Amicus Therapeutics Announces Third Quarter 2019 Financial Results and Corporate Updates

November 11, 2019

3Q19 Galafold® (migalastat) Revenue of $48.8M
and 1,000+ Patients on Therapy Reflects Continued Strong Global Uptake

Reiterating Upwardly Revised FY19 Revenue Guidance of $170M-$180M

Complete Enrollment of 120+ Patients in AT-GAA Pompe Pivotal Study on Track by YE19

Continued Progress Across Industry Leading Rare Disease Gene Therapy Portfolio

Strong Balance Sheet with $514M+ Cash Provides Cash Runway Well into 1H22

Conference Call and Webcast Today at 8:30 a.m. ET

CRANBURY, N.J., Nov. 11, 2019 (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, 2019. The Company also summarized recent program updates and reiterated its full-year 2019 guidance.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. stated, “The third quarter represented another consecutive period of significant growth and adoption for Galafold across all geographies, as well as continued momentum in our Pompe pivotal study and our gene therapy pipeline. We are on track to meet or exceed each of our key strategic priorities for the year as we lay the foundation for our long-term success. Importantly, we have significantly strengthened our financial outlook with careful management of our expenses and investments. With confidence in our base business and overall financial outlook, we are well capitalized to continue to grow our revenues, advance our pipeline, and move toward self-sustainability and profitability as we continue to build Amicus into a leading global rare disease biotechnology company delivering on our mission for patients and shareholders.”

Corporate Highlights for 3Q19 and Early 4Q19

- **Global revenue for Fabry precision medicine Galafold in the third quarter of 2019 was $48.8 million and continues to track toward the upwardly revised full-year 2019 revenue guidance of $170 million to $180 million.** Third quarter revenue represented a year-over-year increase of 137% from total revenue of $20.6 million in the third quarter of 2018, and a quarter over-quarter increase of 11% from total revenue of $44.1 million in the second quarter of 2019**.** As of September 30, 2019, Galafold represented an estimated 30% of global market share of treated amenable patients. Global compliance and adherence rates continue to exceed 90%.

- **Financial outlook strengthened with current cash runway now revised to well into 1H 2022 through major portfolio milestones and global growth.**

- **Positive Phase 2 clinical data for AT-GAA in Pompe disease**. Amicus presented initial six-month data in additional ERT-switch patients (Cohort 4) and full 24-month data from the first three cohorts in Phase 1/2 ATB200-02 clinical study at World Muscle Society.

- **Pompe Pivotal PROPEL study is expected to over-enroll (~120 Patients) by YE 2019.** Given the strong global interest among the Pompe patient and physician community for AT-GAA, which has U.S. Breakthrough Therapy designation, this global study is now expected to enroll ~120 patients by year-end 2019. Pompe manufacturing also continues to advance with PPQ runs now initiated at WuXi.

- **Positive interim Phase 1/2 clinical data for gene therapy in CLN6 Batten disease.** Initial results as well as additional supportive data at Child Neurology Society showed AAV-CLN6 gene therapy has the potential to halt the progression of a devastating disease that causes loss of brain function and is fatal in childhood.

- **Pompe gene therapy clinical candidate declared to move into IND-enabling studies**. Dose-ranging preclinical studies are currently underway to build off the initial preclinical results showing robust uptake and glycogen reduction in multiple tissues, including brain and spinal cord.

- **Robust portfolio of gene therapy programs and technologies provides foundation for future, including two clinical-stage programs (CLN6 and CLN3), and eight preclinical gene therapies.**

3Q19 Financial Results

- Total revenue in the third quarter 2019 was $48.8 million, a year-over-year increase of 137% from total revenue of $20.6 million in the third quarter of 2018, and a quarter over-quarter increase of 11% from total revenue of $44.1 million in the second quarter of 2019**.** On a constant currency basis, third quarter 2019 total revenue was $50.3 million, representing operational revenue growth measured at constant currency exchange rates of 143%, which was offset by a negative currency impact of $1.3 million, or 6%.

- Cash, cash equivalents, and marketable securities totaled $514.2 million at September 30, 2019, compared to $504.2 million at December 31, 2018.

