Amicus Therapeutics Announces Third Quarter 2018 Financial Results and Corporate Updates

November 5, 2018

U.S. Galafold® (Migalastat) Fabry Launch Tracking Significantly Ahead of Expectations- 100+ Patients Prescribed Galafold Since August Launch

3Q18 Global Galafold Net Product Sales of $20.6M Driven by Continued International Growth - 500+ International Fabry Patients Now on Galafold

Reaffirming Higher End of FY18 Revenue Guidance of $80M-$90M –
Balance Sheet Strength Sufficient to Fund Operations into at least 2021

Gene Therapy Pipeline Provides 14 New Programs and Future Growth Platform

Conference Call and Webcast Today at 8:30am ET

CRANBURY, N.J., Nov. 05, 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 third quarter ended September 30, 2018. The Company also summarized recent program updates, reiterated its full-year 2018 revenue guidance and reduced its net cash spend guidance for the year.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. stated, “The third quarter marked a major transformation for Amicus that brings us several steps closer toward our 2023 vision to treat at least 5,000 patients and to achieve $1 billion in annual global revenue. In the last three months, we received our first U.S. drug approval for Galafold, expanded our pipeline to include 14 new gene therapy programs for rare metabolic diseases, and presented positive 18-month data for our differentiated Pompe treatment paradigm. With a strong revenue base and $500M+ peak sales opportunity for Galafold, as well as $564 million in cash, we have never been in a stronger position to advance our robust portfolio to drive significant value for shareholders and the patient communities that we serve.”

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

- Total revenue in the third quarter 2018 was $20.6 million, a year-over-year increase of 89% from total revenue of $10.9 million in the third quarter of 2017. Third quarter revenue was impacted by uneven ordering patterns over the summer months in Europe.
- Cash, cash equivalents, and marketable securities totaled $564.4 million at September 30, 2018, compared to $358.6 million at December 31, 2017.
- Total operating expenses for the third quarter 2018 were $172.5 million compared to $284.3 million in the third quarter 2017. The decrease is due primarily to a non-cash impairment charge incurred in 3Q17, partially offset by an upfront payment of $100 million for the Celenex asset acquisition which was reflected in the third quarter 2018 as a research and development expense.
- Net cash spend was $35.2 million for the third quarter 2018. Net loss was $159.2 million, or $0.84 per share, for the third quarter 2018 compared to a net loss of $111.7 million, or $0.69 per share, for the third quarter 2017. Total net operating expenses were $172.5 million, which includes the $100 million Celenex asset acquisition cost.

“We are very pleased with the momentum of the global Galafold launch,” said Bradley L. Campbell, President and Chief Operating Officer of Amicus Therapeutics. “We see continued strong uptake and growth, along with very high compliance and adherence to this new oral Fabry treatment option, in both patients who are ERT-experienced and an increasing number who are ERT-treatment naïve in Europe. As anticipated, quarter-over-quarter revenue reflect some uneven ordering patterns as well as a rising number of extended, 90-day prescriptions as we headed into the summer months, which have normalized since the start of the fourth quarter. These adoption trends may reflect the emerging Fabry treatment paradigm with a stable oral medication that can be taken during summer travels and on holidays. Japan is also off to a solid start, now with a double-digit number of patient prescriptions. Importantly, the first 12 weeks of the U.S. launch have significantly exceeded our expectations. With more than 100 individual prescriptions for Galafold in the U.S., we are seeing robust patient demand from a broad prescriber base of more than 40 Fabry physicians. Given this global momentum, we are confident in meeting the higher end of our full-year 2018 guidance and setting a solid foundation for 2019.”

2018 Financial Guidance

For the full-year 2018 the Company reiterated its total Galafold revenue guidance to $80 to $90 million. This reflects global revenue from all expected 2018 commercial markets. The Company is lowering its full-year 2018 net cash spend to $190 to $210 million from the previous range of $220 to $250 million. The current cash position, including Galafold revenues, is sufficient to fund ongoing operations into at least 2021. Potential future business development collaborations, pipeline expansion, and investment in manufacturing capabilities could impact the Company's future capital requirements.

Program Highlights

Galafold (Migalastat) Oral Precision Medicine for Fabry Disease
Galafold is an oral precision medicine for Fabry disease approved in the EU and other geographies to treat Fabry disease in patients 16 years or older who have amenable genetic mutations. The U.S. FDA approved Galafold under Subpart H for the treatment of adult patients with a confirmed diagnosis of Fabry disease and an amenable genetic variant. For patients who are not suitable for treatment with Galafold on the basis of their genetic mutations, or variants, Amicus is advancing a next-generation gene therapy.

