Amicus Therapeutics Announces Full-Year 2017 Financial Results and 2018 Corporate Updates

February 28, 2018

Galafold Expansion Continues with 360+ Fabry Patients Treated Today - on Track to Achieve FY18 Revenue Guidance of $75M-$85M

Significant Progress Across Pompe Clinical, Regulatory and Manufacturing Activities - Successfully Completed First GMP Manufacturing Campaign of ATB200 Drug Substance at 1000L Scale

$359M Year-End Cash Balance Strengthened with $300 Million of Gross Proceeds from February 2018 Equity Financing

Conference Call and Webcast Today at 8:30am ET

CRANBURY, N.J., Feb. 28, 2018 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD), a global biotechnology company focused on discovering, developing and delivering novel medicines for rare metabolic diseases, today announced financial results for the full year ended December 31, 2017. The Company also summarized recent program updates and reiterated full-year 2018 revenue and net cash spend guidance.

Corporate Highlights for Full-Year 2017 and Year-to-Date 2018

- Full-year 2017 revenue for Fabry oral precision medicine Galafold (migalastat) totaled $36.9 million. On track to achieve full-year 2018 revenue guidance of $75M-$85M with more than 360 patients treated today.
- Regulatory decisions for migalastat anticipated in 1H18 in Japan (J-NDA) and in 3Q18 in the U.S. (NDA filed under priority review; PDUFA action date August 13, 2018).
- Novel, highly differentiated Pompe treatment regimen ATB200/AT2221 continues to show persistent and durable improvements on functional outcomes and key disease biomarkers, with favorable safety profile, following up to 12 months of treatment in Pompe patients.
- Significant Pompe biologics manufacturing milestones achieved. Agreement reached with FDA on biocomparability between 250L GMP and 1000L engineering batches in 4Q17. Initial 1000L GMP drug substance manufacturing campaign now complete in 1Q18.
- Building robust Pompe clinical data package to include current and additional patients in ongoing Phase 1/2 clinical study, as well as a supportive retrospective natural history study and prospective observational study. Regulatory update expected in 2Q18.
- Cash, cash equivalents and marketable securities totaled $358.6 million at December 31, 2017. Balance sheet further strengthened with $300M in gross proceeds from a follow on public offering in February 2018.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. stated, “Throughout 2017 we continued to build a leading global rare disease biotech company. In the early part of 2018 we have made significant progress to further advance our vision to maximize the impact of our medicines for people living with rare metabolic diseases. There is tremendous momentum for the launch and regulatory review processes for Galafold as we expand global access to this important oral precision medicine for Fabry disease. In addition, the longer term data from our Pompe clinical study further strengthen the potential of our new treatment paradigm for this devastating muscle disease. With these key value drivers in Fabry and Pompe, supported by a strengthened balance sheet and several pipeline opportunities, Amicus is well positioned to create significant near- and long-term value for patients and shareholders.”

2018 Key Strategic Priorities

- Double global revenue for Galafold ($75 million - $85 million)
- Secure approvals for migalastat in Japan and the U.S.
- Achieve clinical, manufacturing and regulatory milestones to advance ATB200/AT2221 toward global regulatory submissions and approvals as soon as possible
- Develop and expand preclinical pipeline to ensure at least one new clinical program in 2019
- Maintain a strong balance sheet

Full-Year 2017 Financial Results

- Total revenue in the full-year 2017 was $36.9 million, an increase from total revenue of $5.0 million in the full-year 2016. Total revenue represents commercial sales of Galafold (migalastat) which commenced in May 2016, as well as reimbursed Expanded Access Programs (EAPs).
- Cash, cash equivalents, and marketable securities totaled $358.6 million at December 31, 2017 compared to $330.4 million at December 31, 2016.
- Total operating expenses increased to $472.7 million for the full-year 2017 compared to $186.0 million in the full-year 2016.

Operating expenses in 2017 included $210.8 million of non-cash charges related to the Phase 3 ESSENCE study in epidermolysis bullosa (EB).

- Operating expenses, as adjusted, excluding the impact of non-cash charges related to the ESSENCE study, were $261.9 million for the full-year 2017, reflecting increased investments in the Pompe and EB programs as well as increased investment in the Galafold commercial launch.
- Net cash spend was $216.5 million for the full-year 2017, in-line with full-year 2017 net cash spend guidance of $200 million to $225 million.
- Net loss was $284.0 million, or $1.85 per share, compared to a net loss of $200.0 million, or $1.49 per share, for the full-year 2016. Net loss, as adjusted, excludes the impact of the non-cash charges related to the ESSENCE study and was $237.9 million or $1.55 per share.