- Total GAAP operating expenses of $100.5 million for the third quarter of 2019 decreased as compared to $172.5 million in the third quarter of 2018. The decrease is primarily due to an upfront payment of $100 million for the Celenex asset acquisition in 2018 partially offset by continued investments in the Galafold launch, Pompe clinical study program, and our gene therapy pipeline.

- Total non-GAAP operating expenses of $89.7 million for the third quarter of 2019 increased as compared to $63.0 million in the third quarter of 2018, reflecting continued investments in the Galafold launch, Pompe clinical study program, and our gene therapy pipeline. Full reconciliation of GAAP results to the Company's non-GAAP adjusted measures for all reporting periods appear in the tables to this press release.

- Net loss was $61.8 million, or $0.24 per share, compared to a net loss of $159.2 million, or $0.84 per share, for the third quarter 2018.

2019 Financial Guidance

Following the success in the first three quarters of the year, in addition to the strength in global Galafold launch metrics across all major geographies, Amicus raised the lower end of the full-year 2019 Galafold revenue guidance from $160 to $180 million to $170 to $180 million. The Company anticipates full-year 2019 non-GAAP operating expense of $410 million to $420 million. A reconciliation of the differences between the non-GAAP expectation and the corresponding GAAP measure is not available without unreasonable effort due to high variability, complexity and low visibility as to the items that would be excluded from the GAAP measure.

Cash, cash equivalents, and marketable securities totaled $514.2 million at September 30, 2019. Following a diligent review of current and outer year operating and capital expense projections, and robust outlook for Galafold revenue, Amicus now expects to end 2019 with more than $420 million in cash on hand and has extended the cash runway projection from 2021 to well into the first half of 2022.

2019 Key Strategic Priorities

- Nearly double annual worldwide revenue for Galafold with over 1,000 Fabry patients on Galafold by year end.

- Complete enrollment in pivotal Phase 3 PROPEL clinical study in Pompe disease and report additional Phase 2 data.

- Report additional two-year results from Phase 1/2 clinical study in CLN6 Batten disease and complete enrollment in ongoing CLN3 Batten disease Phase 1/2 study.

- Establish preclinical proof of concept for Fabry and Pompe gene therapies.

- Maintain a strong financial position.

Conference Call and Webcast

Amicus Therapeutics will host a conference call and audio webcast today, November 11, 2019 at 8:30 a.m. ET to discuss the third quarter 2019 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: 8654236.

A live 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 register on the website 15 minutes prior to the start of the call. A replay of the call will be available for seven days beginning at 11:30 a.m. ET on November 11, 2019. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); conference ID: 8654236.

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 Argentina, 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-dedicated 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 on Twitter and LinkedIn.

Non-GAAP Financial Measures
In addition to financial information prepared in accordance with U.S. GAAP, this press release also contains adjusted financial measures that we believe provide investors and management with supplemental information relating to operating performance and trends that facilitate comparisons between periods and with respect to projected information. These adjusted financial measures are non-GAAP measures and should be considered in addition to, but not as a substitute for, the information prepared in accordance with U.S. GAAP. We typically exclude certain GAAP items that management does not believe affect our basic operations and that do not meet the GAAP definition of unusual or non-recurring items. Other companies may define these measures in different ways. Full reconciliations of GAAP results to the comparable non-GAAP measures for the reported periods appear in the financial tables section of this press release. When we provide our expectation for non-GAAP operating expenses on a forward-looking basis, a reconciliation of the differences between the non-GAAP expectation and the corresponding GAAP measure generally is not available without unreasonable effort due to potentially high variability, complexity and low visibility as to the items that would be excluded from the GAAP measure in the relevant future period, such as unusual gains or losses. The variability of the excluded items may have a significant, and potentially unpredictable, impact on our future GAAP results.

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, Japan, the US 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, 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.

