Amicus Therapeutics Announces Third Quarter 2021 Financial Results and Corporate Updates

November 9, 2021

3Q21 Total Galafold® Revenue of $79.5M – an 18% increase over 3Q20

Reiterating 2021 Revenue Guidance of $300M-$315M

AT-GAA BLA and NDA for Pompe Disease Accepted for Review by the U.S. FDA;
Marketing Authorization Applications Submitted to European Medicines Agency

On Track to Complete Planned Business Combination of Amicus Gene Therapy Business
with ARYA IV Resulting in the Launch of Caritas Therapeutics in Late 2021/Early 2022

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

PHILADELPHIA, Nov. 09, 2021 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD), a patient-dedicated global biotechnology company focused on discovering, developing, and delivering novel medicines for rare diseases, today announced financial results for the quarter ended September 30, 2021.

"In the second half of 2021, we have furthered our mission for people living with devastating rare diseases through the commercial execution of Galafold and advancement of the global regulatory filings and launch preparations for AT-GAA," stated, John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. "As previously announced, our teams are proceeding with the business combination of our leading gene therapy portfolio with ARYA IV to launch Caritas Therapeutics, a next-generation genetic medicines company. This transaction will serve patients and shareholders well by accelerating funding for our gene therapy pipeline, while simultaneously strengthening the financial profile of Amicus. We are immensely excited for what the future of science and biotechnology holds as we accelerate our commitment to extraordinary patient dedication."

Corporate Highlights

- **Global revenue for Galafold® (migalastat) in the third quarter of 2021 reached $79.5 million, representing a year-over-year increase of 18% from total revenue of $67.4 million in the third quarter of 2020.**

- **Long-term Galafold data published in the September 2021 Issue of *Molecular Genetics and Metabolism Reports* showing generally stable renal function in patients with an amenable variant during long-term treatment, up to 8.6 years, of Galafold, irrespective of treatment status, gender or phenotype.**

- **U.S. Food and Drug Administration (FDA) accepted for review the Biologics License Application (BLA) for cipaglucosidase alfa and the New Drug Application (NDA) for miglustat, the two components of AT-GAA.** The FDA has set a Prescription Drug User Fee Act (PDUFA) action date of May 29, 2022 for the NDA and July 29, 2022 for the BLA.

- **Marketing Authorization Applications (MAA) submitted to the European Medicines Agency (EMA) for AT-GAA in Pompe disease.** The filings are based on the evaluation of the effects of AT-GAA in people living with Pompe disease and its safety profile, which include data from the Phase 1/2 and Phase 3 PROPEL studies as well as data from the long-term open-label extension study.

- **Amicus and ARYA Sciences Acquisition Corp IV ("ARYA IV"), a special purpose acquisition company, announced their intent to launch a next-generation genetic medicine company, Caritas Therapeutics, Inc.** Through a definitive business combination agreement, the Amicus gene therapy business will be acquired by ARYA IV. The transaction will result in two independent publicly traded companies with attractive stand-alone investment profiles. Amicus will become the largest shareholder in Caritas with a ~36% ownership stake (assuming no redemptions by ARYA's shareholders) and retain co-development and commercialization rights to the Fabry and Pompe gene therapy programs, as well as negotiation rights on select future muscular dystrophy programs. The Form S-4 is now filed with the Securities and Exchange Commission and the launch of Caritas Therapeutics is expected in late 2021 or early 2022.

- **Cash position sufficient to achieve self-sustainability and profitability in 2023.** Following the transaction with ARYA IV, the previously announced ~$200 million private investment from leading biotechnology investors, and through careful management of expenses, the Company is on the path to achieve self-sustainability and profitability by 2023 as it executes on the global Galafold launch and AT-GAA global regulatory filings.