Global Galafold Updates:

- U.S. FDA approval on August 10, 2018
- U.S. launch tracking ahead of internal expectations with 103 new patient prescriptions, also known as patient referral forms (PRFs), as of October 31, 2018. Time to shipment is approximately 60 days, limiting 2018 revenue impact but providing a strong foundation for 2019.
- Launched in Australia on November 1 following formal listing on life saving drugs program
- Pricing and reimbursement secured in 22 countries
- Approvals secured in eight total geographies including Australia, Canada, EU, Israel, Japan, South Korea, Switzerland, and United States and pending in Taiwan

AT-GAA 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 to stabilize ATB200 while in the circulation to deliver active therapeutic enzyme.

Positive results from a global Phase 1/2 clinical study (ATB200-02) have shown consistent and durable responses across key measures of safety, functional outcomes and biomarkers in both ERT-switch and ERT-naïve Pompe patients following up to 18 months of treatment with AT-GAA. The Company's strategy is to enhance the body of clinical data for AT-GAA in ongoing studies and the upcoming pivotal study (PROPEL, also referred to as ATB200-03) to deliver this potential new therapy to as many people living with Pompe disease as soon as possible.

Recent and Anticipated AT-GAA Program Milestones:

- Positive 18-month data from ATB200-02 Phase 1/2 clinical study at World Muscle Society
- 1,000L GMP material released for pivotal study.
- Initiation of PROPEL pivotal study to support full approval in U.S. and EU, and other geographies (4Q18)
- Completion of retrospective natural history study in approximately 100 ERT-treated Pompe patients (4Q18)
- Additional ATB200-02 study data from up to 10 additional ERT-switch patients in Cohort 4 (2019)
- Initiation of studies in additional patient populations, including pediatric patients (2019)
- Update on long-term manufacturing strategy

As part of the Company's long-term commitment to provide multiple solutions to address the significant unmet needs of the Pompe community, Amicus is also advancing a next-generation gene therapy as a potential cure for Pompe disease.

Gene Therapy Portfolio: 14 New Programs for Rare Metabolic Diseases
During the third quarter and early fourth quarter, Amicus expanded its pipeline [link here] to include 14 new gene therapy programs and future growth platform for rare metabolic diseases. The Company acquired 10 preclinical and clinical stage adeno associated virus (AAV) programs (intrathecal delivery) for neurologic lysosomal storage disorders (LSDs) currently in development at Nationwide Children's Hospital. In collaboration with the University of Pennsylvania, the Company is advancing four next-generation AAV gene therapies for Fabry disease, Pompe disease, CDKL5 deficiency disorder (CDD) and one additional undisclosed rare metabolic disorder.

Additional details are available in the third quarter 2018 results slide presentation at www.amicusrx.com in the Investors & Media section [link here].

Gene Therapy Pipeline Highlights:

- **Batten Disease**: Compelling proof-of-concept demonstrated in preclinical studies in CLN6, CLN3, and CLN8, as well as initial clinical safety and efficacy in a Phase 1/2 study in patients with CLN6.
- **Pompe Disease**: Early proof-of-principle for Amicus DNA constructs for an optimized gene therapy to address all aspects of Pompe disease including the central nervous sytem, heart, and muscles.
- **Fabry Disease**: Early proof-of-principle for Amicus DNA constructs for an optimized gene therapy to deliver stable, active enzyme to lysosomes.

Upcoming Gene Therapy Pipeline Milestones in 2018 and 2019:

- First Patient in CLN3 Batten disease Phase 1/2 Study (4Q18)
- Completion of enrollment in CLN6 Batten disease Phase 1/2 study
- Preliminary data from CLN6 Batten disease Phase 1/2 study
- Enrollment of full initial cohort in CLN3 Batten disease Phase 1/2 study
- Preclinical data for next-generation gene therapies for Fabry, Pompe and CDD
- Preclinical work across additional neurologic LSDs

Conference Call and Webcast

Amicus Therapeutics will host a conference call and audio webcast today, November 5, 2018, at 8:30 a.m. ET to discuss the third 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: 2292105.

An audio webcast and slide presentation 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 November 5, 2018. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); conference ID: 2292105.

About Galafold

Galafold™ (migalastat) 123 mg capsules is an oral pharmacological chaperone of alpha-Galactosidase A (alpha-Gal A) for the treatment of Fabry disease in adults who have amenable *GLA* variants. In these patients, Galafold works by stabilizing the body's own dysfunctional enzyme so that it can clear the accumulation of disease substrate. Globally, Amicus Therapeutics estimates that approximately 35 to 50 percent of Fabry patients may have amenable *GLA* variants, though amenability rates within this range vary by geography. Galafold is approved in Australia, Canada, European Union, Israel, Japan, South Korea, Switzerland and the U.S.

U. S. INDICATIONS AND USAGE

Galafold is indicated for the treatment of adults with a confirmed diagnosis of Fabry disease and an amenable galactosidase alpha gene (*GLA*) variant based on *in vitro* assay data.

This indication is approved under accelerated approval based on reduction in kidney interstitial capillary cell globotriaosylceramide (KIC GL-3) substrate. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials.

