



March 3, 2014

## **Amicus Therapeutics Announces Full-Year 2013 Financial Results and Corporate Updates**

*Executing 3-in-3 Strategy to Advance 3 Next-Generation ERTs into Clinic in Next 3 Years*

*Reiterating FY14 Cash Spend Guidance of \$54-\$59 Million*

CRANBURY, N.J., March 3, 2014 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD), a biopharmaceutical company at the forefront of therapies for rare and orphan diseases, today announced financial results for the full-year ended December 31, 2013. The Company also provided program updates and reiterated full-year 2014 operating expense guidance.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc., stated, "During 2013 we focused on strengthening our biologics business strategy to develop next-generation ERTs for patients with lysosomal storage diseases. Through our purchase of Callidus Biopharma, we have acquired a proprietary Pompe ERT as well as a peptide tagging technology that is complementary to our CHART platform. We believe that these technologies together provide a unique tool set to enhance enzyme activity, increase enzyme uptake into tissues, and potentially address the tolerability and immunogenicity associated with current ERTs. During 2014 we are strongly positioned and well capitalized to execute our 3-in-3 strategy to advance three next-generation ERTs into the clinic in the next three years, with lead programs in Fabry, Pompe and MPS I."

### **Financial Highlights for Full Year Quarter Ended December 31, 2013**

- Cash, cash equivalents, and marketable securities totaled \$82.0 million at December 31, 2013 compared to \$99.1 million at December 31, 2012.
- Total operating expenses decreased to \$64.5 million compared to \$71.3 million for the full-year 2012 primarily due to decreases in clinical development costs on the Fabry monotherapy program.
- Net cash spend was \$47.1 million, within the full-year 2013 guidance range of \$47-53 million.
- Net loss was \$59.6 million, or \$1.16 per share, compared to a net loss of \$48.8 million, or \$1.07 per share, for the full-year 2012.

### **2014 Financial Guidance**

Cash, cash equivalents, and marketable securities totaled \$82.0 million at December 31, 2013 compared to \$99.1 million at December 31, 2012. The Company's balance sheet was strengthened in the fourth quarter of 2013 with a \$15.0 million equity financing and a \$25.0 million debt financing under which \$15.0 million was drawn and \$10.0 million remains available. Amicus continues to expect full-year 2014 net cash spend between \$54 million and \$59 million. The current cash position is projected to fund operations into the second half of 2015.

### **Program Updates**

Amicus owns exclusive global rights to its next-generation ERTs, as well as all applications of its Chaperone-Advanced Replacement Therapy (CHART™) and enzyme targeting technology platforms. In each CHART program, a unique pharmacological chaperone is designed to bind to and stabilize a specific therapeutic enzyme in its properly folded and active form. Through its purchase of Callidus Biopharma Amicus has also acquired a differentiated peptide tagging technology that can be used to uniquely engineer bio-better ERTs. These platform technologies provide a complementary tool set to design next-generation therapies for enhanced tissue uptake of active enzyme, greater lysosomal activity, more reduction of substrate, and potentially address the tolerability and immunogenicity associated with currently marketed ERTs.

### **Next-Generation ERT for Pompe Disease**

Amicus is advancing a recombinant human acid alpha-glucosidase (rhGAA) for Pompe disease into late preclinical development. The Company's acquisition of Callidus Biopharma, brings a differentiated Pompe ERT, designated AT-B200, with a unique carbohydrate structure. In preclinical studies AT-B200 has shown superior tissue uptake and activity when compared to current standard of care. This ERT may be further optimized through co-formulation with Amicus' pharmacological chaperone AT2220 to improve enzyme stability and tolerability, and by applying the Company's peptide tagging technology for better targeting.

## Next-Generation ERT for Fabry Disease

In combination with ERT, Amicus' pharmacological chaperone migalastat HCl is designed to bind and stabilize the infused alpha-Gal A enzyme, independent of a patient's genetic mutation. Amicus believes this approach has the potential to benefit all patients with Fabry disease.

