August 7, 2017

Amicus Therapeutics Announces Second Quarter 2017 Financial Results and Corporate Updates

179 Fabry Disease Patients on Reimbursed Galafold (migalastat) as of July 31 - On Target to Reach 300 Patients by Year-End 2017

Migalastat NDA Submission Targeted for 4Q17

Phase 3 EB Topline Data and Complete Phase 1/2 Pompe Data on Track for Late 3Q17

CRANBURY, N.J., Aug. 07, 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 second quarter ended June 30, 2017. The Company also provided near-term program updates and reiterated full-year 2017 financial guidance.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, stated, "During the second quarter and into the third quarter we have continued to successfully execute across all five of our strategic priorities which include the international launch for our oral precision medicine Galafold (migalastat) for Fabry disease, the advancement of our lead clinical programs in Epidermolysis Bullosa and Pompe disease, and further strengthening of our balance sheet. Our progress during the first half of this year has been driven by our focused execution and groundbreaking science which is tremendous for patients as well as our shareholders. We have created a solid foundation to continue building on this momentum as we approach several important milestones in the second half of this year. I believe that today, more than ever before, Amicus is well-positioned to become a leading global biotechnology company with the potential to deliver significant benefits to people living with rare devastating diseases."

Second Quarter 2017 Financial Results

- Total revenue in the second quarter 2017 was approximately $7.2 million, a sequential increase of 71.4% from total revenue of $4.2 million in the first quarter 2017. 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 $227.2 million at June 30, 2017 compared to $330.4 million at December 31, 2016.
- Total operating expenses increased to $53.2 million compared to $48.5 million for the second quarter 2016 primarily due to increases in manufacturing scale-up investments on the Pompe program.
- Net cash spend was $103.5 million for the six months ending June 30, 2017.
- Net loss was $48.1 million, or $0.34 per share, compared to a net loss of $51.1 million, or $0.40 per share, for the second quarter 2016.

2017 Financial Guidance

Cash, cash equivalents, and marketable securities totaled $227.2 million at June 30, 2017 compared to $330.4 million at December 31, 2016. The Company added to the quarter-ending cash position with $243.2 million in net proceeds from a follow on public offering in July 2017.

Amicus 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, in addition to the net proceeds of $243.2 million from the follow-on public offering, is anticipated to fund ongoing operations into at least the second half of 2019.

Program Highlights

Migalastat for Fabry Disease

Migalastat is an oral precision medicine intended to treat Fabry disease in patients who have amenable genetic mutations. The European Commission (EC), in addition to regulatory authorities in Switzerland and Israel, have 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. In the U.S., as previously announced, the FDA has confirmed that Amicus may submit a new drug application (NDA) for migalastat.

International Launch and Expanded Access Programs (EAP) Updates:

- 179 patients (naïve and ERT-switch) on reimbursed Galafold as of July 31, 2017
- 12 countries with reimbursement (commercial or EAP) including the top four largest EU markets
- Reimbursement dossiers submitted and pricing discussions are now underway in 13 countries
- Target of 300 patients treated with reimbursed Galafold by year-end 2017

Global Regulatory Updates:

- Two additional approvals secured outside the EU (Switzerland and Israel)
- Regulatory submissions completed in seven additional territories outside the EU, including Japan, Canada and Australia
- U.S. FDA confirmed NDA submission may be based on existing data (no additional gastrointestinal symptoms study required)

Anticipated Upcoming Fabry Disease Program Milestones:

- International commercial launch and EAPs in additional countries
- Additional regulatory submissions including a U.S. NDA (4Q17)
- Regulatory decision in Japan (1H18)
- 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 functional data in initial patients were reported during the second quarter 2017 from an ongoing global Phase 1/2 clinical study (ATB200-02) to evaluate safety, tolerability, pharmacokinetics (PK), and pharmacodynamics (PD) of ATB200/AT2221. The study enrolled a total of 20 patients across three cohorts, 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:

- Full ATB200-02 study data, including 6-month functional data in all patients (late 3Q17)
- Meetings with US and EU regulators

SD-101 for Epidermolysis Bullosa (EB)

SD-101 is a novel, late-stage, proprietary topical treatment with the potential to be the first approved 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. As previously announced, Amicus has completed the analysis plan for the primary endpoints in the blinded ongoing ESSENCE study, and top-line Phase 3 data are on track for late in the third quarter of 2017. More than 95% of patients completing the primary treatment period have elected to continue in the open-label extension study

SD-101 was the first investigational treatment to show improvements in wound closure across all major EB types in completed Phase 2 clinical studies. SD-101 has been granted FDA Breakthrough Therapy designation, rare pediatric disease designation, and orphan drug designation.

