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## Amicus Therapeutics Commences Phase 1/2 Study of Novel ERT for Treatment of Pompe Disease

*IND Open and Clinical Sites Being Initiated*

*First Amicus Proprietary Biologic to Enter Clinic*

CRANBURY, N.J., Dec. 22, 2015 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD), a biopharmaceutical company at the forefront of rare and orphan diseases, today announced that its investigational new drug (IND) application, submitted to U.S. Food and Drug Administration (FDA), is now effective which allows Amicus to begin site initiation and enrollment of a Phase 1/2 study of ATB200 in patients with Pompe disease. Amicus intends to seek regulatory authorization to evaluate ATB200 in European patients as well. This novel enzyme replacement therapy (ERT) consists of a uniquely engineered recombinant human acid alpha-glucosidase (rhGAA) enzyme with an optimized carbohydrate structure, administered in combination with a small molecule pharmacological chaperone (AT2221). In preclinical studies, ATB200 was associated with increased tissue enzyme levels and reduced substrate, which was further improved when AT2221 was co-administered with ATB200.

"The start of our Pompe clinical program is a major step forward for people living with Pompe disease and their families. This novel ERT has been developed over the past several years by Amicus scientists and represents the potential for a very differentiated biologic treatment option for patients. There is still so much need in the Pompe community and we are dedicated to this mission. We look forward to seeing data from this study in 2016," stated John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc.

The key features of this Phase 1/2 study include:

- | Open-label, dose-escalation to evaluate the safety, tolerability, pharmacokinetics and pharmacodynamics of intravenous ATB200 co-administered with oral AT2221
- | Subjects in the first cohorts will be adult Pompe patients switched from currently marketed ERT
- | Primary treatment period will be 18 weeks, with all patients eligible to enroll in an open label extension study
- | Dose selection for a Phase 3 clinical study will be informed by the findings from this Phase 1/2 study

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Amicus plans to present additional study design details in early 2016. First patient dosing is expected in early 2016.

### **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 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) products for Fabry disease, Pompe disease, and other lysosomal storage disorders.

### **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 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, 2014 and Form 10-Q for the quarter ended June 30, 2015. 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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