2018 Financial Guidance

Amicus recorded $36.9 million in full-year 2017 revenue from commercial sales and reimbursed expanded access programs for Galafold. For the full-year 2018 the Company anticipates total Galafold revenue of $75 million to $85 million.

Cash, cash equivalents, and marketable securities totaled $358.6 million at December 31, 2017. As previously announced, the Company further strengthened the balance sheet with $300 million in gross proceeds from a follow on public offering in February 2018. The Company continues to expect full-year 2018 net cash spend of between $230 million and $260 million. The current cash position, including proceeds from the recent equity offering and expected Galafold revenues, is sufficient to fund ongoing Fabry and Pompe program operations into at least 2021. Potential future business development collaborations, pipeline expansion, and investment in biologics manufacturing capabilities could impact our future capital requirements.

Program Highlights

Migalastat for Fabry Disease
Migalastat is an oral precision medicine intended to treat Fabry disease in patients who have amenable genetic mutations. Regulatory authorities in the European Union, Switzerland, Israel, Canada, Australia, and South Korea have granted full approval for migalastat under the trade name Galafold. The EU approval may serve as the basis for regulatory approvals in more than two-thirds of the global Fabry market that is outside the U.S. In the U.S., the U.S. FDA accepted the Company's new drug application (NDA) for migalastat under priority review with a six-month PDUFA goal date of August 13, 2018.

Amicus is committed to advancing the highest quality therapies for all people living with Fabry disease. For people with non-amenable mutations who are not eligible for migalastat as an oral precision medicine, the strategy is to advance next-generation therapies such as a novel Fabry ERT (ATB101) co-formulated with migalastat or other innovative technologies that continue to be evaluated.

Global Fabry Updates:

- 360+ patients (naïve and ERT-switch) on reimbursed Galafold as of February 28, 2017
- Total full-year 2017 revenue of $36.9 million from global commercial sales and expanded access programs (EAPs)
- Pricing and reimbursement secured in 18 countries
- Approvals secured in EU, Australia, Canada, Israel, South Korea and Switzerland
- Approvals pending in Japan, U.S. and Taiwan

Anticipated Milestones:

- Regulatory decisions on Japanese J-NDA (1H18) and U.S. NDA (3Q18)
- Total full-year 2018 revenue guidance of $75 million to $85 million
- ATB101 co-formulated with migalastat advancing toward the clinic in 2019

ATB200/AT2221 for Pompe Disease

ATB200/AT2221 is a novel treatment paradigm that consists of ATB200, a unique recombinant human acid alpha-glucosidase (rhGAA) enzyme with optimized carbohydrate structures, particularly mannose-6 phosphate (M6P), to enhance uptake, co-administered with AT2221, a pharmacological chaperone. During WORLD*Symposium*™ in February 2018, Amicus presented updated positive data from an ongoing global Phase 1/2 clinical study (ATB200-02) to evaluate safety, tolerability, pharmacokinetics (PK), pharmacodynamics (PD), and functional outcomes of ATB200/AT2221 across ambulatory ERT-switch patients (Cohort 1), non-ambulatory ERT-switch patients (Cohort 2), and ERT-naïve patients (Cohort 3).

The Company is in the midst of a series of collaborative discussions with U.S. and EU regulators regarding the best and fastest pathway forward for this novel treatment option. Amicus expects to provide a Pompe regulatory pathway update in the second quarter of 2018. While these discussions are underway, Amicus continues to make progress with clinical and manufacturing activities to support the needs of the Pompe community.

Pompe clinical activities:

- Phase 1/2 clinical study includes 19 current patients treated for up to 12 months, with four to six additional patients to be enrolled
- Retrospective study (POM-002) on the natural history of Pompe disease in up to 100 ERT-treated Pompe patients to help provide context for the ATB200-02 clinical study results
- Prospective observational study (POM-003) to assess safety and functional outcomes in patients currently treated with standard of care ERT, and to serve as a potential run-in for a registration study

Pompe manufacturing activities:

- FDA agreed on comparability between 250L scale and 1000L engineering batches, as well as the testing strategy for demonstrating comparability between 250L scale and 1000L GMP batches
- Initial GMP production runs of ATB200 drug substance completed at the 1,000 liter commercial scale

Anticipated Upcoming Pompe Program Milestones:

- Expansion of ongoing ATB200-02 clinical study to include four to six additional ambulatory ERT-switch patients
- Final demonstration of comparability between 1,000L GMP material and 250L material and release for clinic of 1,000L GMP material
- Pompe regulatory pathway update (2Q18)
- Initiation of larger registration-directed study (2H18)

Conference Call and Webcast
Amicus Therapeutics will host a conference call and audio webcast today, February 28, 2018 at 8:30 a.m. ET to discuss full year 2017 financial results and corporate updates. Interested participants and investors may access the conference call by dialing 877-303-5859 (U.S./Canada) or 678-224-7784 (international), conference ID: 9598004.

