Amicus Therapeutics Announces Second Quarter 2021 Financial Results and Corporate Updates

August 5, 2021

2Q21 Total Galafold® (migalastat) Revenue of $77.4M, a 24% increase over 2Q20

On-Track to Achieve Revenue Guidance of $300M-$315M

Completed the Rolling BLA and NDA Submissions to the U.S. FDA for AT-GAA in Pompe Disease

Positive EMA Rapporteur and Co-Rapporteur Meeting Support the MAA Submissions for AT-GAA; Global Submissions On-Track in 2021

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

PHILADELPHIA, Aug. 05, 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 June 30, 2021.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc., stated, “Throughout this year and into the third quarter, the global Amicus team has continued to advance our mission for patients and made significant strides towards achieving our 2021 strategic priorities, including continued commercial execution of Galafold, the completion of our rolling BLA submission with the U.S. FDA and progression of additional global regulatory work for AT-GAA, as well as advancing our industry-leading gene therapy pipeline. Through our efforts, we remain well positioned to deliver on our mission for patients and shareholders, and to continue building Amicus into a leading global rare disease biotechnology company. We are especially excited for and confident in our Pompe program now moving through regulatory reviews around the world and hopeful that it will reach many more people living with Pompe disease as soon as possible.”

Corporate Highlights

- **Global revenue for Galafold® (migalastat) in the second quarter of 2021 reached $77.4 million, representing a year-over-year increase of 24.0% from total revenue of $62.4 million in the second quarter of 2020.** Second quarter total revenue benefited from a positive currency impact of $4.3 million. On a constant currency basis, second quarter total revenue was $73.1 million, a growth of 17.2% measured at constant currency exchange rates.

- **Galafold EU label expanded following the European Commission approval for use in adolescents.** Galafold is the first and only oral therapy approved in the EU for the long-term treatment of adolescents with Fabry disease aged 12 to <16 years weighing ≥ 45 kg and who have an amenable mutation.

- **Rolling Biologics License Application (BLA) for cipaglucosidase alfa and the New Drug Application (NDA) for miglustat have been submitted to the U.S. Food and Drug Administration (FDA).**

- **In the European Union, following a positive rapporteur and co-rapporteur meeting, regulators are supportive of Marketing Authorization Application (MAA) submissions for AT-GAA in the second half of this year.**

- **AT-GAA granted positive scientific opinion through the Early Access to Medicines Scheme (EAMS) by the U.K.’s Medicines and Healthcare Products Regulatory Agency (MHRA).** The MHRA’s positive scientific opinion recognizes the high unmet medical need and permits eligible adults living with Late-Onset Pompe disease (LOPD) who have received alglucosidase alfa for at least 2 years to switch and have access to AT-GAA prior to marketing authorization in the U.K.

- **Clinical Batten gene therapy programs continue to advance**. The Company continues to follow the first 13 patients with CLN6 and the 4 patients with CLN3 in their respective Phase 1/2 studies. Focus remains on progressing manufacturing, clinical and regulatory activities to enable next clinical studies.

- **Cash position sufficient to achieve self-sustainability without the need for any future dilutive financings.** The Company continues to carefully manage expenses and investments, while executing on the Galafold launch, proceeding with AT-GAA global regulatory submissions and advancing development programs.

Second Quarter 2021 Financial Results

- Total revenue in the second quarter of 2021 was $77.4 million, a year-over-year increase of 24.0% from total revenue of $62.4 million in the second quarter of 2020. On a constant currency basis, second quarter 2021 total revenue was $73.1

million, representing operational revenue growth measured at constant currency exchange rates of 17.2%. Reported revenue was aided by a positive currency impact of $4.3 million, or 6.8%.

- Cash, cash equivalents, and marketable securities totaled $383.1 million at June 30, 2021, compared to $483.3 million at December 31, 2020.
- Total GAAP operating expenses of $107.9 million for the second quarter of 2021 increased as compared to $107.0 million for the second quarter 2020.
- Total non-GAAP operating expenses of $93.5 million for the second quarter of 2021 decreased as compared to $95.9 million in the second quarter of 2020, reflecting the timing of investments in our pipeline.[1]
- Net loss was $51.2 million, or $0.19 per share, compared to a net loss of $52.5 million, or $0.20 per share, for the second 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, U.K. 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

- Completed the BLA and NDA submissions in 3Q21; EU MAA submissions to be completed in 2H2021
- Ongoing supportive studies, including pediatric and extension studies

Gene Therapy Portfolio

- Advance manufacturing activities and regulatory discussions for the CLN6 Batten disease gene therapy program to enable dosing of additional patients with GMP clinical grade material
- 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 GMP clinical-grade material
- Continue to progress IND-enabling work in both Pompe and Fabry 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, August 5, 2021 at 8:30 a.m. ET to discuss the second 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: 7374935.

