop and manufacture our own cell line at clinical scale. We look forward to moving our next-generation ERT into the clinic later this year."

Amicus is leveraging its biologics capabilities and CHART™ (ChaperonAdvanced Replacement Therapy) platform to develop a next-generation Pompe ERT. This ERT consists of a uniquely engineered recombinant human acid alpha-glucosidase (rhGAA) enzyme (designated ATB200) with an optimized carbohydrate structure to enhance uptake, administered in combination with a pharmacological chaperone to improve activity and stability. In earlier preclinical studies, ATB200 demonstrated greater tissue enzyme levels and further substrate reduction compared to the current approved ERT for Pompe disease (alglucosidase alfa). Clinical studies^{1,2} of pharmacological chaperones in combination with currently marketed ERTs have established initial human proof-of-concept that a chaperone can stabilize enzyme activity and potentially improve ERT tolerability.

An oral presentation³ and [posters](#)^{3,4} at WORLDSymposium describe updated preclinical results that support clinical development of ATB200 in combination with a chaperone:

- A proprietary cell line and manufacturing processes have been developed to produce ATB200 with optimized carbohydrate structures for efficient cellular uptake and lysosomal targeting
- ATB200 was significantly better than the approved ERT for reducing disease substrate in multiple key muscle tissues in a Pompe animal model (*Gaa* knock-out mice)
- The addition of a pharmacological chaperone further improved glycogen reduction by ATB200
- ATB200 has been successfully scaled up to 250L production, and is on track to enter the clinic in the second half of 2015.

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 in development include the small molecule pharmacological chaperone [migalastat](#) as a monotherapy for Fabry disease, as well as next-generation enzyme replacement therapy (ERT) products for Fabry disease, Pompe disease, and MPS-1.

1. Kishnani, *et al.*, LDN WORLD 2013
2. Doerfler, *et al.*, LDN WORLD 2014
3. Gotschall, *et al.*, WORLDSymposium 2015
4. Lun, *et al.*, WORLDSymposium 2015

Forward-Looking Statements

This press release contains "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, financing plans, 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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Source: Amicus Therapeutics, Inc.

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