Amicus Therapeutics Announces Full-Year 2021 Financial Results and Corporate Updates

February 24, 2022

Galafold® Revenue Growth of 17% YoY to $306M in 2021

Galafold Global Sales Growing at Double-Digits (15-20%) with $350M-$365M in 2022

U.S. and EU Regulatory Filings Under Review and Launch Preparations Accelerating for AT-GAA in Pompe Disease

Amicus and ARYA IV Mutually Agree to Terminate Planned Business Combination Agreement

Strategic Portfolio and R&D Prioritization to Drive ~$400M in Operating Expense Savings Anticipated Through 2026

Profitability Projected in 2023

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

PHILADELPHIA, Pa., Feb. 24, 2022 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD), a patient-dedicated global biotechnology company focused on developing and commercializing novel medicines for rare diseases, today announced financial results for the full year ended December 31, 2021.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc., stated, “In 2022 we will continue to advance Galafold growth worldwide while securing AT-GAA approvals for global launches. We have also made the strategic decision now not to spin off our gene therapy programs and technologies. As a result, we are streamlining our portfolio and aligning our organization around a more focused R&D pipeline. These actions will remove approximately $400 million in operating expenses through 2026. We are strongly committed to profitability in 2023 and will continue to be self-sustaining without the need for any further equity financings. As we reach these major inflection points of a second approved medicine as well as profitability, we are taking a significant step forward toward our vision to be one of the world’s leading biotechnology companies focused on rare diseases.”

“We are very pleased with the continued strong uptake of Galafold globally, which is expected to drive double-digit revenue growth again in 2022. Our teams remain heavily focused on progressing the regulatory reviews and launch preparations for AT-GAA and ensuring that this novel treatment is available to people living with Pompe disease as quickly as possible upon approval,” stated Bradley Campbell, President and Chief Operating Officer of Amicus Therapeutics, Inc. “Amicus remains highly focused toward achieving profitability in 2023. In order to accomplish this, we will concentrate the vast majority of our efforts and investments in our priority growth franchises in Fabry disease and Pompe disease, including in next generation therapies. These necessary portfolio and accompanying organizational changes will enable Amicus to deliver sustainable long-term performance to continue to develop and deliver life-changing therapies.”

Corporate Highlights:

- **Global revenue for Galafold® (migalastat) in the full year of 2021 was $305.5 million**. Full year revenue represented a year-over-year increase of 17% from total revenue of $260.9 million in the full year of 2020. On a constant currency basis, full year 2021 total revenue was $298.6 million, representing operational revenue growth measured at constant currency exchange rates of 14%, which was further benefited by a positive currency impact of $6.9 million, or 3%. Galafold performance was driven largely by strong new patient accruals and sustained patient compliance and adherence rates.
- **AT-GAA regulatory reviews are underway:** In the U.S., the 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. In the EU, the Marketing Authorization Applications (MAA) were submitted and validated in the fourth quarter by the European Medicines Agency (EMA).
- **AT-GAA launch preparations accelerating:** Development of global launch plans, targeted investments in additional personnel, and launch inventory are fully underway as the Company believes AT-GAA has the potential become the new standard of care treatment regimen for people living with Pompe disease.
- **Amicus Therapeutics and ARYA Sciences Acquisition Corp IV, a special purpose acquisition company or SPAC, have agreed to mutually terminate the previously announced Business Combination Agreement originally entered into on September 29, 2021.** This decision results from unfavorable market conditions affecting IPOs, follow-on financings, and SPACs in the biotech sector as well as an increasingly challenging environment for stand-alone gene therapy companies. Neither party will be required to pay the other a termination fee as a result of the mutual decision to terminate the Business Combination Agreement.
- **Strategic portfolio and R&D alignment:** Amicus will focus and continue to invest in Galafold for Fabry disease and in AT-GAA for Pompe disease, while also investing in technologies that secure and advance the core Fabry and Pompe franchises. The Amicus Science team will continue to focus discovery efforts in core science and platform technologies to

address safe and efficient gene transfer. The prioritization of our gene therapy pipeline as well as alignment of our internal R&D organization is expected to result in approximately $400M in net savings through 2026, an approximately similar amount in savings associated with the previously announced business combination agreement and spin off.
- **Committed to achieving profitability in 2023.** Through this portfolio prioritization and careful management of expenses, the Company is on the path to achieve profitability[3] in 2023 as it executes on the global expansion of Galafold and prepares for the global launch of AT-GAA.