An audio webcast can also be accessed via the Investors section of the Amicus Therapeutics corporate website at http://ir.amicusrx.com/, and will be archived for 30 days. Web participants are encouraged to go to the website 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 11:30 a.m. ET on February 28, 2018. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); conference ID: 9598004.

Non-GAAP Financial Measures
In addition to the United States generally accepted accounting principles (GAAP) results, this earnings release contains non-GAAP financial measures that we believe, when considered together with the GAAP information, provides useful information to investors that promotes a more complete understanding of our operating results and financial position for the current period. Management uses these non-GAAP financial measures internally for planning, forecasting, evaluating and allocating resources to the Company's programs. The non-GAAP results exclude the impact of the following GAAP items specifically related to the Phase 3 ESSENCE study in EB: changes in fair value of contingent consideration, impairment of assets and adjustments to income tax expense. These non-GAAP financial measures should be considered in addition to, and not as replacements for, or superior to, measures of financial performance prepared in accordance with GAAP. Please refer to the attached Reconciliation of Non-GAAP Financial Measures for explanations of the amounts adjusted to arrive at non-GAAP total operating expense, net loss attributable to common stockholders and net loss attributable to common stockholders per common share - basic and diluted for the year ended December 31, 2017.

EU Important Safety Information
Treatment with GALAFOLD should be initiated and supervised by specialists experienced in the diagnosis and treatment of Fabry disease. GALAFOLD is not recommended for use in patients with a nonamenable mutation.

- GALAFOLD is not intended for concomitant use with enzyme replacement therapy.
- GALAFOLD is not recommended for use in patients with Fabry disease who have severe renal impairment (<30 mL/min/1.73 m^2). The safety and efficacy of GALAFOLD in children 0–15 years of age have not yet been established.
- No dosage adjustments are required in patients with hepatic impairment or in the elderly population.
- There is very limited experience with the use of this medicine in pregnant women. If you are pregnant, think you may be pregnant, or are planning to have a baby, do not take this medicine until you have checked with your doctor, pharmacist, or nurse.
- While taking GALAFOLD, effective birth control should be used. It is not known whether GALAFOLD is excreted in human milk.
- Contraindications to GALAFOLD include hypersensitivity to the active substance or to any of the excipients listed in the PRESCRIBING INFORMATION.
- It is advised to periodically monitor renal function, echocardiographic parameters and biochemical markers (every 6 months) in patients initiated on GALAFOLD or switched to GALAFOLD.
- OVERDOSE: General medical care is recommended in the case of GALAFOLD overdose.
- The most common adverse reaction reported was headache, which was experienced by approximately 10% of patients who received GALAFOLD. For a complete list of adverse reactions, please review the SUMMARY OF PRODUCT CHARACTERISTICS.
- Call your doctor for medical advice about side effects.

For further important safety information for Galafold, including posology and method of administration, special warnings, drug interactions and adverse drug reactions, please see the European SmPC for Galafold available from the EMA website at www.ema.europa.eu.

About Amicus Therapeutics
Amicus Therapeutics (Nasdaq:FOLD) is a global, patient-centric biotechnology company focused on discovering, developing and delivering novel high-quality medicines for people living with rare metabolic diseases. The cornerstone of the Amicus portfolio is migalastat, an oral precision medicine for people living with Fabry disease who have amenable genetic mutations. Migalastat is currently approved under the trade name Galafold™ in the European Union, with additional approvals granted and pending in several geographies. The lead biologics program in the Amicus pipeline is ATB200/AT2221, a novel, late-stage, potential best-in-class treatment paradigm for Pompe disease. The Company is committed to advancing and expanding a robust pipeline of cutting-edge, first- or best-in-class medicines for rare metabolic diseases.

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 our product candidates, the timing and reporting of results from preclinical studies and clinical trials, the prospects and timing of the potential regulatory approval of our product candidates, commercialization plans, manufacturing and supply plans, financing plans, and the projected revenues and cash position for the Company. The inclusion of forward-looking statements should not be regarded as a representation by us that any of our plans will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong and can be affected by inaccurate assumptions we 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 in particular 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 our business, including, without limitation: the potential that results of clinical or preclinical 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, including the FDA, EMA, and PMDA, may not grant or may delay approval for our product candidates; the potential that we may not be successful in commercializing Galafold in Europe and other geographies or our other product candidates if and when approved; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; the potential that we may not be able to manufacture or supply sufficient clinical or commercial products; and the potential that we will need additional funding to complete all of our studies and manufacturing. 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 revenue and 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, 2017 to be filed on March 1, 2018. 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 we undertake no obligation to revise or update this news release to reflect events or circumstances after the date hereof.