Amicus has completed a Phase 2 clinical study ([Study 013](#)) of migalastat HCl co-administered with currently approved ERTs for Fabry disease (Fabrazyme<sup>®</sup> and Replagal<sup>®</sup>) as well as preclinical studies of migalastat HCl co-formulated with a proprietary investigational ERT for Fabry disease (JCR Pharmaceutical Co Ltd's JR-051). JR-051 is a human recombinant alpha-Gal A enzyme that is designed to be biosimilar to Fabrazyme. Positive results from these clinical and preclinical studies demonstrated increased enzyme activity in plasma and greater enzyme uptake into tissues in the presence of the chaperone compared to any of these ERTs alone<sup>1,2</sup>.

In the first half of 2014 Amicus plans to conduct a Phase 1 study to assess the pharmacokinetics of an intravenous formulation of migalastat HCl in healthy volunteers to identify the optimal dose for co-formulation with ERT. In the second half of 2014, Amicus expects to initiate a Phase 1/2 study to evaluate migalastat HCl co-formulated with JR-051. Amicus is currently evaluating its long-term strategy for supplying late-stage clinical and commercial ERT, which may include developing or in-licensing a recombinant alpha-Gal A enzyme comparable to JR-051.

## Next-Generation ERT for MPS I

Amicus is leveraging its CHART platform to develop a proprietary human recombinant alpha-L-iduronidase (rhIDUA) enzyme for MPS I. In support of its development of this next-generation ERT, Amicus has received funding of up to \$250,000 from a private U.S.-based donor that provides medical research grants to find better treatments and cures for rare genetic disorders, including lysosomal storage diseases.

## Migalastat HCl Monotherapy for Fabry Disease

Migalastat HCl monotherapy is being investigated in two ongoing Phase 3 studies for Fabry patients with amenable mutations. Interim 6-month [data](#) from the first ongoing Phase 3 study ([Study 011](#)) have been reported, and 12- and 24-month data from this study are anticipated in the second quarter of 2014. Top-line, 18-month clinical data from the second ongoing Phase 3 study ([Study 012](#)) are expected in the second half of 2014.

## Novel Small Molecules for Parkinson's Disease

In September 2013 Amicus and Biogen Idec entered a multi-year [collaboration](#) to discover of a new class of small molecules that target the glucocerebrosidase (GCase) enzyme for further development and commercialization by Biogen Idec. Biogen Idec is responsible for funding all discovery, development, and commercialization activities. Amicus will be reimbursed for all full-time employees working on the project. In addition Amicus is eligible to receive development and regulatory milestones, as well as modest royalties on global net sales.

## Conference Call and Webcast

Amicus Therapeutics will host a conference call and audio webcast today, March 3, 2014 at 5:00 p.m. ET to discuss full-year 2013 financial results and program updates. Interested participants and investors may access the conference call at 5:00 p.m. ET by dialing 877-303-5859 (U.S./Canada) or 678-224-7784 (international).

An audio webcast can also be accessed via the Investors section of the Amicus Therapeutics corporate web site at <http://www.amicusrx.com>, and will be archived for 30 days. 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. A telephonic replay of the call will be available for seven days beginning at 8:00 p.m. ET today. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); participant code 5821419.

## About Amicus Therapeutics

[Amicus Therapeutics](#) (Nasdaq:FOLD) is a biopharmaceutical company at the forefront of therapies for rare and orphan diseases. The Company is developing novel, first-in-class treatments for a broad range of human genetic diseases, with a focus on delivering new benefits to individuals with lysosomal storage diseases. Amicus' lead programs include the small molecule pharmacological chaperones [migalastat HCl](#) as a monotherapy and in combination with enzyme replacement therapy (ERT) for Fabry disease; and [AT2220 \(duvoglustat HCl\)](#) in combination with ERT for Pompe disease.

## About Chaperone-Advanced Replacement Therapy (CHART)

The Chaperone-Advanced Replacement Therapy (CHART™) platform combines unique pharmacological chaperones with enzyme replacement therapies (ERTs) for lysosomal storage diseases (LSDs). In a chaperone-advanced replacement therapy, a unique pharmacological chaperone is designed to bind to and stabilize a specific therapeutic enzyme in its properly folded and active form. This proposed CHART mechanism may allow for enhanced tissue uptake of active enzyme, greater lysosomal activity, more reduction of substrate, and lower immunogenicity compared to ERT alone. Improvements in enzyme stability may also enable more convenient delivery of next-generation therapies. Amicus is leveraging the CHART platform to develop proprietary next-generation therapies that consist of lysosomal enzymes co-formulated with pharmacological chaperones.