CONTACTS:

Investors:
Sara Pellegrino, IRC
Vice President, Investor Relations
spellegrino@amicusrx.com
(609) 662-5044

Media:
Christopher Byrne
Executive Director, Corporate Communications
cbyrne@amicusrx.com
(609) 662-2798

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TABLE 1

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	**2018**	**2019**	**2018**
Revenue:				
Net product sales	$ 48,768	$ 20,596	$ 126,944	$ 58,601
Cost of goods sold	5,596	4,310	15,018	10,060
Gross profit	43,172	16,286	111,926	48,541
Operating expenses:				
Research and development	58,892	138,227	194,466	213,685
Selling, general, and administrative	39,680	31,867	126,561	88,435
Changes in fair value of contingent consideration payable	789	1,300	2,652	2,700
Depreciation and amortization	1,116	1,073	3,261	3,015
Total operating expenses	100,477	172,467	326,940	307,835
Loss from operations	(57,305)	(156,181)	(215,014)	(259,294)
Other income (expense):				
Interest income	2,752	2,721	7,990	7,371
Interest expense	(4,026)	(4,715)	(15,105)	(13,763)
Loss on exchange of convertible notes	—	—	(40,624)	—
Change in fair value of derivatives	—	—	—	(2,739)
Other expense	(3,481)	(1,039)	(3,272)	(3,593)
Loss before income tax	(62,060)	(159,214)	(266,025)	(272,018)
Income tax benefit (expense)	251	51	(634)	1,104
Net loss attributable to common stockholders	$ (61,809)	$ (159,163)	$ (266,659)	$ (270,914)

Net loss attributable to common stockholders per common share — basic and diluted	$ (0.24)	$ (0.84)	$ (1.13)	$ (1.47)
Weighted-average common shares outstanding — basic and diluted	254,674,422	189,162,841	235,527,540	184,606,790

TABLE 2

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

	September 30, 2019	December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	$ 166,319	$ 79,749
Investments in marketable securities	347,875	424,403
Accounts receivable	33,731	21,962
Inventories	9,154	8,390
Prepaid expenses and other current assets	19,578	16,592
Total current assets	576,657	551,096
Operating lease right-of-use assets, less accumulated amortization of $2,420 and $0 at September 30, 2019 and December 31, 2018, respectively	35,814	—
Property and equipment, less accumulated depreciation of $17,907 and $15,671 at September 30, 2019 and December 31, 2018, respectively	34,673	11,375
In-process research & development	23,000	23,000
Goodwill	197,797	197,797
Other non-current assets	14,351	6,683
Total Assets	$ 882,292	$ 789,951
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable, accrued expenses, and other current liabilities	$ 81,475	$ 80,625
Deferred reimbursements	5,250	5,500
Operating lease liabilities	6,356	—
Total current liabilities	93,081	86,125
Deferred reimbursements	8,906	10,156
Convertible notes	2,096	175,006
Senior secured term loan	147,164	146,734
Contingent consideration payable	22,036	19,700
Deferred income taxes	6,465	6,465
Operating lease liabilities	49,686	—
Other non-current liabilities	4,591	2,853
Total liabilities	334,025	447,039
Commitments and contingencies		
Stockholders' equity:		
Common stock, $0.01 par value, 500,000,000 shares authorized, 254,772,163 and 189,383,924 shares issued and outstanding at September 30, 2019 and December 31, 2018, respectively	2,591	1,942
Additional paid-in capital	2,210,890	1,740,061
Accumulated other comprehensive loss:		
Foreign currency translation adjustment	1,156	495
Unrealized gain (loss) on available-for-sale securities	124	(427)
Warrants	12,387	13,063
Accumulated deficit	(1,678,881)	(1,412,222)
Total stockholders' equity	548,267	342,912
Total Liabilities and Stockholders' Equity	$ 882,292	$ 789,951

TABLE 3

Amicus Therapeutics, Inc.
Reconciliation of Non-GAAP Financial Measures

(in thousands)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	**2018**	**2019**	**2018**
Total operating expenses - as reported GAAP	**$ 100,477**	**$ 172,467**	**$ 326,940**	**$ 307,835**
Research and development:				
Share-based compensation	3,106	2,905	12,090	8,603
Research and development asset acquisition expense	-	100,000	-	100,000
Selling, general and administrative:				
Share-based compensation	5,737	4,149	19,432	12,270
Changes in fair value of contingent consideration payable	789	1,300	2,652	2,700
Depreciation and amortization	1,116	1,073	3,261	3,015
Total operating expense adjustments to reported GAAP	10,748	109,427	37,435	126,588
Total operating expenses - as adjusted Non-GAAP	$ 89,729	$ 63,040	$ 289,505	$ 181,247

Source: Amicus Therapeutics, Inc.