Amicus Therapeutics Announces First Quarter 2010 Financial Results and Product Pipeline Advancements

Recent Positive Phase 2 Extension study data for Amigal(TM) support confidence in Phase 3 program in Fabry; Focus on completing enrollment for Phase 3 U.S. Registration by year end; Preliminary Phase 3 results expected mid-2011

CRANBURY, N.J., May 6, 2010 /PRNewswire via COMTEX News Network/ -- Amicus Therapeutics (Nasdaq: FOLD) today announced financial results for the quarter ended March 31, 2010 and reviewed the progress on its product development pipeline including an update that the Company does not plan to advance AT2220, its investigational drug AT2220 (1-deoxynojirimycin HCl) for the treatment of Pompe disease, as a monotherapy at this time.

First Quarter Highlights:

- Amicus presented positive data from its Phase 2 extension study of Amigal(TM) (migalastat HCl) for Fabry disease and encouraging preclinical data relating to its programs in Parkinson's disease and the combination use of chaperones and enzyme replacement therapies;
- The Company completed an $18.5 million Registered Direct Offering of common stock, (net proceeds of approximately $17.1 million) further strengthening its financial position;
- The Alzheimer's Drug Discovery Foundation (ADDF) provided Amicus a grant for preclinical research on the use of pharmacological chaperones in the treatment of Alzheimer's disease; and
- Amicus' Board of Directors named the Company's Chief Executive Officer, John F. Crowley, Chairman of the Board, and Donald J. Hayden, Jr., Lead Independent Director.

John F. Crowley, Chairman and CEO of Amicus Therapeutics stated, "We began 2010 with positive momentum and are very pleased with our progress throughout the first quarter. Importantly, we further strengthened our financial position and focused additional resources on the solid execution of our global Amigal Phase 3 program."

First Quarter Financials Summary

As of March 31, 2010, Amicus held $81.4 million of cash, cash equivalents, and marketable securities.

For the three months ended March 31, 2010, Amicus reported a net loss of $13.2 million, or $0.54 per share attributable to common stockholders, compared to a net loss of $12.5 million, or $0.55 per share attributable to common stockholders for the same period in 2009.

Pipeline Overview:

Chaperone Monotherapy Programs

Amigal(TM) (migalastat HCl) for the Treatment of Fabry Disease

In February 2010 at the Lysosomal Disease Network WORLD Symposium, Amicus announced additional preliminary data from the ongoing extension study with its lead product candidate, migalastat HCl. These data focused on renal function as evaluated by estimated glomerular filtration rate (eGFR) and proteinuria. At the conference, Amicus presented data indicating that eGFR remained stable out to 2-3 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 +2.0 mL/min/1.73m2. Additionally, trends of reduced proteinuria continued to be observed in subjects identified as responders to migalastat HCl.

Twenty-three of the original 26 subjects who completed Phase 2 studies continued to receive treatment in a voluntary 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, fifteen subjects have been treated with migalastat HCl for approximately 2-3 years and eight subjects have been treated with migalastat HCl for more than 3 years. Nineteen subjects continue to receive treatment in the ongoing extension study.

The Phase 3 U.S. registration study (Study 011) of migalastat HCl remains the Company's number one priority. The Company plans to conduct the study in approximately 40 sites worldwide and to complete enrollment by the end of 2010. The Company expects to have preliminary results from this study in mid-2011.

As previously announced, Amicus expects to commence an additional Phase 3 study (Study 012) before year end. Study 012, a registration trial for approval in the European Union, 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).

AT2220 (1-deoxynojirimycin HCl) for the Treatment of Pompe Disease

In June 2008, the Company announced the commencement of a Phase 2 clinical trial of AT2220 in adults with Pompe disease based on data from both preclinical and Phase 1 studies. In February 2009, the Company announced it had suspended enrollment after two patients enrolled in the trial experienced serious adverse events that were probably related to treatment with AT2220. The AT2220 Investigational New Drug application (IND) was subsequently placed on clinical hold by the U.S. Food and Drug Administration (FDA).

As previously announced, the Company completed a thorough evaluation of all data from these two subjects and additional preclinical studies of AT2220. Based on these data, the Company proposed a Phase 1 study to FDA in order to further evaluate the pharmacokinetics of AT2220 in muscle, the key target tissue in Pompe disease. The FDA agreed to Amicus' proposal for the Phase 1 study and subsequently converted the clinical hold of AT2220 to a partial hold to allow the conduct of this study. The Company initiated this Phase 1 study in September 2009.

The Company announced today the results of the open-label single dose Phase 1 study with AT2220 for the treatment of Pompe disease. The primary objective of this study was to evaluate the pharmacokinetics of AT2220 in muscle tissue in healthy adult subjects. The results of this study indicated that AT2220 was well tolerated with no serious adverse events reported. The pharmacokinetic analysis demonstrated that AT2220 was cleared relatively slowly from muscle tissue. Based on the collective data from this trial and previously completed preclinical and clinical studies of AT2220, the Company has decided not to advance AT2220 as a monotherapy for Pompe disease at this time and plans to focus on the development of AT2220 in combination with enzyme replacement therapy.