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TABLE 1
Amicus Therapeutics, Inc.
Consolidated Statements of Operations
(in thousands, except share and per share amounts)

	Years Ended December 31,		
	2017	**2016**	**2015**
Revenue:			
Net Product Sales	$ 36,930	$ 4,958	$ —
Total revenue	36,930	4,958	—
Cost of goods sold	6,236	833	—
Gross Profit	30,694	4,125	—
Operating Expenses:			
Research and development	149,310	104,793	76,943
Selling, general and administrative	88,671	71,151	47,269
Changes in fair value of contingent consideration payable	(234,322)	6,760	4,377
Loss on impairment of assets	465,427	—	—
Restructuring charges	—	69	15
Depreciation	3,593	3,242	1,833
Total operating expenses	472,679	186,015	130,437
Loss from operations	(441,985)	(181,890)	(130,437)
Other income (expenses):			

Interest income	4,096	1,602	929
Interest expense	(17,240)	(5,398)	(1,578)
Loss on extinguishment of debt	—	(13,302)	(952)
Other income (expense)	6,008	(4,793)	(80)
Loss before income tax benefit	(449,121)	(203,781)	(132,118)
Income tax benefit	165,119	3,739	—
Net loss attributable to common stockholders	$(284,002)	$(200,042)	$(132,118)
Net loss attributable to common stockholders per common share — basic and diluted	$(1.85)	$(1.49)	$(1.20)
Weighted-average common shares outstanding — basic and diluted	153,355,144	134,401,588	109,923,815

TABLE 2

Amicus Therapeutics, Inc.
Consolidated Balance Sheets
(in thousands, except share and per share amounts)

	December 31, 2017	2016
Assets		
Current assets:		
Cash and cash equivalents	$49,060	$187,026
Investments in marketable securities	309,502	143,325
Accounts receivable	9,464	1,304
Inventories	4,623	3,416
Prepaid expenses and other current assets	19,316	4,993
Total current assets	391,965	340,064
Property and equipment, less accumulated depreciation of $12,515 and $12,495 at December 31, 2017 and 2016, respectively	9,062	9,816
In-process research & development	23,000	486,700
Goodwill	197,797	197,797
Other non-current assets	5,200	2,468
Total Assets	$ 627,024	$1,036,845
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable, accrued expenses, and other current liabilities	$ 53,890	$41,008
Deferred reimbursements, current portion	7,750	13,850
Contingent consideration payable, current portion	8,400	56,101
Total current liabilities	70,040	110,959
Deferred reimbursements	14,156	21,906
Convertible notes	164,167	154,464
Contingent consideration payable	17,000	213,621
Deferred income taxes	6,465	173,771
Other non-current liability	2,346	1,973
Commitments and contingencies		
Stockholders' equity:		
Common stock, $.01 par value, 250,000,000 shares authorized, 166,989,790 shares issued and outstanding at December 31, 2017 Common stock, $.01 par value, 250,000,000 shares authorized, 142,691,986 shares issued and outstanding at December 31, 2016	1,721	1,480
Additional paid-in capital	1,400,758	1,120,156
Accumulated other comprehensive loss:		
Foreign currency translation adjustment	(1,659)	1,945
Unrealized gain/ (loss) on available-for securities	(436)	102
Warrants	16,076	16,076
Accumulated deficit	(1,063,610)	(779,608)
Total stockholders' equity	352,850	360,151

Total Liabilities and Stockholders' Equity	$ 627,024	$1,036,845

TABLE 3
Amicus Therapeutics, Inc.
Reconciliation of Non-GAAP Financial Measures
(in thousands, except share and per share amounts)

	Year Ended December 31, 2017
Total operating expenses – as reported	$ 472,679
Loss on impairment of assets related to the Phase 3 ESSENCE study in EB	465,427
Changes in fair value of contingent consideration payable related to the Phase 3 ESSENCE study in EB	(254,650)
Total operating expenses – as adjusted	$ 261,902
Net loss attributable to common stockholders – as reported	$ (284,002)
Loss on impairment of assets related to the Phase 3 ESSENCE study in EB	465,427
Changes in fair value of contingent consideration payable related to the Phase 3 ESSENCE study in EB	(254,650)
Income tax benefit (1)	164,683
Net loss attributable to common stockholders – as adjusted	$ (237,908)
Net loss attributable to common stockholders per common share – basic and diluted – as reported	$ (1.85)
Net loss attributable to common stockholders per common share – basic and diluted – as adjusted	$ (1.55)
Weighted-average common shares outstanding – basic and diluted – as reported and adjusted	153,355,144

(1) Related to the reversal of the deferred tax liability associated with the Scioderm in process research and development asset.

Source: Amicus Therapeutics, Inc.