Amicus Therapeutics Provides Positive Data Update from Phase 2 Long-Term Extension Study of Amigal™ for Fabry Disease

Company also announces encouraging new preclinical data from Chaperone-ERT Co-Administration and Neurodegenerative Disease Studies

CRANBURY, N.J., Feb. 16, 2011 /PRNewswire/ -- Amicus Therapeutics (Nasdaq: FOLD) today announced that additional positive data from the ongoing Phase 2 extension study of its investigational drug Amigal™ (migalastat HCl) for Fabry disease will be presented at the Lysosomal Disease Network WORLD Symposium in Las Vegas, Nevada, February 16-18th, 2011. In addition, the Company announced that it will present encouraging data from its preclinical studies evaluating the co-administration of pharmacological chaperones with enzyme replacement therapy (ERT) in Fabry disease, as well as from preclinical studies examining the use of pharmacological chaperones for the treatment of genetically defined subpopulations of Parkinson's disease and Alzheimer's disease.

Preliminary Data Update from Phase 2 Long-Term Extension Study of Migalastat HCl for Fabry Disease

Twenty-six subjects completed either 12 or 24 weeks of treatment with migalastat HCl during the initial Phase 2 studies and 23 subjects enrolled in a separate, long-term extension study designed to evaluate the long-term safety and efficacy of migalastat HCl. Over the course of the initial Phase 2 and extension studies, 15 subjects have been treated with migalastat HCl for more than 3 years and 7 subjects have been treated with migalastat HCl for more than 4 years. Seventeen subjects continue to receive treatment in the ongoing extension study.

During the course of the extension study, treatment with migalastat HCl has continued to be generally well tolerated, with no drug-related serious adverse events. The most common adverse events have been headache, arthralgia, diarrhea and fatigue.

Renal function continues to be evaluated by two measures in the extension study, estimated glomerular filtration rate (eGFR) and 24-hour urine protein. Preliminary data indicate that eGFR has remained stable out to 3-4 years for all subjects continuing in the extension study and the average annual rate of change in eGFR in subjects identified as responders to migalastat HCl, excluding hyperfiltrators, was +1.6 mL/min/1.73m2. Additionally, reduced 24-hour urine protein continues to be observed in multiple subjects identified as responders to migalastat HCl, with a mean 21% and median 34% reduction from baseline in this group of subjects.

Co-administration with ERT in Fabry Disease and Pompe Disease

Amicus previously reported promising preclinical data demonstrating that the co-administration of a pharmacological chaperone with ERT has the potential to address key limitations of ERT. The addition of a pharmacological chaperone has been shown to prevent the loss of activity of ERT in the circulation, increase tissue uptake, and increase substrate reduction in multiple disease-relevant tissues. Preclinical proof of concept has been established for Fabry disease and Pompe disease.

The Company will present a review of new and historical data from preclinical studies of migalastat HCl co-administered with ERT in an animal model of Fabry disease. Amicus and its partner GlaxoSmithKline PLC (GSK) are sponsoring an ongoing Phase 2 study evaluating the co-administration of migalastat HCl with ERT for Fabry disease. Results from this study are expected in the second half of 2011.

In addition, Amicus will present pharmacokinetics and muscle distribution data from a Phase 1 study of AT2220 that support the planned Phase 2 clinical study of AT2220 co-administered with ERT in Pompe patients. The Company expects to initiate this study in the first half of 2011 and to report preliminary results in the second half of 2011. The Company intends to seek U.S. FDA approval to lift the current hold on the AT2220 program as part of its development plan.

Diseases of Neurodegeneration

Amicus is investigating the potential use of pharmacological chaperones for the treatment of genetically defined subpopulations of patients with Parkinson's disease and Alzheimer's disease. Amicus previously reported encouraging results from preclinical studies evaluating the use of a pharmacological chaperone for the treatment of Parkinson's disease. Amicus will present additional data from preclinical studies that evaluated the pharmacological chaperone AT2101 in mouse models of
Parkinson's disease as well as data related to the properties of new compounds, including AT3375. In 2011, the Company expects to complete late-stage preclinical proof of concept studies, including IND-enabling activities, for AT3375. The Amicus Parkinson's Disease program is funded in part by a grant from The Michael J. Fox Foundation (MJFF).

Additionally, Amicus continues to advance its preclinical program evaluating a pharmacological chaperone approach for the treatment of Alzheimer's disease. The Company will present scientific data on the link between lysosomal dysfunction and neurodegeneration, including data related to a preclinical program evaluating a pharmacological chaperone approach for the treatment of Alzheimer's disease. The Company expects to continue preclinical proof of concept studies during 2011. The Amicus Alzheimer's Disease program is funded in part by a grant from the Alzheimer's Drug Discovery Foundation (ADDF).

About Amigal

On October 29, 2010, Amicus announced a definitive agreement with GlaxoSmithKline PLC (GSK) to develop and commercialize Amigal (migalastat HCl), currently in Phase 3 for the treatment of Fabry disease as a monotherapy. Under the terms of the agreement, GSK received an exclusive worldwide license to develop, manufacture and commercialize migalastat HCl. GSK and Amicus are also investigating Amigal as a treatment for Fabry disease when co-administered with ERT and have commenced a Phase 2 study as noted above.

The Phase 3 study (Study 011) of migalastat HCl is ongoing and patients are being enrolled at 36 investigational sites worldwide. A majority of the planned 60 subjects have been enrolled in the study. The Company expects to complete enrollment in the first half of 2011 and to report top line results from this study in the second half of 2011.

Amicus and GSK intend to commence an additional Phase 3 study (Study 012) in the first quarter of 2011. Study 012 will be an 18-month, randomized, open-label study comparing migalastat HCl to enzyme replacement therapy (ERT) in approximately 60 subjects. The primary outcome of efficacy will be renal function as measured by glomerular filtration rate (GFR).

About Fabry Disease

Fabry disease is an inherited lysosomal storage disorder caused by deficiency of an enzyme called alpha-galactosidase A (alpha-Gal A). The role of alpha-Gal A within the body is to break down a complex lipid called globotriaosylceramide (GL-3). Reduced or absent levels of alpha-Gal A activity leads to the accumulation of GL-3 in the affected tissues, including the central nervous system, heart, kidneys, and skin. This accumulation of GL-3 is believed to cause the various symptoms of Fabry disease, including pain, kidney failure, and increased risk of heart attack and stroke.

It is currently estimated that Fabry disease affects approximately 5,000 to 10,000 people worldwide.

About Amicus Therapeutics

Amicus Therapeutics is a biopharmaceutical company focused on developing treatments for rare 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 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 studies 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 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. 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, 2009. 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.

CONTACT:
Amicus Therapeutics
(609) 662-2000
ir@amicustherapeutics.com

SOURCE Amicus Therapeutics

News Provided by Acquire Media