Amicus Therapeutics Announces First Quarter 2021 Financial Results and Corporate Updates

May 10, 2021

Galafold® (migalastat) Performance Reflects Continued Strong Adoption in All Key Global Regions; On-Track to Achieve Revenue Guidance of $300M-$315M

1Q21 Total Galafold Revenue of $66.4M Driven by Continued Global Growth

Positive Pre-BLA Meeting Held with U.S. FDA for AT-GAA in Pompe Disease; Rolling BLA Submission On-Track for Completion in 2Q21 with Global Submissions Expected Throughout 2021

New Data from Pompe and Fabry Gene Therapy Programs to be Presented at American Society of Gene & Cell Therapy 24th Annual Meeting May 11th-14th

Continue to Strengthen Senior Management Team with Addition of Sébastien Martel as SVP, Strategy & Business Development

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

PHILADELPHIA, May 10, 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 March 31, 2021.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc., stated, “Throughout the first quarter, we remained focused on furthering our vision for patients and on achieving our 2021 key strategic priorities, including the continued commercial growth of Galafold, progressing the regulatory and launch preparations for AT-GAA, and advancing our robust gene therapy pipeline. Through these efforts, we continued to fuel our mission to make a profound difference in the lives of patients.”

“We are very pleased with the continued momentum of the Galafold uptake globally,” said Bradley L. Campbell, President and Chief Operating Officer of Amicus Therapeutics, Inc. “In Q1, we saw an increase of patients on treatment and a continued high rate of compliance, despite quarter-over-quarter revenue being impacted by the typical uneven ordering patterns from Q4 to Q1 that were exacerbated by COVID. Importantly, the number of patients on Galafold at the end of the quarter were ahead of our internal estimates and the first weeks of Q2 continue to exceed expectations. Based on this momentum, and continuing to anticipate a second half recovery from COVID, we are confident in meeting our full-year 2021 guidance.”

Corporate Highlights

- **Global revenue for Galafold® (migalastat) in the first quarter of 2021 was $66.4 million.** First quarter revenue represented a year-over-year increase of 9.8% from total revenue of $60.5 million in the first quarter of 2020. On a constant currency basis, first quarter total revenue was $63.0 million, representing operational revenue growth measured at constant currency exchange rates of 4.1%, which was benefited by a positive currency impact of $3.4 million, or 5.7%.
- **Galafold momentum continues to track ahead of internal expectations and remains on track to achieve the full year revenue guidance of $300M to $315M.**
  - First quarter revenue reflected increased patient demand offset by the timing of orders in ex-U.S. geographies, reauthorizations in the U.S., and irregular ordering patterns due to COVID, which was consistent with our expectation of non-linear quarter-to-quarter growth.
  - In Q1, the number of patients on Galafold were ahead of the Company’s internal estimates as there was continued growth across the 30+ markets where Galafold is reimbursed.
  - The Company continues to expect the number of new patients starting on Galafold treatment in 2021 to be greater than in 2020.
  - Global compliance and adherence rates continue to exceed 90%.
- **On-track to complete the rolling BLA submission of AT-GAA in Pompe disease in the second quarter of this year** following positive written communication from a pre-BLA meeting with the U.S. FDA. Additional regulatory submissions in the European Union and in other geographies are expected throughout 2021.
- **Preclinical data from the Company’s Fabry and Pompe gene therapy clinical candidates to be presented at the American Society of Gene & Cell Therapy 24th Annual Meeting on May 11th-14th.** As part of the research collaboration with the Gene Therapy Program of the Perelman School of Medicine at the University of Pennsylvania (Penn), new non-human primate data from the Fabry AAV gene therapy program will be presented. In addition, new data from our optimized gene therapy candidate in Pompe disease, which in preclinical models prevented the development of muscle fiber pathology in young Pompe mice and reversed pre-existing muscle fiber pathology in aged Pompe mice, will be

presented. The data continue to validate the synergies of combining Amicus-engineered transgenes with Penn's AAV technologies to develop next-generation gene therapies.

- **Clinical Batten gene therapy programs continue to advance**. The Company continues to follow the first 13 CLN6 patients and the 4 CLN3 patients 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.

