March 1, 2017

Amicus Therapeutics Announces Full-Year 2016 Financial Results and Corporate Updates

Growing Momentum for EU Galafold (Migalastat) Launch for Fabry Disease Tracking Toward 300 Patients by Year-End 2017

Target Enrollment Achieved in Phase 1/2 Pompe Study - Additional Data Expected in 2Q17 and 3Q17

Phase 3 EB Program Remains on Track for Topline Data in Mid-2017

CRANBURY, N.J., March 01, 2017 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD), a biotechnology company at the forefront of therapies for rare and orphan diseases, today announced financial results for the full year ended December 31, 2016. The Company also provided program updates and reiterated full-year 2017 financial guidance.

"Throughout 2016 we made significant progress with the international launch of our first commercial product Galafold and continued to advance and expand our robust pipeline of first- and/or best-in-class medicines for people living with devastating rare diseases," stated John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. "During 2017 we are laser focused on five key strategic priorities to advance our vision to develop and deliver great medicines for patients and to create significant shareholder value: 1) advancing the international launch of Galafold for Fabry disease, 2) completing our regulatory submission for migalastat in Japan (J-NDA), 3) establishing our novel Pompe treatment paradigm ATB200/AT2221 as a highly differentiated therapy, 4) successfully completing our Phase 3 clinical study in patients with epidermolysis bullosa, and 5) maintaining our financial strength. With one commercial-stage medicine and two medicines in clinical development, as well as a biologics platform for future growth, we are building a leading global biotechnology company focused on delivering meaningful benefits for patients living with devastating rare diseases."

2016 Full-Year Financial Results

- Total product revenue in the full year 2016 was approximately $5.0 million, which represents commercial sales of Galafold (migalastat) in Germany as well as reimbursed Expanded Access Programs (EAPs) in two countries during the third and fourth quarter of 2016.
- Cash, cash equivalents, and marketable securities totaled $330.4 million at December 31, 2016 compared to $214.0 million at December 31, 2015.
- Total operating expenses increased to $186.0 million compared to $130.4 million for the full year 2015 primarily due to increases in commercial costs of the Fabry monotherapy program and manufacturing scale-up on the Pompe program.
- Net cash spend was $154.3 million, within the full-year 2016 guidance of $135-155 million.
- Net loss was $200.0 million, or $1.49 per share, compared to a net loss of $132.1 million, or $1.20 per share, for the full year 2015.

2017 Financial Guidance

Cash, cash equivalents, and marketable securities totaled $330.4 million at December 31, 2016. As previously announced, the Company strengthened the balance sheet during 2016 with a $100 million at-the-market (ATM) equity financing as well as a $250 million convertible debt offering. The Company expects full-year 2017 net operating cash spend of between $175 million to $200 million and expects full-year 2017 total net cash spend (including third-party milestone payments and capital expenditures) of between $200 million and $225 million. The current cash position is anticipated to fund ongoing operations into the second half of 2018.

Program Highlights

Migalastat for Fabry Disease

Migalastat is an oral precision medicine intended to treat Fabry disease in patients who have amenable genetic mutations. As previously announced, the European Commission (EC) has granted full approval for migalastat under the trade name Galafold. The EC 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. The Company has also defined a U.S. pathway to support full approval.

International Launch and Expanded Access Programs (EAP) Updates:

- 75 patients (naïve and ERT-switch) on reimbursed Galafold as of February 28, 2017
- 10 countries with reimbursement (commercial or EAP)
- Reimbursement dossiers submitted and pricing discussions are now underway in 14 countries
- Launch commenced in United Kingdom following National Institute for Health and Care Excellence (NICE) publication of final guidance recommending reimbursement of Galafold in England
- Target of 300 patients treated with reimbursed Galafold by year-end 2017

Regulatory Updates:

