Amicus Therapeutics Presents Preclinical Studies of Chaperone AT3375 for Gaucher Disease

Chaperone-ERT Combination Increases Uptake of Active Enzyme in Disease-Relevant Tissues

CRANBURY, N.J., Feb. 9, 2012 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD), a biopharmaceutical company at the forefront of therapies for rare and orphan diseases, presented preclinical studies of AT3375 as a monotherapy and in combination with enzyme replacement therapy (ERT) for Gaucher disease during the 8th Annual Lysosomal Disease Network WORLD Symposium (LDN WORLD).

AT3375 is a next-generation, small molecule pharmacological chaperone that targets the glucocerebrosidase (GCase) enzyme deficient in Gaucher disease, a mechanism with the potential to address Gaucher and Parkinson's. AT3375 was the lead compound selected from a series of chaperones designed by Amicus to improve upon the properties of AT2101 (isofagomine tartrate), a first-generation chaperone that Amicus was originally developing for Gaucher disease.

The oral presentation at LDN WORLD was titled, "Preclinical Results Exploring the Use of Pharmacological Chaperone AT3375 Alone and in Combination with Recombinant Human Beta-Glucosidase for Gaucher Disease." Key highlights were as follows:

- In combination with ERT, AT3375 binds to and stabilizes the recombinant enzyme, minimizing its thermal denaturation and loss of activity *in vitro.*
- AT3375 co-administered with ERT increased active enzyme in plasma and Gaucher-disease relevant tissues (liver, spleen and lung) compared to AT2101 co-administered with ERT. In addition, ERT co-administered with either chaperone increased enzyme activity compared to ERT alone.
- When used as a monotherapy, AT3375 increased GCase activity in patient-derived cells with greater potency compared to AT2101.
- In mouse models, higher levels of AT3375, orally administered as a monotherapy, were present in the brain and the compound was cleared more rapidly than AT2101. AT3375 increased brain GCase levels with greater potency compared to AT2101.

Recombinant GCase ERTs (imiglucerase and velaglucerase alfa) are current standard of therapy for Gaucher disease, however, they are unable to cross the blood-brain-barrier to address CNS aspects of the disease. These ERTs are also highly unstable outside the lysosome, and could potentially benefit from the presence of a small molecule chaperone that binds to and stabilizes recombinant enzyme.

David J. Lockhart, Chief Scientific Officer of Amicus Therapeutics said, "Our initial preclinical studies of AT3375 demonstrate the potential for chaperone-ERT co-administration to improve the properties of ERTs for Gaucher disease. These results also suggest that AT3375 can increase brain GCase levels, supporting our investigation of AT3375 to address Parkinson's disease by targeting GCase."

Gaucher disease is a lysosomal storage disorder caused by inherited genetic mutations in the GBA gene, which result in deficient activity of the GCase enzyme and lysosomal accumulation of glucocerebroside inside certain cells. Mutations in the GBA1 gene that encodes for the GCase enzyme are also the most common genetic risk factor known for Parkinson's disease. Gaucher disease affects an estimated 8,000 to 10,000 people worldwide. Gaucher patients have an estimated 20-fold increased risk of developing Parkinson's disease, and an estimated 5% to 10% of the diagnosed Parkinson's population are carriers of Gaucher disease.

Michael J. Fox Foundation Partnering Program

AT3375 has been evaluated in preclinical studies for Gaucher disease and is currently in IND-enabling studies for Parkinson's disease. The preclinical studies for Parkinson's disease are funded in part by a grant awarded by the Michael J. Fox Foundation (MJFF).

MJFF is currently piloting a Partnering Program designed to proactively showcase promising research results in the MJFF portfolio for funders who may wish to invest in their continued development. In the first quarter of 2012, featured MJFF-supported projects will include preclinical work from Amicus for the development of small molecule drugs aimed at reducing the

accumulation of the protein alpha-synuclein in the brain, which is a key pathological hallmark of Parkinson's.

About Amicus Therapeutics

Amicus Therapeutics (Nasdaq:FOLD) is a biopharmaceutical company at the forefront of developing therapies for rare and orphan diseases. The Company is developing orally-administered, small molecule drugs called pharmacological chaperones, a novel, first-in-class approach to treating a broad range of diseases including lysosomal storage disorders and diseases of neurodegeneration. Amicus' lead program migalastat HCl is in Phase 3 for the treatment of Fabry disease.

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 and the timing and reporting of results from preclinical and clinical trials evaluating Amicus' candidate drug products. Words such as, but not limited to, "look forward to," "believe," "expect," "anticipate," "estimate," "intend," "plan," "targets," "likely," "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 the potential goals, progress, timing and results of preclinical 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. 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, 2010. 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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