Third Quarter 2021 Financial Results

- Total revenue in the third quarter of 2021 was $79.5 million, a year-over-year increase of 18% from total revenue of $67.4 million in the third quarter of 2020. On a constant currency basis, third quarter 2021 total revenue was $78.5 million, representing operational revenue growth measured at constant currency exchange rates of 16.5%. Reported revenue was aided by a positive currency impact of $1.0 million, or 1.5%.
- Cash, cash equivalents, and marketable securities totaled $557.0 million at September 30, 2021, compared to $483.3 million at December 31, 2020.
- Total GAAP operating expenses of $110.2 million for the third quarter of 2021 decreased as compared to $111.8 million for the third quarter 2020, reflecting the timing of investments in our pipeline.
- Total non-GAAP operating expenses of $93.6 million for the third quarter of 2021 increased as compared to $92.4 million in the third quarter of 2020.[1]
- Net loss was reduced to $50.3 million, or $0.19 per share, compared to a net loss of $64.0 million, or $0.25 per share, for the third quarter 2020.

[1] 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.

2021 Financial Guidance

- For the full-year 2021, the Company anticipates total Galafold revenue of $300 million to $315 million. Double-digit revenue growth in 2021 is expected to be driven by continued operational growth and commercial execution across all major markets, including the U.S., EU, UK and Japan.
- Non-GAAP operating expense guidance for the full-year 2021 is $410 million to $420 million, driven by continued investment in the global Galafold launch, AT-GAA clinical studies and pre-launch activities, and advancing our gene therapy pipeline.[2]
- Based on current operating models, the Company believes that the current cash position and expected future revenues are sufficient to fund the Company's operations and ongoing research programs through to self-sustainability.

[2] 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.

2021 Milestones by Program

Galafold (migalastat) Oral Precision Medicine for Fabry Disease

- Continue revenue growth in 2021
- EU label expanded to cover adolescent population
- Continue geographic expansion
- Registry and other Phase 4 studies

AT-GAA for Pompe Disease

- BLA and NDA submissions accepted for review in 3Q21; EU MAA submissions completed in 4Q21
- Ongoing supportive studies, including pediatric and extension studies

Gene Therapy Portfolio

- Reported initial data from the CLN3 Batten disease gene therapy Phase 1/2 study; advance manufacturing activities and regulatory discussions to enable dosing additional patients with Good Manufacturing Practice (GMP) clinical-grade material
- Advance manufacturing activities and regulatory discussions for the CLN6 Batten disease gene therapy program
- Continue to progress IND-enabling studies, manufacturing activities and regulatory discussions in both Fabry and Pompe gene therapies
- Disclose additional preclinical data and potential IND candidate declarations across multiple preclinical programs
- Manufacturing advancements and updates across the portfolio

Conference Call and Webcast

Amicus Therapeutics will host a conference call and audio webcast today, November 9, 2021, at 8:30 a.m. ET to discuss the third quarter 2021 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: 3686792.

A live audio webcast and related presentation materials can also be accessed via the Investors section of the Amicus Therapeutics corporate website at ir.amicusrx.com. 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 9, 2021. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); conference ID: 3686792.

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 galactosidase alpha gene (*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 over 40 countries around the world, including the U.S., EU, U.K., Japan and others.

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 m2). The safety and efficacy of Galafold in children less than 12 years of age have not yet been established. No data are available.
- 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.
- Galafold 123 mg capsules are not for children (≥12 years) weighing less than 45 kg.
- 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, and revenue goals, including as they are impacted by COVID-19 related disruption, are based on current information. The potential impact on operations and/or revenue from the COVID-19 pandemic is inherently unknown and cannot be predicted with confidence and may cause actual results and performance to differ materially from the statements in this release, including without limitation, because of the impact on general political and economic conditions, including as a result of efforts by governmental authorities to mitigate COVID-19, such as travel bans, shelter in place orders and third-party business closures and resource allocations, manufacturing and supply chain disruptions and limitations on patient access to commercial or clinical product or to treatment sites. In addition to the impact of the COVID-19 pandemic, 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, UK, 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, commercialization 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 corporate financial guidance and financial goals and the attainment of such goals and 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, 2020 and Quarterly Report 10-Q for the quarter ended September 30, 2021, to be filed today. 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:
Andrew Faughnan
Executive Director, Investor Relations
afaughnan@amicusrx.com
(609) 662-3809

Media:
Diana Moore
Head of Global Corporate Communications
dmoore@amicusrx.com
(609) 662-5079