- One additional approval secured outside EU (Switzerland)
- Regulatory submissions completed in six additional territories outside EU
- Phase 3 gastrointestinal (GI) symptoms study protocol nearly complete and detailed feasibility study underway to support U.S. full approval pathway

Anticipated Upcoming Fabry Disease Program Milestones:

- EU commercial launch in additional countries and EAP in additional territories
- Additional regulatory submissions including a Japanese regulatory submission (J-NDA) targeted for 1H17
- U.S. intermediate EAP
- Phase 3 gastrointestinal (GI) symptoms study
- Fabry ERT cell line development and program update

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. Positive preliminary data were reported in the fourth quarter of 2016 and during the 13th Annual WORLDSymposium™ in San Diego, CA in February 2017 from a global clinical study (ATB200-02) to evaluate safety, tolerability, PK, and pharmacodynamics (PD) of ATB200/AT2221. The study is enrolling 3 cohorts of patients, including ambulatory ERT-switch patients (Cohort 1), non-ambulatory ERT-switch patients (Cohort 2), and ERT-naïve patients (Cohort 3).

Key Preliminary Data Highlights from ATB200-02 Study in Initial ERT-Switch and ERT-Naïve Patients:

- No infusion-associated reactions following 150+ infusions in initial patients treated for a maximum of 36 weeks (n=13)
- Available PK and PD (muscle and glycogen biomarkers) data through week 18 in eight initial ERT-switch patients and two ERT-naïve showed:
  - The desired PK profile
  - Improvements in key muscle damage biomarkers (creatine kinase (CK) enzyme, alanine aminotransferase (ALT), and aspartate aminotransferase (AST)) in a majority of ERT-switch patients and both ERT-naïve patients
  - Reductions in a biomarker of glycogen substrate urine hexose tetrasaccharide (Hex4) in all patients
- Target enrollment achieved across all patient cohorts

Anticipated Upcoming Pompe Disease Program Milestones:

- ATB200-02 study data in additional naïve and non-ambulatory patients, as well as extension-phase data on all patient cohorts, in the second and third quarter of 2017
- Meetings with US and EU regulators

SD-101 for Epidermolysis Bullosa (EB)

SD-101 is a novel, late-stage, proprietary topical treatment and potential first-to-market therapy for EB. SD-101 is currently being investigated in a registration-directed Phase 3 study (ESSENCE, also known as SD-005) to support global regulatory submissions.

SD-101 was granted FDA Breakthrough Therapy designation in 2013 based on results from a Phase 2a study for the treatment of lesions in patients suffering with EB. SD-101 is the first-ever treatment in clinical studies to show improvements

in wound closure across all major EB types.

EB Phase 3 ESSENCE Study Highlights:

- Significant momentum enrolling patients diagnosed with Simplex, Recessive Dystrophic, or Junctional non-Herlitz EB
- More than 95% of patients completing the primary treatment period have elected to continue in the open-label extension study

Anticipated EB Program Milestones:

- Top-line Phase 3 data anticipated mid-2017

Conference Call and Webcast
Amicus Therapeutics will host a conference call and audio webcast today, March 1, 2017 at 8:30 a.m. ET to discuss full-year 2016 financial results and corporate updates. Interested participants and investors may access the conference call at 8:00 a.m. ET by dialing 877-303-5859 (U.S./ Canada) or 678-224-7784 (international) participant code 77299407.

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 11:30 a.m. ET today. Access numbers for this replay are 855-859-2056 (U.S. /Canada) and 404-537-3406 (international); participant code 77299407.

