May 9, 2017

Amicus Therapeutics Announces First Quarter 2017 Financial Results and Corporate Updates

100+ Fabry Disease Patients Now on Reimbursed Galafold (migalastat) in the EU

On Target to Reach 300 Patients on Galafold by Year-End 2017

Additional Important Phase 1/2 Pompe Clinical Data Expected in 2Q17 and 3Q17

Phase 3 EB Topline Data on Track for 3Q17

CRANBURY, N.J., May 09, 2017 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD), a global biotechnology company at the forefront of therapies for rare and orphan diseases, today announced financial results for the first quarter ended March 31, 2017. The Company also provided program updates and reiterated full-year 2017 financial guidance.

"During the first quarter we continued to advance the international commercial launch of our oral precision medicine Galafold for Fabry disease, as we brought Galafold to more Fabry patients in Germany and other European markets with both new ERT switch patients as well as a notable proportion of ERT-naïve patients," stated John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. "We are extremely pleased with the progress of our Galafold launch in terms of patient and physician adoption, as well as pricing and reimbursement. With launch now just initiating this month in the UK, Italy, France, and additional markets, we are confident in achieving our target of 300 patients on Galafold by the end of this year. In addition to the launch, we have also made significant progress on our other four key strategic priorities for 2017: 1) targeting a Japanese new drug application (J-NDA) submission for migalastat in the second quarter, 2) establishing definitive clinical proof of concept for our novel Pompe treatment paradigm ATB200/AT2221, 3) completing our Phase 3 clinical study in patients with epidermolysis bullosa, and 4) maintaining our financial strength. We are particularly looking forward to sharing additional, important Pompe clinical data this quarter. We continue to believe that our Pompe program will be a crucial driver toward our vision of becoming one of the world's leading global rare disease biotechnology companies."

First Quarter 2017 Financial Results

- Total product revenue in the first quarter 2017 was approximately $4.2 million, a sequential increase of 50% over total product revenue of $2.8 million in the fourth quarter 2016. Total product revenue for both periods 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 $279.8 million at March 31, 2017 compared to $330.4 million at December 31, 2016.
- Total operating expenses increased to $55.4 million compared to $43.0 million for the first quarter 2016 primarily due to increases in commercial costs of the Fabry monotherapy program and manufacturing scale-up on the Pompe program.
- Net cash spend was $50.5 million.
- Net loss was $55.0 million, or $0.39 per share, compared to a net loss of $43.7 million, or $0.35 per share, for the first quarter 2016.

2017 Financial Guidance

Cash, cash equivalents, and marketable securities totaled $279.8 million at March 31, 2017. The Company continues to expect full-year 2017 net operating cash spend of between $175 million to $200 million and 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:

- 101 patients (naïve and ERT-switch) on reimbursed Galafold as of April 30, 2017
- 11 countries with reimbursement (commercial or EAP) including top four largest EU markets
- Reimbursement dossiers submitted and pricing discussions are now underway in 12 countries
- 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

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) (2Q17)
- Fabry ERT cell line development and optimization

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 from a global Phase 1/2 clinical study (ATB200-02) to evaluate safety, tolerability, PK, and pharmacodynamics (PD) of ATB200/AT2221. The study is fully enrolled with 3 cohorts of patients, including ambulatory ERT-switch patients (Cohort 1), non-ambulatory ERT-switch patients (Cohort 2), and ERT-naïve patients (Cohort 3).

Anticipated Upcoming Pompe Disease Program Milestones:

- Additional ATB200-02 study data (2Q17 and 3Q17)
- 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:

- Enrollment complete and target exceeded with more than 160 patients
- More than 95% of patients completing the primary treatment period have elected to continue in the open-label extension study

Anticipated EB Program Milestones:

- Agreement on final statistical analysis plan (SAP) with U.S. Food and Drug Administration (FDA)
- Top-line Phase 3 data (3Q17)

Conference Call and Webcast

Amicus Therapeutics will host a conference call and audio webcast today, May 9, 2017 at 8:30 a.m. ET to discuss first

quarter 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); participant code 16795077.

An audio webcast can also be accessed via the Investors section of the Amicus Therapeutics corporate web site at http://ir.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 16795077.

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. 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 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. 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. 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)

	Three months ended March 31,	
	2017	**2016**
Revenue:		
Net product sales	$ 4,169	$ —
Cost of goods sold	775	—
Gross Profit	3,394	—
Operating Expenses:		
Research and development	30,876	23,425
Selling, general and administrative	19,132	15,701
Changes in fair value of contingent consideration payable	4,578	3,152
Restructuring charges	—	50
Depreciation	823	673
Total operating expenses	55,409	43,001
Loss from operations	(52,015)	(43,001)
Other income (expenses):		
Interest income	759	307
Interest expense	(4,290)	(945)
Other income (expense)	610	(52)
Loss before income tax benefit	(54,936)	(43,691)
Income tax expense	(56)	—
Net loss attributable to common stockholders	$ (54,992)	$ (43,691)
Net loss attributable to common stockholders per common share — basic and diluted	$ (0.39)	$ (0.35)
Weighted-average common shares outstanding — basic and diluted	142,770,629	125,178,517

TABLE 2

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

	March 31,	December 31,
	2017	**2016**
Assets		
Current assets:		
Cash and cash equivalents	$ 44,755	$ 187,026
Investments in marketable securities	235,087	143,325
Accounts receivable	1,875	1,304
Inventories	3,698	3,416
Prepaid expenses and other current assets	10,792	4,993
Total current assets	296,207	340,064
Property and equipment, less accumulated depreciation of $13,316 and $12,495 at March 31, 2017 and December 31, 2016, respectively	9,745	9,816
In-process research & development	486,700	486,700
Goodwill	197,797	197,797
Other non-current assets	2,932	2,468
Total Assets	$ 993,381	$ 1,036,845

Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable, accrued expenses, and other current liabilities	$ 39,645	$ 41,008
Deferred reimbursements, current portion	13,850	13,850
Contingent consideration payable, current portion	56,101	56,101
Total current liabilities	109,596	110,959
Deferred reimbursements	21,906	21,906
Convertible notes	156,859	154,464
Contingent consideration payable	218,199	213,621
Deferred income taxes	173,820	173,771
Other non-current liability	2,223	1,973
Commitments and contingencies		
Stockholders' equity:		
Common stock, $0.01 par value, 250,000,000 shares authorized, 142,829,530 and 142,691,986 shares issued and outstanding at March 31, 2017 and December 31, 2016, respectively	1,482	1,480
Additional paid-in capital	1,126,148	1,120,156
Accumulated other comprehensive loss:		
Foreign currency translation adjustment, less tax expense of $1,293 at March 31, 2017 and at December 31, 2016	1,487	1,945
Unrealized gain/ (loss) on available-for securities	185	102
Warrants	16,076	16,076
Accumulated deficit	(834,600)	(779,608)
Total stockholders' equity	310,778	360,151
Total Liabilities and Stockholders' Equity	$ 993,381	$ 1,036,845

FOLD-G

CONTACTS:

Investors/Media:

Amicus Therapeutics

Sara Pellegrino

Senior Director, Investor Relations

spellegrino@amicusrx.com

(609) 662-5044

Primary Logo

Source: Amicus Therapeutics, Inc

News Provided by Acquire Media