First Quarter 2021 Financial Results

- Total revenue in the first quarter 2021 was $66.4 million, a year-over-year increase of 9.8% from total revenue of $60.5 million in the first quarter of 2020. On a constant currency basis, first quarter 2021 total revenue was $63.0 million, representing operational revenue growth measured at constant currency exchange rates of 4.1%. Reported revenue was aided by a positive currency impact of $3.4 million, or 5.7%.
- Cash, cash equivalents, and marketable securities totaled $417.4 million at March 31, 2021, compared to $483.3 million at December 31, 2020.
- Total GAAP operating expenses of $112.9 million for the first quarter 2021 decreased as compared to $132.0 million for the first quarter 2020, reflecting the timing of investments in our pipeline.
- Total non-GAAP operating expenses of $90.5 million for the first quarter of 2021 decreased as compared to $116.7 million in the first quarter of 2020, reflecting the timing of investments in our pipeline.[1]
- Net loss was $65.7 million, or $0.25 per share, compared to a net loss of $88.9 million, or $0.35 per share, for the first 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]
- Cash, cash equivalents, and marketable securities totaled $417.4 million at March 31, 2021. 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.

Anticipated 2021 Milestones by Program

Galafold (migalastat) Oral Precision Medicine for Fabry Disease

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

AT-GAA for Pompe Disease

- Complete the BLA submission in 2Q21 and the EU MAA submission 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, May 10, 2021 at 8:30 a.m. ET to discuss the first 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: 5767104.

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 May 10, 2021. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); conference ID: 5767104.

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 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, 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 March 31, 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
(Unaudited)
(in thousands, except share and per share amounts)

	Three Months Ended March 31,	
	2021	2020
Net product sales	$ 66,402	$ 60,525
Cost of goods sold	6,539	6,552
Gross profit	59,863	53,973
Operating expenses:		
Research and development	64,117	89,120
Selling, general, and administrative	46,726	40,215
Changes in fair value of contingent consideration payable	471	931
Depreciation and amortization	1,604	1,764
Total operating expenses	112,918	132,030
Loss from operations	(53,055)	(78,057)
Other (expense) income:		
Interest income	165	1,515
Interest expense	(7,992)	(3,729)
Other expense	(3,200)	(8,316)
Loss before income tax	(64,082)	(88,587)
Income tax expense	(1,582)	(361)
Net loss attributable to common stockholders	$ (65,664)	$ (88,948)
Net loss attributable to common stockholders per common share — basic and diluted	$ (0.25)	$ (0.35)
Weighted-average common shares outstanding — basic and diluted	264,369,317	256,968,248

TABLE 2

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

	March 31, 2021	December 31, 2020
Assets		
Current assets:		
Cash and cash equivalents	$ 184,833	$ 163,240
Investments in marketable securities	232,596	320,029
Accounts receivable	44,931	46,923
Inventories	18,801	19,556
Prepaid expenses and other current assets	21,730	29,721
Total current assets	502,891	579,469
Operating lease right-of-use assets, less accumulated amortization of $7,499 and $7,574 at March 31, 2021 and December 31, 2020, respectively	22,363	23,296
Property and equipment, less accumulated depreciation of $15,961 and $14,487 at March 31, 2021 and December 31, 2020, respectively	43,445	43,863
In-process research & development	23,000	23,000
Goodwill	197,797	197,797
Other non-current assets	20,538	19,095
Total Assets	$ 810,034	$ 886,520
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 16,110	$ 17,063
Accrued expenses and other current liabilities	57,178	96,841
Contingent consideration payable	19,600	8,900
Operating lease liabilities	6,764	6,872
Total current liabilities	99,652	129,676
Deferred reimbursements	7,406	7,406
Long-term debt	389,789	389,254
Contingent consideration payable	6,696	16,925
Deferred income taxes	4,896	4,896
Operating lease liabilities	44,431	45,604
Other non-current liabilities	6,268	6,379
Total liabilities	559,138	600,140

Commitments and contingencies		
Stockholders' equity		
Common stock, $0.01 par value, 500,000,000 shares authorized, 266,007,718 and 262,063,461 shares issued and outstanding at March 31, 2021 and December 31, 2020, respectively	2,680	2,650
Additional paid-in capital	2,350,507	2,308,578
Accumulated other comprehensive income (loss):		
Foreign currency translation adjustment	9,020	8,412
Unrealized loss on available-for-sale securities	(185)	(185)
Warrants	—	12,387
Accumulated deficit	(2,111,126)	(2,045,462)
Total stockholders' equity	250,896	286,380
Total Liabilities and Stockholders' Equity	$ 810,034	$ 886,520

TABLE 3

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

	Three Months Ended March 31,	
	2021	**2020**
Total operating expenses - as reported GAAP	**$ 112,918**	**$ 132,030**
Research and development:		
Share-based compensation	6,305	5,253
Selling, general and administrative:		
Share-based compensation	14,049	7,343
Changes in fair value of contingent consideration payable	471	931
Depreciation and amortization	1,604	1,764
Total operating expense adjustments to reported GAAP	22,429	15,291
Total operating expenses - as adjusted	$ **90,489**	$ **116,739**