Full Year 2021 Financial Results

- Total revenue in the full year 2021 was $305.5 million, a year-over-year increase of 17% from total revenue of $260.9 million in the full year of 2020. On a constant currency basis, full year 2021 total revenue was $298.6 million, representing operational revenue growth measured at constant currency exchange rates of 14%. Reported revenue was aided by a positive currency impact of $6.9 million, or 3%.
- Cash, cash equivalents, and marketable securities totaled $482.5 million at December 31, 2021, compared to $483.3 million at December 31, 2020.
- Total GAAP operating expenses of $477.5 million for the full year 2021 were broadly stable as compared to $476.8 million for the full year 2020.
- Total non-GAAP operating expenses of $406.9 million for the full year of 2021 decreased as compared to $415.7 million in the full year of 2020, reflecting the timing of investments in our pipeline, partially offset by third-party costs.[1]
- Net loss was $250.5 million, or $0.92 per share, for the full year of 2021, and was reduced compared to a net loss of $276.9 million, or $1.07 per share, for the full year 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.

2022 Financial Guidance

- For the full-year 2022, the Company anticipates total Galafold revenue of at least $350 million to $365 million. Double-digit revenue growth (15-20%) in 2022 is expected to be driven by continued underlying demand from both switch and naïve patients, geographic expansion, the continued diagnosis of new Fabry patients 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 2022 is $470 million to $485 million, driven by continued investment in the global Galafold launch, AT-GAA clinical studies and pre-launch activities, in addition to certain non-recurring costs for manufacturing to support the global launch of AT-GAA and committed obligations for the gene therapy portfolio. In 2023, Amicus expects non-GAAP operating expense levels to come down to a similar level as in 2021.[2]
- Cash, cash equivalents, and marketable securities totaled $482.5 million at December 31, 2021. Based on current operating models, the Company believes that the current and projected cash flows are sufficient to achieve 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.

[3] Based on projections of Amicus non-GAAP Net Income under current operating plans, which includes successful AT-GAA regulatory approvals and continued Galafold growth. We define non-GAAP Net Income as GAAP Net Income excluding the impact of share-based compensation expense, changes in fair value of contingent consideration, depreciation and amortization, acquisition related income (expense), loss on extinguishment of debt, loss on impairment of assets, restructuring charges and income taxes.

Company Leadership Transition Update:

As previously announced in September 2021, **Bradley Campbell** will succeed **John F. Crowley** as CEO of Amicus Therapeutics. That transition will take place on August 1, 2022. At that time, Mr. Campbell will become President and Chief Executive Officer of Amicus. Mr. Crowley will become the Executive Chairman of Amicus for a two-year term effective upon the August 1, 2022 transition and serving as Executive Chairman until August 1, 2024, after which he is expected to continue as the non-executive Chairman of the Board.

Anticipated 2022 Milestones by Program

Galafold (migalastat) Oral Precision Medicine for Fabry Disease

- Sustain double-digit revenue growth in 2022 of $350 million to $365 million
- Continue geographic expansion
- Registry and other Phase 4 studies ongoing

AT-GAA for Pompe Disease

- U.S. Prescription Drug User Fee Act (PDUFA) action date of May 29, 2022 for the NDA and July 29, 2022 for the BLA

- EU Committee for Medicinal Products for Human Use (CHMP) opinion expected in late 2022
- Continue to broaden access through early access plans in the U.K., Germany, Japan, and other countries
- Ongoing supportive studies, including pediatric and extension studies

Conference Call and Webcast

Amicus Therapeutics will host a conference call and audio webcast today, February 24, 2022 at 8:30 a.m. ET to discuss the full year 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: 1792414.

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 February 24, 2022. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); conference ID: 1792414.

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 Fabry Disease
Fabry disease is an inherited lysosomal disorder caused by deficiency of an enzyme called alpha-galactosidase A (alpha-Gal A), which results from mutations in the GLA gene. The primary biological function of alpha-Gal A is to degrade specific lipids in lysosomes, including globotriaosylceramide (referred to here as GL-3 and also known as Gb3). Lipids that can be degraded by the action of alpha-Gal A are called "substrates" of the enzyme. Reduced or absent levels of alpha-Gal A activity lead to the accumulation of GL-3 in the affected tissues, including heart, kidneys, and skin. Accumulation of GL-3 and progressive deterioration of organ function is believed to lead to the morbidity and mortality of Fabry disease. The symptoms can be severe, differ from person to person, and begin at an early age.