Anticipated EB Program Milestones:

- Top-line Phase 3 data (late 3Q17)

Conference Call and Webcast
Amicus Therapeutics will host a conference call and audio webcast today, August 7, 2017 at 8:30 a.m. ET to discuss second 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 60127813.

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 60127813.

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 and our Quarterly Report on Form 10-Q for the quarter ended June 30, 2017. 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
(Unaudited)
(in thousands, except share and per share amounts)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Revenue:				
Net product sales	$ 7,158	$ —	$ 11,327	$ —
Cost of goods sold	1,061	—	1,836	—
Gross Profit	6,097	—	9,491	—
Operating Expenses:				
Research and development	31,985	18,281	62,861	41,706
Selling, general and administrative	19,311	19,300	38,443	35,001
Changes in fair value of contingent consideration payable	1,050	10,186	5,628	13,338
Restructuring charges	—	8	—	58
Depreciation	812	767	1,636	1,440
Total operating expenses	53,158	48,542	108,568	91,543
Loss from operations	(47,061)	(48,542)	(99,077)	(91,543)
Other income (expenses):				
Interest income	753	331	1,512	638
Interest expense	(4,179)	(1,055)	(8,469)	(2,000)
Other income (expense)	2,400	(2,237)	3,010	(2,289)
Loss before income tax (expense)/benefit	(48,087)	(51,503)	(103,024)	(95,194)
Income tax (expense)/ benefit	(49)	453	(105)	453
Net loss attributable to common stockholders	$ (48,136)	$ (51,050)	$ (103,129)	(94,741)
Net loss attributable to common stockholders per common share — basic and diluted	$ (0.34)	$ (0.40)	$ (0.72)	(0.75)
Weighted-average common shares outstanding — basic and diluted	143,000,718	129,122,175	142,886,614	127,160,943

TABLE 2

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

	June 30,	December 31,
	2017	2016
Assets		
Current assets:		
Cash and cash equivalents	$ 37,394	$ 187,026
Investments in marketable securities	189,838	143,325
Accounts receivable	3,786	1,304
Inventories	3,948	3,416
Prepaid expenses and other current assets	6,023	4,993
Total current assets	240,989	340,064
Property and equipment, less accumulated depreciation of $ 13,951 and $12,495 at June 30, 2017 and December 31, 2016, respectively	10,471	9,816
In-process research & development	486,700	486,700

Goodwill	197,797	197,797
Other non-current assets	3,009	2,468
Total Assets	$ 938,966	$ 1,036,845
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable, accrued expenses, and other current liabilities	$ 35,645	$ 41,008
Deferred reimbursements, current portion	18,850	13,850
Contingent consideration payable, current portion	46,188	56,101
Total current liabilities	100,683	110,959
Deferred reimbursements	16,906	21,906
Convertible notes	159,171	154,464
Contingent consideration payable	219,162	213,621
Deferred income taxes	173,869	173,771
Other non-current liability	2,283	1,973
Commitments and contingencies		
Stockholders' equity:		
Common stock, $0.01 par value, 250,000,000 shares authorized, 143,371,243 and 142,691,986 shares issued and outstanding at June 30, 2017 and December 31, 2016, respectively	1,485	1,480
Additional paid-in capital	1,132,229	1,120,156
Accumulated other comprehensive loss:		
Foreign currency translation adjustment, less tax expense of $ 1,293 at June 30, 2017 and December 31, 2016	(192)	1,945
Unrealized gain on available-for securities	31	102
Warrants	16,076	16,076
Accumulated deficit	(882,737)	(779,608)
Total stockholders' equity	266,892	360,151
Total Liabilities and Stockholders' Equity	$ 938,966	$ 1,036,845

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