Amicus Therapeutics Announces First Quarter 2018 Financial Results and Corporate Updates

May 8, 2018

Continued Global Galafold® (Migalastat) Adoption and Expansion – Reaffirms FY18 Revenue Guidance of $75M-$85M at Top End of Range

Significant Momentum with Pompe Clinical, Regulatory and Manufacturing Activities - GMP Manufacturing Campaigns of ATB200 Drug Substance and Drug Product Completed at 1000L Scale

$605.2M Cash Balance to Support Current Operating Plan and Invest in Rare Metabolic Disease Pipeline

Conference Call and Webcast Today at 8:30am ET

CRANBURY, N.J., May 08, 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 first quarter ended March 31, 2018. The Company also summarized recent program updates and reiterated full-year 2018 revenue and net cash spend guidance.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. stated, “Since the beginning of 2018, we have successfully executed across our five key strategic priorities for the year. We are on track to more than double our Galafold® revenue from last year, we secured approval of Galafold in Japan, and we have advanced our Pompe clinical, manufacturing and regulatory activities. Along with our key value drivers in Fabry and Pompe, we continue to evaluate other potential first- or best-in-class technologies that we believe may deliver significant benefits for many more people living with rare metabolic diseases. Today, more than ever, we are well positioned to achieve our vision to impact as many people as possible who are living with rare metabolic diseases as we continue to build a leading global rare disease biotech company.”

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 AT-GAA (also known as 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

First Quarter 2018 Financial Results and Full-Year 2018 Financial Guidance

- Total revenue in the first quarter 2018 was $16.7 million, a year-over-year increase of 300% from total revenue of $4.2 million in the first quarter of 2018.
- Cash, cash equivalents, and marketable securities totaled $605.2 million at March 31, 2018, compared to $358.6 million at December 31, 2017.
- Total operating expenses increased to $70.3 million for the first quarter 2018 compared to $55.4 million in the first quarter 2017 reflecting increased investment in Pompe clinical and manufacturing activities as well as Galafold commercial launch and launch preparations.
- Net cash spend was $48.0 million for the first quarter 2018.
- Net loss was $49.9 million, or $0.28 per share, compared to a net loss of $55.0 million, or $0.39 per share, for the first quarter 2017.

“The first quarter of 2018 marked another period of continued growth for Galafold and our strongest balance sheet in our Company’s history,” said Bradley L. Campbell, President and COO of Amicus Therapeutics. “We are confident in our ability to achieve the high end of our full-year 2018 Galafold revenue guidance given the current and anticipated increase in patient and physician adoption; expansion into new countries; and what we are seeing as a very high rate of compliance and adherence for this oral precision medicine.”

2018 Financial Guidance

For the full-year 2018 the Company anticipates total Galafold revenue at the high end of the $75 million to $85 million guidance range. The Galafold revenue guidance includes anticipated revenue from countries with final pricing and reimbursement, and does not currently include potential partial year contributions from Japan or the U.S.

The Company continues to expect full-year 2018 net cash spend between $230 million and $260 million. The current cash position, including proceeds from the February 2018 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 the Company’s future capital requirements.

Program Highlights

Migalastat for Fabry Disease
Amicus is committed to advancing the highest quality therapies for all people living with Fabry disease. Migalastat is an oral precision medicine intended to treat Fabry disease in patients 16 years or older who have amenable genetic mutations. Regulatory authorities in the European Union, Japan, Switzerland, Israel, Canada, Australia, and South Korea have granted full approval for migalastat under the trade name Galafold. In the U.S., the 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.

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:

- Pricing and reimbursement secured in 18 countries with first commercial patients treated in multiple new countries in 2018
- Approvals secured in EU, Australia, Canada, Japan, Israel, South Korea and Switzerland
- Launch team hired and trained in Japan to execute upcoming launch
- U.S. leadership, including Mike Keavany, to serve as Senior Vice President of the U.S. Business, and majority of field team now in place to support planned U.S. launch

Anticipated Milestones:

- Japanese launch (2Q18)
- U.S. FDA regulatory decision (3Q18)

Advanced and Targeted GAA (AT-GAA, also known as ATB200/AT2221) for Pompe Disease

AT-GAA 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 AT-GAA across ambulatory ERT-switch patients (Cohort 1), non-ambulatory ERT-switch patients (Cohort 2), and ERT-naïve patients (Cohort 3).