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 biotechnology company at the forefront of therapies for rare and orphan diseases. The Company has a robust pipeline of advanced therapies for a broad range of human genetic diseases. Amicus' lead programs in development include the small molecule pharmacological chaperone migalastat as a monotherapy for Fabry disease, SD-101 for Epidermolysis Bullosa (EB), as well as novel enzyme replacement therapy (ERT) and biologic products for Fabry disease, Pompe disease, and other rare and devastating 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, financing plans, and the projected cash position for the Company. In particular, this press release

relates to the preliminary data from a global Phase 1/2 study (ATB200-02) to investigate ATB200/AT2221. The inclusion of forward-looking statements arising from this preliminary data and study 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 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; and the potential that we will need additional funding to complete all of our studies. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results for any of our product candidates. The preliminary data and Phase 1/2 study discussed herein is inherently preliminary and early in the study, derived from a limited patient set, and later trial results with this patient set or others may not be consistent with these preliminary 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 previous filings with the SEC and in our Annual Report on Form 10-K for the year ended December 31, 2016 to be filed later today. 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.

TABLE 1

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

	Years Ended December 31,		
	2016	**2015**	**2014**
Revenue:			
Net Product Sales	$ 4,958	$ —	$ —
Research revenue	—	—	1,224
Total revenue	4,958	—	1,224
Cost of goods sold	833	—	
Gross Profit	4,125	—	1,224
Operating Expenses:			
Research and development	104,793	76,943	47,624
Selling, general and administrative	71,151	47,269	20,717
Changes in fair value of contingent consideration payable	6,760	4,377	100
Restructuring charges	69	15	(63)
Depreciation	3,242	1,833	1,547
Total operating expenses	186,015	130,437	69,925
Loss from operations	(181,890)	(130,437)	(68,701)
Other income (expenses):			
Interest income	1,602	929	223
Interest expense	(5,398)	(1,578)	(1,484)
Loss on extinguishment of debt	(13,302)	(952)	—
Other expense	(4,793)	(80)	(77)
Loss before income tax benefit	(203,781)	(132,118)	(70,039)
Income tax benefit	3,739	—	1,113
Net loss attributable to common stockholders	$ (200,042)	$ (132,118)	$ (68,926)
Net loss attributable to common stockholders per common share — basic and diluted	$ (1.49)	$ (1.20)	$ (0.93)
Weighted-average common shares outstanding — basic and diluted	134,401,588	109,923,815	74,444,157

TABLE 2

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

	December 31, 2016	2015
Assets		
Current assets:		
Cash and cash equivalents	$ 187,026	$ 69,485
Investments in marketable securities	143,325	144,548
Accounts receivable	1,304	—
Inventories	3,416	—
Prepaid expenses and other current assets	4,993	2,568
Total current assets	340,064	216,601
Property and equipment, less accumulated depreciation of $12,495 and $13,353 at December 31, 2016 and 2015, respectively	9,816	6,178
In-process research & development	486,700	486,700
Goodwill	197,797	197,797
Other non-current assets	2,468	1,108
Total Assets	$ 1,036,845	$ 908,384
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable, accrued expenses, and other current liabilities	$ 41,008	$ 32,216
Deferred reimbursements, current portion	13,850	—
Contingent consideration payable, current portion	56,101	41,400
Total current liabilities	110,959	73,616
Deferred reimbursements	21,906	35,756
Convertible notes	154,464	—
Due to related party	—	41,601
Contingent consideration payable	213,621	232,677
Deferred income taxes	173,771	176,219
Other non-current liability	1,973	681
Commitments and contingencies		
Stockholders' equity:		
Common stock, $.01 par value, 250,000,000 shares authorized, 142,691,986 shares issued and outstanding at December 31, 2016		
Common stock, $.01 par value, 250,000,000 shares authorized, 125,027,034 shares issued and outstanding at December 31, 2015,	1,480	1,306
Additional paid-in capital	1,120,156	917,454
Accumulated other comprehensive loss:		
Foreign currency translation adjustment, less tax benefit of $1,293 at December 31, 2016	1,945	—
Unrealized gain/ (loss) on available-for securities	102	(115)
Warrants	16,076	8,755
Accumulated deficit	(779,608)	(579,566)
Total stockholders' equity	360,151	347,834
Total Liabilities and Stockholders' Equity	$ 1,036,845	$ 908,384

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