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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,	
	2021	2020	2021	2020
Net product sales	$ 79,545	$ 67,437	$ 223,360	$ 190,315
Cost of goods sold	11,696	8,399	26,615	21,627
Gross profit	67,849	59,038	196,745	168,688

Operating expenses:				
Research and development	59,333	70,419	186,453	229,150
Selling, general, and administrative	46,107	37,850	135,109	112,722
Changes in fair value of contingent consideration payable	3,288	1,034	4,780	2,680
Depreciation and amortization	1,520	2,496	4,691	6,299
Total operating expenses	110,248	111,799	331,033	350,851
Loss from operations	(42,399)	(52,761)	(134,288)	(182,163)
Other income (expense):				
Interest income	108	518	323	2,898
Interest expense	(8,165)	(6,784)	(24,307)	(14,148)
Loss on extinguishment of debt	(257)	(7,276)	(257)	(7,276)
Other income (expense)	237	3,019	(2,729)	29
Loss before income tax	(50,476)	(63,284)	(161,258)	(200,660)
Income tax benefit (expense)	182	(727)	(5,925)	(4,791)
Net loss attributable to common stockholders	$ (50,294)	$ (64,011)	$ (167,183)	$ (205,451)
Net loss attributable to common stockholders per common share — basic and diluted	$ (0.19)	$ (0.25)	$ (0.63)	$ (0.80)
Weighted-average common shares outstanding — basic and diluted	267,464,637	259,161,799	266,085,788	258,091,170

TABLE 2

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

	September 30, 2021	December 31, 2020
Assets		
Current assets:		
Cash and cash equivalents	$ 385,903	$ 163,240
Investments in marketable securities	171,057	320,029
Accounts receivable	51,427	46,923
Inventories	22,072	19,556
Prepaid expenses and other current assets	20,081	29,721
Total current assets	650,540	579,469
Operating lease right-of-use assets, net	21,270	23,296
Property and equipment, less accumulated depreciation of $18,789 and $14,487 at September 30, 2021 and December 31, 2020, respectively	41,991	43,863
In-process research & development	23,000	23,000
Goodwill	197,797	197,797
Other non-current assets	22,077	19,095
Total Assets	$ 956,675	$ 886,520
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 24,474	$ 17,063
Accrued expenses and other current liabilities	72,453	96,841
Contingent consideration payable	17,000	8,900
Operating lease liabilities	7,175	6,872
Total current liabilities	121,102	129,676
Deferred reimbursements	7,406	7,406
Long-term debt	388,719	389,254
Contingent consideration payable	7,605	16,925
Deferred income taxes	4,896	4,896
Operating lease liabilities	43,495	45,604
Other non-current liabilities	6,823	6,379
Total liabilities	580,046	600,140
Commitments and contingencies		
Stockholders' equity:		
Common stock, $0.01 par value, 500,000,000 shares authorized, 278,585,092 and 262,063,461 shares issued and outstanding at September 30, 2021 and December 31, 2020, respectively	2,805	2,650
Additional paid-in capital	2,579,953	2,308,578

Accumulated other comprehensive income (loss):		
Foreign currency translation adjustment	6,617	8,412
Unrealized loss on available-for-sale securities	(184)	(185)
Warrants	83	12,387
Accumulated deficit	(2,212,645)	(2,045,462)
Total stockholders' equity	376,629	286,380
Total Liabilities and Stockholders' Equity	$ 956,675	$ 886,520

TABLE 3

Amicus Therapeutics, Inc.
Reconciliation of Non-GAAP Financial Measures
(in thousands)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	**2020**	**2021**	**2020**
Total operating expenses - as reported GAAP	**$ 110,248**	**$ 111,799**	**$ 331,033**	**$ 350,851**
Research and development:				
Share-based compensation	3,775	8,626	13,232	17,241
Selling, general and administrative:				
Share-based compensation	8,066	7,282	30,699	19,671
Changes in fair value of contingent consideration payable	3,288	1,034	4,780	2,680
Depreciation and amortization	1,520	2,496	4,691	6,299
Total operating expense adjustments to reported GAAP	16,649	19,438	53,402	45,891
Total operating expenses - as adjusted	$ 93,599	$ 92,361	$ 277,631	$ 304,960

[2] 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.