



November 20, 2013

## **Amicus Therapeutics Strengthens Biologics Business Strategy**

***Callidus Biopharma Acquisition Adds Next-Generation Pompe ERT and Enzyme Technology Platform***

***Revised Agreement with GSK Provides Global Development & Commercial Rights to Next-Generation Fabry ERT***

***Company Funded into Late 2015 with \$40 Million in Equity and Expected Debt Financing***

***Conference Call Today at 5:00 p.m. ET***

CRANBURY, N.J., Nov. 20, 2013 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD), a biopharmaceutical company at the forefront of therapies for rare and orphan diseases, today announced a broad strengthening of its biologics business strategy. The details of this important strategic repositioning, updates on all development programs, and upcoming milestones will be discussed during a conference call and webcast at 5:00 p.m. ET today.

### **Key Highlights:**

-- Amicus now owns exclusive worldwide rights to three next-generation Enzyme Replacement Therapies (ERTs) in preclinical development.

- Callidus Biopharma acquisition adds next-generation Pompe ERT and complementary enzyme targeting technologies.
- Revised GSK collaboration provides full ex-US rights to next-generation Fabry ERT.
- Next-generation ERT for MPS I continues to be supported by a grant from anonymous U.S.-based donor

-- Current cash, including \$40 million in equity and expected debt financing, anticipated to fund operating plan into late 2015.

-- Organization restructured and realigned to support next-generation biologics development strategy, saving approximately \$4.0 million annually.

-- Monotherapy programs in ongoing Phase 3 studies for Fabry disease and in preclinical studies for Parkinson's disease.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc., stated, "Today marks a bold step forward for Amicus shareholders and the orphan disease community, especially patients with Fabry, Pompe and MPS I. With the culmination of several transformative business development transactions and a new financing, we are advancing an Amicus that is better resourced and more sharply focused toward our valuable biologics business. Not only do we own global rights to all of our CHART and Fabry monotherapy programs, but we have also brought in a uniquely engineered, proprietary next-generation ERT in late preclinical development for Pompe disease. We believe we now have the optimal set of technologies, portfolio, financing and leadership team to be able to advance our vision of developing improved therapies for people living with many rare and orphan diseases."

### **Callidus Biopharma Acquisition**

Amicus has acquired [Callidus Biopharma](#), a privately-held biologics company focused on developing best-in-class enzyme replacement therapies (ERTs) for lysosomal storage diseases (LSDs). Callidus' lead ERT is a recombinant human acid-alpha glucosidase (rhGAA) for Pompe disease in late preclinical development. This Pompe ERT has shown superior uptake and activity when compared to Lumizyme<sup>®</sup> in preclinical studies, and may be further improved by incorporating Amicus' pharmacological chaperone AT2220 as a stabilizer to potentially enhance tissue uptake and reduce the immunogenicity of the ERT. Callidus' enzyme targeting technology is also applicable to multiple ERTs. These Callidus assets complement Amicus' CHART<sup>™</sup> platform for the development of bibeppers for multiple LSDs.

"We believe that this is a highly synergistic strategic combination," said Mr. Crowley. "With the addition of Callidus' Pompe program, we can move a next-generation Pompe ERT into the clinic in early 2015, approximately 12 months faster than our

internal Pompe program. Even more significantly, we believe that the Callidus ERT for Pompe is a highly innovative and potentially superior ERT due to its carbohydrate structure which should provide for greater uptake of the ERT into target muscle cells in Pompe patients. For these reasons we believe that the best course forward for patients is to advance the next-generation ERT as quickly as possible instead of the planned Phase 2 study of AT2220 co-administered with ERT. In addition, we believe Callidus' targeting technology complements our CHART platform and can be used together to develop bio-better ERTs."

Under terms of the agreement, Callidus shareholders will receive \$15 million in shares of Amicus common stock; up to \$10 million in milestone payments through Phase 2 development of the Pompe program; and up to \$105 million for the achievement of late-stage development, regulatory, and approval milestones spread across three products. In conjunction with the transaction, Hung Do, PhD, Founder and Chief Scientific Officer of Callidus Biopharma, has been appointed Amicus' Senior Vice President, Discovery Biology. Dr. Do has nearly fifteen years of experience in the field of LSDs and ERTs while working at Novazyme, Genzyme, Amicus, and Callidus.

### **\$40 Million in Equity and Expected Debt Financing**

Amicus has secured approximately \$40 million in equity and expected debt financing:

- \$15 million was raised in a private placement of 7.5 million shares of common stock priced at \$2.00 per share, plus the issuance of warrants to purchase an additional 1.6 million shares at \$2.50/share, with a one-year term exercisable between July 1, 2014 and June 30, 2015. Participants were Redmile Group and GSK.
- \$25 million in debt financing expected to close in coming weeks, at a cost of capital of less than 10%, with no warrant coverage

The Company projects that the current cash position, including the proceeds from the private placement and debt financings, are sufficient to fund operations into late 2015.

### **Organizational Restructuring**

Amicus has additionally restructured the organization to reduce costs and to align its resources with its biologics business strategy. The workforce, including full-time employees across all levels and departments, has been reduced by approximately 14% to 91 employees. Amicus will also close its San Diego research facility and will consolidate all operations at its Cranbury, NJ headquarters. As part of this restructuring, David J. Lockhart, PhD, will step down from his role as Chief Scientific Officer and will continue as a member of Amicus' Scientific Advisory Board.

"In broadening our biologics business strategy, and as part of our commitment to judiciously manage our cash flow, we have had to make some difficult decisions in restructuring and realigning our organization," said Mr. Crowley. "These employees have made significant contributions during their time at Amicus. On behalf of the executive team and our board of directors, I am very grateful for their commitment to Amicus during their time here and wish them well in their future endeavors, especially Dr. David Lockhart, our Chief Scientific Officer who will be leaving Amicus. David is a brilliant scientist and has been a great leader for Amicus. As our strategy evolves towards a focus on the development of next-generation ERTs, I look forward to working with David as a member of our Scientific Advisory Board."

The company estimates that it will record charges of approximately \$2.5 million during the fourth quarter of 2013 for employment termination costs payable in cash in connection with the workforce reduction, as well as facilities closing costs. The restructuring is expected to save approximately \$4.0 million annually.

### **Conference Call and Webcast**

Amicus Therapeutics will host a conference call and audio webcast today, November 20, 2013 at 5:00 p.m. ET to discuss the strengthening of its business strategy. 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 14704378.

### **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). Amicus is leveraging the CHART platform to develop proprietary next-generation therapies that consist of lysosomal enzymes co-formulated with pharmacological chaperones.

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.

### **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, financing plans, 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.

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Source: Amicus Therapeutics, Inc.

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