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 Statement
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, including as they are impacted by COVID-19 related disruption, are based on current information. The potential impact on operations 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. 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, 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. Statements regarding corporate financial guidance and financial goals and the attainment of such goals. 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, 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)

	Years Ended December 31,		
	2021	**2020**	**2019**
Net product sales	$ 305,514	$ 260,886	$ 182,237
Cost of goods sold	34,466	31,044	21,963
Gross profit	271,048	229,842	160,274
Operating expenses:			
Research and development	272,049	308,443	286,378
Selling, general, and administrative	192,710	156,407	169,861
Changes in fair value of contingent consideration payable	6,514	3,144	3,297
Depreciation and amortization	6,209	8,846	4,775
Total operating expenses	477,482	476,840	464,311
Loss from operations	(206,434)	(246,998)	(304,037)
Other (expense) income:			
Interest income	509	3,226	10,249
Interest expense	(32,471)	(22,425)	(18,872)
Loss on exchange of convertible notes	—	—	(40,624)
Loss on extinguishment of debt	(257)	(7,276)	—
Other expense	(2,901)	(781)	(2,626)
Loss before income tax	(241,554)	(274,254)	(355,910)
Income tax (expense) benefit	(8,906)	(2,598)	(478)
Net loss attributable to common stockholders	$ (250,460)	$ (276,852)	$ (356,388)
Net loss attributable to common stockholders per common share — basic and diluted	$ (0.92)	$ (1.07)	$ (1.48)
Weighted-average common shares outstanding — basic and diluted	271,421,986	258,867,380	240,421,001

TABLE 2

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

	December 31,	
	2021	**2020**
Assets		
Current assets:		
Cash and cash equivalents	$ 245,197	$ 163,240
Investments in marketable securities	237,299	320,029
Accounts receivable	52,672	46,923
Inventories	26,818	19,556
Prepaid expenses and other current assets	34,848	29,721
Total current assets	596,834	579,469
Operating lease right-of-use assets, net	20,586	23,296
Property and equipment, less accumulated depreciation of $19,882 and $14,487 at December 31, 2021 and December 31, 2020, respectively	42,496	43,863
In-process research & development	23,000	23,000
Goodwill	197,797	197,797
Other non-current assets	24,427	19,095
Total Assets	$ 905,140	$ 886,520
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 21,513	$ 17,063
Accrued expenses and other current liabilities	98,153	96,841
Contingent consideration payable	18,900	8,900

Operating lease liabilities	7,409	6,872
Total current liabilities	145,975	129,676
Deferred reimbursements	5,906	7,406
Long-term debt	389,357	389,254
Contingent consideration payable	1,439	16,925
Deferred income taxes	4,930	4,896
Operating lease liabilities	43,363	45,604
Other non-current liabilities	6,801	6,379
Total liabilities	597,771	600,140
Commitments and contingencies		
Stockholders' equity:		
Common stock, $0.01 par value, 500,000,000 shares authorized, 278,912,800 and 262,063,461 shares issued and outstanding at December 31, 2021 and December 31, 2020, respectively	2,808	2,650
Additional paid-in capital	2,595,419	2,308,578
Accumulated other comprehensive loss:		
Foreign currency translation adjustment	5,251	8,412
Unrealized (loss) gain on available-for securities	(270)	(185)
Warrants	83	12,387
Accumulated deficit	(2,295,922)	(2,045,462)
Total stockholders' equity	307,369	286,380
Total Liabilities and Stockholders' Equity	$ 905,140	$ 886,520

TABLE 3

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

	December 31		
	2021	**2020**	**2019**
Total operating expenses - as reported GAAP	**$ 477,482**	**$ 476,840**	**$ 464,311**
Research and development:			
Share-based compensation	17,340	20,817	17,575
Selling, general and administrative:			
Share-based compensation	40,498	28,334	26,855
Changes in fair value of contingent consideration payable	6,514	3,144	3,297
Depreciation and amortization	6,209	8,846	4,775
Total operating expense adjustments to reported GAAP	70,561	61,141	52,502
Total operating expenses - as adjusted	$ **406,921**	$ **415,699**	$ **411,809**