Chaperone-ERT Combination Therapy Programs

Amicus also recently presented new data from preclinical studies that evaluated the combination use of migalastat HCl with ERT and AT2220 with ERT in mouse models of Fabry and Pompe disease, respectively, at the Lysosomal Disease Network WORLD Symposium. These preclinical studies of both combinations demonstrated that co-administration of the chaperone with ERT resulted in prolonged half-life of ERT in the circulation, increased enzyme activity in cells and greater substrate reduction in target tissues compared to that seen with ERT alone. Amicus has also completed promising preclinical in vitro studies of its chaperone Plicera**(TM)** (afegostat tartrate) in combination with ERT for Gaucher disease.

Based on continued positive preclinical data, the Company plans to initiate a Phase 2 study with migalastat HCl in combination with ERT for Fabry disease before the end of 2010. In addition, the Company is evaluating options for clinical development of AT2220 and ERT for Pompe disease and afegostat tartrate and ERT for Gaucher disease.

John F. Crowley, Chairman and CEO of Amicus Therapeutics commented, "We continue to be enthusiastic about the expansion of our technology platform with our chaperone-ERT combination therapy programs in Fabry, Pompe and Gaucher, along with our advancements in Parkinson's and Alzheimer's disease. Together, with our Amigal late-stage monotherapy program, we are very pleased with the development of both our rare disease and CNS franchises."

Neurodegenerative Diseases

Amicus presented data from preclinical studies that evaluated the chaperone AT2101 in mouse models of Parkinson's disease at the Lysosomal Disease Network WORLD Symposium. The studies demonstrated that treatment with AT2101 increased the activity of beta-glucocerebrosidase (GCase), prevented accumulation of alpha-synuclein in the brain and improved motor function as assessed in various behavioral tests. At that time, the Company also announced that new compounds have been identified that improve on the properties of AT2101 and expand the range of doses and regimens that show motor improvement in mouse models of the disease.

Amicus previously announced that its second preclinical pharmacological chaperone program for neurodegenerative diseases is for the treatment of Alzheimer's disease. Recently, Amicus was awarded a grant of $210,300 from the Alzheimer's Drug Discovery Foundation to evaluate a novel pharmacological chaperone approach for Alzheimer's disease. The new grant from the ADDF will fund preclinical studies to evaluate the use of pharmacological chaperones in the treatment of Alzheimer's disease. Additionally, Amicus continues to develop other pharmacological chaperone approaches for the treatment of

Alzheimer's disease.

2010 Financial Guidance

As previously reported, the Company expects to spend a total of $40 to $50 million on 2010 operating expenses. The current cash position is expected to be sufficient to fund operations and capital expenditure requirements into the second half of 2011.

Conference Call and Webcast

Amicus Therapeutics will host a conference call and webcast today, Thursday, May 6, 2010, at 5:00 P.M. EDT to review financial results and provide a corporate update. Interested participants and investors may access the conference call at 5 p.m. EDT by dialing 877-303-5859 (U.S./Canada) or 678-224-7784 (international). A telephonic replay of the call will be available for seven days beginning at 8 p.m. EDT. Access numbers for this replay are 800-642-1687 (U.S./Canada) and 706-645-9291 (international); participant code 70672340.

An audio webcast can also be accessed via the investor section of the Amicus Therapeutics Web site at www.amicustherapeutics.com under Investors: Events and Presentations. Web participants are encouraged to go to the Web site 15 minutes prior to the start of the call to register, download and install any necessary software. After the live webcast, an audio webcast replay will remain available in the Investors section of the Amicus Therapeutics Web site for 30 days.

Amicus' press releases are available at www.amicustherapeutics.com.

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 diseases including lysosomal storage disorders and neurodegenerative diseases. Amicus' lead program is in Phase 3 for the treatment of Fabry disease.

Forward-Looking Statements

This press release contains, and the accompanying conference call will contain, "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," "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 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, 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.

Table 1

Amicus Therapeutics, Inc.
(a development stage company)
Consolidated Statements of Operations
(Unaudited)
(In thousands, except share and per share amounts)

	Three Months Ended March 31,		Period from February 4, 2002 (inception) to March 31,
	2009	2010	2010
Revenue:			
Research revenue	$3,912	$-	$31,108
Collaboration revenue	694	-	50,000
Total revenue	4,606	-	81,108
Operating Expenses:			
Research and development	11,875	8,889	184,611
General and administrative	5,195	3,925	81,634
Restructuring charges	-	-	1,522
Impairment of leasehold improvements	-	-	1,030
Depreciation and amortization	505	536	6,956
In-process research and development	-	-	418
Total operating expenses	17,575	13,350	276,171
Loss from operations	(12,969)	(13,350)	(195,063)
Other income (expenses):			
Interest income	526	53	13,810
Interest expense	(29)	(83)	(2,008)
Change in fair value of warrant liability	-	204	(250)
Other expense	-	-	(1,116)
Loss before tax benefit	(12,472)	(13,176)	(184,627)
Benefit from income taxes	-	-	695
Net loss	(12,472)	(13,176)	(183,932)
Deemed dividend	-	-	(19,424)
Preferred stock accretion	-	-	(802)
Net loss attributable to common stockholders	$(12,472)	$(13,176)	$(204,158)
Net loss attributable to common stockholders per common share - basic and diluted	$(0.55)	$(0.54)	
Weighted-average common shares outstanding - basic and diluted	22,613,850	24,289,422	

See accompanying notes to consolidated financial statements

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