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 August 5, 2021. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); conference ID: 7374935.

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 June 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.

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TABLE 1

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2021	2020	2021	2020
Net product sales	$ 77,413	$ 62,353	$ 143,815	$ 122,878
Cost of goods sold	8,380	6,676	14,919	13,228
Gross profit	69,033	55,677	128,896	109,650
Operating expenses:				
Research and development	63,003	69,611	127,120	158,731
Selling, general, and administrative	42,276	34,657	89,002	74,872
Changes in fair value of contingent consideration payable	1,021	715	1,492	1,646
Depreciation and amortization	1,567	2,039	3,171	3,803
Total operating expenses	107,867	107,022	220,785	239,052
Loss from operations	(38,834)	(51,345)	(91,889)	(129,402)
Other income (expense):				

Interest income	50	865	215	2,380
Interest expense	(8,150)	(3,635)	(16,142)	(7,364)
Other expense	234	5,326	(2,966)	(2,990)
Loss before income tax	(46,700)	(48,789)	(110,782)	(137,376)
Income tax benefit (expense)	(4,525)	(3,703)	(6,107)	(4,064)
Net loss attributable to common stockholders	$ (51,225)	$ (52,492)	$ (116,889)	$ (141,440)
Net loss attributable to common stockholders per common share — basic and diluted	$ (0.19)	$ (0.20)	$ (0.44)	$ (0.55)
Weighted-average common shares outstanding — basic and diluted	266,398,516	257,973,329	265,384,865	257,548,623

TABLE 2

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

	June 30, 2021	December 31, 2020
Assets		
Current assets:		
Cash and cash equivalents	$ 176,538	$ 163,240
Investments in marketable securities	206,530	320,029
Accounts receivable	49,172	46,923
Inventories	24,086	19,556
Prepaid expenses and other current assets	24,176	29,721
Total current assets	480,502	579,469
Operating lease right-of-use assets, less accumulated amortization of $8,150 and $7,574 at June 30, 2021 and December 31, 2020, respectively	22,028	23,296
Property and equipment, less accumulated depreciation of $17,410 and $14,487 at June 30, 2021 and December 31, 2020, respectively	42,365	43,863
In-process research & development	23,000	23,000
Goodwill	197,797	197,797
Other non-current assets	21,200	19,095
Total Assets	$ 786,892	$ 886,520
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 13,762	$ 17,063
Accrued expenses and other current liabilities	71,325	96,841
Contingent consideration payable	19,800	8,900
Operating lease liabilities	7,106	6,872
Total current liabilities	111,993	129,676
Deferred reimbursements	7,406	7,406
Long-term debt	390,434	389,254
Contingent consideration payable	7,517	16,925
Deferred income taxes	4,896	4,896
Operating lease liabilities	44,201	45,604
Other non-current liabilities	6,535	6,379
Total liabilities	572,982	600,140
Commitments and contingencies		
Stockholders' equity:		
Common stock, $0.01 par value, 500,000,000 shares authorized, 266,532,536 and 262,063,461 shares issued and outstanding at June 30, 2021 and December 31, 2020, respectively	2,685	2,650
Additional paid-in capital	2,364,494	2,308,578
Accumulated other comprehensive income (loss):		
Foreign currency translation adjustment	9,255	8,412
Unrealized loss on available-for-sale securities	(173)	(185)
Warrants	—	12,387
Accumulated deficit	(2,162,351)	(2,045,462)
Total stockholders' equity	213,910	286,380
Total Liabilities and Stockholders' Equity	$ 786,892	$ 886,520

TABLE 3

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2021	2020	2021	2020
Total operating expenses - as reported GAAP	**$ 107,867**	**$ 107,022**	**$ 220,785**	**$ 239,052**
Research and development:				
Share-based compensation	3,152	3,362	9,457	8,615
Selling, general and administrative:				
Share-based compensation	8,584	5,046	22,633	12,389
Changes in fair value of contingent consideration payable	1,021	715	1,492	1,646
Depreciation and amortization	1,567	2,039	3,171	3,803
Total operating expense adjustments to reported GAAP	14,324	11,162	36,753	26,453
Total operating expenses - as adjusted	$ 93,543	$ 95,860	$ 184,032	$ 212,599