U.S. IMPORTANT SAFETY INFORMATION

ADVERSE REACTIONS

The most common adverse reactions reported with Galafold (≥10%) were headache, nasopharyngitis, urinary tract infection, nausea and pyrexia.

USE IN SPECIFIC POPULATIONS

There is insufficient clinical data on Galafold use in pregnant women to inform a drug-associated risk for major birth defects and miscarriage. Advise women of the potential risk to a fetus.

It is not known if Galafold is present in human milk. Therefore, the developmental and health benefits of breastfeeding should be considered along with the mother's clinical need for Galafold and any potential adverse effects on the breastfed child from Galafold or from the underlying maternal condition.

Galafold is not recommended for use in patients with severe renal impairment or end-stage renal disease requiring dialysis.

The safety and effectiveness of Galafold have not been established in pediatric patients.

To report Suspected Adverse Reactions, contact Amicus Therapeutics at 1-877-4AMICUS or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

For additional information about Galafold, including the full U.S. Prescribing Information, please visit https://www.amicusrx.com/pi/Galafold.pdf.

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. With extraordinary patient focus, Amicus Therapeutics is committed to advancing and expanding a robust pipeline of cutting-edge, first- or best-in-class medicines for rare metabolic diseases. For more information please visit the

company's website at www.amicusrx.com, and follow us on Twitter and LinkedIn.

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 as well as our Quarterly Report on Form 10-Q for the quarter September 30, 2018 to be filed November 6, 2018 with the Securities and Exchange Commission. 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 September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Revenue:				
Net product sales	$ 20,596	$ 10,874	$ 58,601	$ 22,201
Cost of goods sold	4,310	1,790	10,060	3,626
Gross Profit	16,286	9,084	48,541	18,575
Operating Expenses:				
Research and development	138,227	40,641	213,685	103,502
Selling, general and administrative	31,867	21,647	88,435	60,090
Changes in fair value of contingent consideration payable	1,300	(244,250)	2,700	(238,622)
Loss on impairment of assets	—	465,427	—	465,427
Depreciation	1,073	851	3,015	2,486
Total operating expenses	172,467	284,316	307,835	392,883
Loss from operations	(156,181)	(275,232)	(259,294)	(374,308)
Other income (expense):				
Interest income	2,721	1,190	7,371	2,702
Interest expense	(4,715)	(4,351)	(13,763)	(12,820)
Change in fair value of derivatives	—	—	(2,739)	163
Other (expense) income	(1,039)	2,044	(3,593)	4,891
Loss before income tax	(159,214)	(276,349)	(272,018)	(379,372)
Income tax benefit	51	164,683	1,104	164,578
Net loss attributable to common stockholders	$ (159,163)	$ (111,666)	$ (270,914)	$ (214,794)

Net loss attributable to common stockholders per common share — basic and diluted	$ (0.84)	$ (0.69)	$ (1.47)	$ (1.44)
Weighted-average common shares outstanding — basic and diluted	189,162,841	160,796,841	184,606,790	148,963,864

TABLE 2

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

	September 30, 2018	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 201,827	$ 49,060
Investments in marketable securities	362,556	309,502
Accounts receivable	14,189	9,464
Inventories	6,311	4,623
Prepaid expenses and other current assets	16,151	19,316
Total current assets	601,034	391,965
Property and equipment, less accumulated depreciation of $15,483 and $12,515 at September 30, 2018 and December 31, 2017, respectively	10,659	9,062
In-process research & development	23,000	23,000
Goodwill	197,797	197,797
Other non-current assets	6,099	5,200
Total Assets	$ 838,589	$ 627,024
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable, accrued expenses, and other current liabilities	$ 54,330	$ 53,890
Deferred reimbursements	2,750	7,750
Contingent consideration payable	8,800	8,400
Total current liabilities	65,880	70,040
Deferred reimbursements	14,156	14,156
Convertible notes	172,186	164,167
Senior secured term loan	146,622	—
Contingent consideration payable	19,300	17,000
Deferred income taxes	6,465	6,465
Other non-current liabilities	3,029	2,346
Total liabilities	427,638	274,174
Commitments and contingencies		
Stockholders' equity:		
Common stock, $0.01 par value, 500,000,000 and 250,000,000 shares authorized, 189,254,341 and 166,989,790 shares issued and outstanding at September 30, 2018 and December 31, 2017, respectively	1,941	1,721
Additional paid-in capital	1,731,174	1,400,758
Accumulated other comprehensive loss:		
Foreign currency translation adjustment	(875)	(1,659)
Unrealized gain on available-for-sale securities	(211)	(436)
Warrants	13,063	16,076
Accumulated deficit	(1,334,141)	(1,063,610)
Total stockholders' equity	410,951	352,850
Total Liabilities and Stockholders' Equity	$ 838,589	$ 627,024

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Source: Amicus Therapeutics, Inc.