The Company is engaged in ongoing collaborative discussions with U.S. and EU regulators regarding a registration-directed study for full approval, manufacturing activities, and the best and fastest pathway forward for this novel treatment regimen. Amicus is pursuing formal scientific advice from the European Medicines Agency (EMA). A scientific advice meeting with the EMA is scheduled in 2Q18. The Company expects to provide an update on the results from that meeting by the end of 2Q. In the U.S., ongoing interactions on this program include a Type C meeting to occur in early 3Q18. Amicus expects to provide an FDA update in 3Q after receipt of written minutes from this meeting. The Company continues to believe that the evolving regulatory path will include a series of further iterative discussions with regulators as the program advances and as additional data are collected, including data from ongoing studies, data from new patients being enrolled and the results of a formal natural history study of Pompe patients receiving current ERT standard of care.

Amicus also continues to make progress with clinical and manufacturing activities to support the needs of the Pompe community.

Pompe Clinical Activities

- All 19 patients for whom extension study data have been previously reported continue treatment with AT-GAA in the ongoing Phase 1/2 ATB200-02 clinical study
- Currently enrolling Cohort 4 (up to 10 additional ambulatory ERT-switch patients) in ATB200-02 study
- Supportive studies underway including a 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; and a 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:

- As previously announced, 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
- GMP production runs of ATB200 drug substance and drug product completed at 1,000L commercial scale

Anticipated Upcoming Pompe Program Milestones:

- Full enrollment of additional patients in ATB200-02 clinical study
- Final demonstration of comparability between 1,000L GMP material and 250L material
- Pompe regulatory updates (2Q18 and 3Q18)
- Release of 1,000L GMP material for initiation of registration-directed study (2H18)
- 18-month data from ATB200-02 clinical study (4Q18)

Conference Call and Webcast
Amicus Therapeutics will host a conference call and audio webcast today, May 8, 2018, at 8:30 a.m. ET to discuss first quarter 2018 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: 6678368.

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 May 8, 2018. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); conference ID: 6678368.

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.

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-dedicated 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 AT-GAA, 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)

	Three Months Ended March 31,	
	2018	**2017**
Revenue:		
Net product sales	$ 16,696	$ 4,169
Cost of goods sold	2,615	775
Gross Profit	14,081	3,394
Operating Expenses:		
Research and development	40,798	30,876
Selling, general and administrative	27,396	19,132
Changes in fair value of contingent consideration payable	1,100	4,578
Depreciation	969	823
Total operating expenses	70,263	55,409
Loss from operations	(56,182)	(52,015)
Other income (expense):		
Interest income	1,737	759
Interest expense	(4,488)	(4,290)
Change in fair value of derivatives	4,861	-
Other income	2,764	610
Loss before income tax	(51,308)	(54,936)
Income tax benefit (expense)	1,392	(56)
Net loss attributable to common stockholders	$ (49,916)	$ (54,992)
Net loss attributable to common stockholders per common share — basic and diluted	$ (0.28)	$ (0.39)
Weighted-average common shares outstanding — basic and diluted	175,977,700	142,770,629

TABLE 2

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

	March 31, 2018	**December 31, 2017**
Assets		
Current assets:		
Cash and cash equivalents	$ 114,322	$ 49,060
Investments in marketable securities	448,198	309,502
Accounts receivable	10,836	9,464
Inventories	8,070	4,623
Prepaid expenses and other current assets	10,607	19,316
Total current assets	592,033	391,965
Investments in marketable securities	42,673	-
Property and equipment, less accumulated depreciation of $13,461 and $12,515 at March 31, 2018 and December 31, 2017, respectively	8,910	9,062

In-process research & development	23,000	23,000
Goodwill	197,797	197,797
Other non-current assets	5,592	5,200
Total Assets	$ 870,005	$ 627,024
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable, accrued expenses, and other current liabilities	$ 43,678	$ 53,890
Deferred reimbursements	7,750	7,750
Derivative liability	80,577	-
Contingent consideration payable	8,700	8,400
Total current liabilities	140,705	70,040
Deferred reimbursements	14,156	14,156
Convertible notes	166,768	164,167
Contingent consideration payable	17,800	17,000
Deferred income taxes	6,465	6,465
Other non-current liability	2,494	2,346
Total liabilities	348,388	274,174
Commitments and contingencies		
Stockholders' equity:		
Common stock, $0.01 par value, 250,000,000 shares authorized 187,972,218 and 166,989,790 shares issued and outstanding at March 31, 2018 and December 31, 2017, respectively	1,929	1,721
Additional paid-in capital	1,621,479	1,400,758
Accumulated other comprehensive loss:		
Foreign currency translation adjustment	(3,847)	(1,659)
Unrealized loss on available-for-sale securities	(877)	(436)
Warrants	16,076	16,076
Accumulated deficit	(1,113,143)	(1,063,610)
Total stockholders' equity	521,617	352,850
Total Liabilities and Stockholders' Equity	$ 870,005	$ 627,024

Source: Amicus Therapeutics, Inc.