<sup>1</sup>[Bichet, et al., American Society of Human Genetics, November 2012](#)

<sup>2</sup>[Benjamin, et al., Molecular Therapy, April 2012](#)

## 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," "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, 2012. 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		Twelve Months		Period from
	Ended December 31,		Ended December 31,		February 4,
	2012	2013	2012	2013	2002
					(inception)
					To
					Dec. 31,
					2013
Revenue:					
Research revenue	\$ --	\$324	\$11,591	\$363	\$57,856
Collaboration and milestone revenue	--	--	6,820	--	64,382

Total revenue	--	324	18,411	363	122,238
Operating Expenses:					
Research and development	11,047	9,120	50,273	41,944	357,837
General and administrative	4,455	4,605	19,364	18,893	151,506
Restructuring charges	--	1,988	--	1,988	3,510
Impairment of leasehold improvements	--	--	--	--	1,030
Depreciation and amortization	421	401	1,705	1,719	13,487
In-process research and development	--	--	--	--	418
Total operating expenses	15,923	16,114	71,342	64,544	527,788
Loss from operations	(15,923)	(15,790)	(52,931)	(64,181)	(405,550)
Other income (expenses):					
Interest income	81	27	316	174	14,563
Interest expense	(12)	(20)	(89)	(46)	(2,468)
Change in fair value of warrant liability	2,594	34	653	908	2,461
Other income	--	--	21	--	252
Loss before tax benefit	(13,260)	(15,749)	(52,030)	(63,145)	(390,742)
Benefit from income taxes	3,245	3,512	3,245	3,512	12,220
Net loss	(10,015)	(12,237)	(48,785)	(59,633)	(378,522)
Deemed dividend	--	--	--	--	(19,424)
Preferred stock accretion	--	--	--	--	(802)
Net loss attributable to common stockholders	<u>\$ (10,015)</u>	<u>\$ (12,237)</u>	<u>\$ (48,785)</u>	<u>\$ (59,633)</u>	<u>\$ (398,748)</u>
Net loss attributable to common stockholders per common share - basic and diluted	<u>\$ (0.20)</u>	<u>\$ (0.22)</u>	<u>\$ (1.07)</u>	<u>\$ (1.16)</u>	
Weighted-average common shares outstanding - basic and diluted	<u>49,477,596</u>	<u>56,173,260</u>	<u>45,565,217</u>	<u>51,286,059</u>	

**Table 2**

**Amicus Therapeutics, Inc.**  
**(a development stage company)**  
**Consolidated Balance Sheets**  
**(Unaudited)**  
**(in thousands, except share and per share amounts)**

	<b>December 31, 2012</b>	<b>December 31, 2013</b>
<b>Assets:</b>		
Current assets:		
Cash and cash equivalents	\$33,971	\$43,640
Investments in marketable securities	65,151	38,360
Receivable due from GSK	3,225	759
Prepaid expenses and other current assets	2,270	5,519
Total current assets	104,617	88,278
Property and equipment, less accumulated depreciation and amortization of \$8,501 and \$9,973 at December 31, 2012 and 2013, respectively	5,029	4,120
In-process research & development	—	23,000
Goodwill	—	11,613
Other non-current assets	442	552

<b>Total Assets</b>	<u>\$110,088</u>	<u>\$127,563</u>
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities:		
Accounts payable and accrued expenses	\$8,845	\$10,162
Current portion of secured loan	<u>398</u>	<u>299</u>
Total current liabilities	19,256	9,243
Deferred reimbursements, less current portion	30,418	36,677
Warrant liability	908	—
Secured loan, less current portion	299	14,174
Contingent consideration payable	—	10,600
Deferred tax liability	—	9,186
Other non-current liability	—	714
Commitments and contingencies		
Stockholders' equity:		
Common stock, \$.01 par value, 125,000,000 shares authorized, 49,631,672 shares issued and outstanding at December 31, 2012, 61,975,416 shares issued and outstanding at December 31, 2013	556	679
Additional paid-in capital	387,539	423,593
Accumulated other comprehensive income	14	1
Deficit accumulated during the development stage	<u>(318,889)</u>	<u>(378,522)</u>
Total stockholders' equity	<u>69,220</u>	<u>45,751</u>
<b>Total Liabilities and Stockholders' Equity</b>	<u>\$110,088</u>	<u>\$127,563</u>

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