



## **Amicus Therapeutics Provides 2011 Business Outlook and Expected Key Milestones**

### **Significant progress expected to further establish Company as a leader in rare diseases**

Cranbury, NJ, January 11, 2011 – Amicus Therapeutics (NASDAQ: FOLD) today will provide the Company's business outlook and expected key milestones for 2011 at the 29th Annual J.P. Morgan Healthcare Conference.

At the conference, Amicus is providing an update on its three key areas of focus: Amigal (migalastat hydrochloride) for the treatment of Fabry Disease, the evaluation of pharmacological chaperones co-administered with ERT, and the investigation of pharmacological chaperones for the treatment of diseases of neurodegeneration. The Company intends to identify key milestones expected in 2011 across these three areas, including results from the following studies:

- Phase 3 study of Amigal for Fabry Disease in 2H11
- Phase 2 study of Amigal co-administered with enzyme replacement therapy (ERT) for Fabry Disease in 2H11
- Phase 2 study of AT2220 co-administered with ERT for Pompe Disease in 2H11
- Late-stage preclinical proof of concept studies of AT3375 for Parkinson's Disease, including completion of additional IND-enabling activities, in 2H11.

"2011 promises to be a transformational year for Amicus. This is an exciting time in the rare disease field and we are uniquely positioned to develop new therapies for patients and to build value for our shareholders," said John F. Crowley, Chairman and CEO of Amicus. "This year we intend to achieve multiple milestones, led by our anticipated Phase 3 results for Amigal in Fabry Disease, which we expect to achieve in collaboration with our new partner, GSK Rare Diseases. In addition, we intend to move forward with Phase 2 studies evaluating chaperones co-administered with enzyme replacement therapy (ERT) in both Fabry and Pompe diseases. Finally, we expect important progress in our preclinical programs investigating the use of pharmacological chaperones in genetically defined sub-populations of Parkinson's disease and Alzheimer's disease. "

### **Financial Guidance**

The Company expects to begin 2011 with a cash balance of approximately \$100 million and to spend between \$45 and \$55 million on 2011 operating expenses (net of cost sharing and milestones related to GSK collaboration). The current cash position, including anticipated payments from GSK in connection with the collaboration, is expected to be sufficient to fund the Company's operations and capital expenditure requirements through the anticipated commercial launch of Amigal in the United States.

In 2011, the Company intends to evaluate additional business development opportunities to further build shareholder value. The Company indicates that it is actively exploring a range of opportunities with multiple potential partners.

### **Amigal (migalastat hydrochloride) for the treatment of Fabry Disease**

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. Under the terms of the agreement, GSK received an exclusive worldwide license to develop, manufacture and commercialize migalastat HCl. Additionally, as part of the agreement, GSK and Amicus also intend to advance clinical studies exploring the co-administration of migalastat HCl with ERT for the treatment of Fabry disease.

The Phase 3 study (Study 011) of migalastat HCl remains the Company's number one priority. Study 011 is ongoing and patients are being enrolled at 36 investigational sites worldwide. A majority of the planned 60 patients 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).

## Chaperone-ERT Combination Therapy

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. Preclinical proof of concept has been established for Fabry disease and Pompe disease.

Amicus and GSK intend to initiate a Phase 2 study with migalastat HCl co-administered with ERT for Fabry disease. This open-label Phase 2 study to investigate drug-drug interactions between migalastat HCl and ERT for Fabry disease is planned to commence in the first quarter of 2011 and results are expected in the second half of 2011.

Additionally, the Company expects to initiate a Phase 2 study with its pharmacological chaperone AT2220 co-administered with ERT for Pompe disease in the first half of 2011 and expects results from this study to be available in the second half of 2011. The Company intends to seek U.S. FDA approval to lift the current partial-hold on the AT2220 program as part of its development plan.

## Diseases of Neurodegeneration

Amicus previously reported encouraging results from preclinical studies evaluating the use of a pharmacological chaperone for the treatment of Parkinson's Disease. Today Amicus will announce that in 2011 it expects to complete late stage preclinical proof of concept studies, including IND-enabling activities, for its pharmacological chaperone molecule AT3375, which is in development for the treatment of Parkinson's Disease. The Amicus Parkinson's Disease program is funded in part by a grant from The Michael J. Fox Foundation (MJFF).

Additionally, Amicus is reporting today that it continues to advance its 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 Amicus Therapeutics

Amicus Therapeutics is developing orally-administered, small molecule drugs called pharmacological chaperones, a novel, first-in-class approach to treating a broad range of rare 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, the timing and reporting of results from preclinical studies and clinical trials evaluating Amicus' candidate drug products, the projected cash position for the Company, including achievement of development and commercialization milestone payments and sales royalties under our collaboration with GlaxoSmithKline, and business development and other transactional activities that seek to strengthen the Company's financial position. 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. 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, including achievement of development and commercialization milestone payments and sales royalties under our collaboration with GlaxoSmithKline. Additionally, with respect to statements relating to potential business development opportunities and other transactions that seek to strengthen our financial position, we may not be successful in identifying suitable collaborators, establishing and implementing such collaborations or completing other transactions that could improve our financial position. 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.