Amicus Therapeutics Announces Full-Year 2020 Financial Results and Corporate Updates

March 1, 2021

2020 Total Galafold Revenue of $260.9M; Increased 43 Percent Year-on-Year

Continued Strong Global Growth of Galafold Expected in 2021 with Revenue of $300M-$315M

AT-GAA Rolling BLA Submission in Pompe Disease Planned for Completion in 2Q21 and Other Global Submissions Expected Throughout 2021

CLN6 Batten Disease Gene Therapy Granted Fast Track Designation by U.S. FDA

Cash Position Sufficient to Achieve Self-Sustainability

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

PHILADELPHIA, March 01, 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 full year ended December 31, 2020.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc., stated, “During 2020, Amicus remained resilient on our journey to becoming a leading global rare disease biotechnology company. Despite the challenges of COVID, Amicus emerged from 2020 a better and stronger company organizationally, strategically, scientifically and financially. Galafold continues its path to becoming the worldwide standard of care for Fabry patients with amenable variants. And we are steadfast and passionate in our commitment to advancing AT-GAA to global regulatory submissions as fast as possible for the benefit of all people living with Pompe disease. The data from the Phase 3 PROPEL study we believe continue to show the overwhelmingly positive benefits of AT-GAA compared to the only approved medicine in this devastating disease. And the unmet need in Pompe is so great. We have the team, the resources and the programs that strongly position us to achieve our vision of delivering groundbreaking new medicines and hopefully, one day, cures for people living with rare diseases.”

Corporate Highlights

- **Global revenue for Galafold® (migalastat) in the full year of 2020 was $260.9 million**. Full year revenue represented a year-over-year increase of 43% from total revenue of $182.2 million in the full year of 2019. On a constant currency basis, full year 2020 total revenue was $258.6 million, representing operational revenue growth measured at constant currency exchange rates of 42%, which was further benefited by a positive currency impact of $2.3 million, or 1%. Galafold performance was driven largely by strong patient demand. Global compliance and adherence rates continue to exceed 90%.

- **Results from the global Phase 3 PROPEL clinical study of AT-GAA in late-onset Pompe disease (LOPD) were presented at the 17th Annual WORLD*Symposium*™ 2021.**The Company plans to complete the BLA submission in the second quarter of this year and anticipates additional regulatory submissions in the European Union and in other geographies throughout 2021.

- **The U.S. Food and Drug Administration (FDA) granted Fast Track Designation to the CLN6 Batten disease gene therapy, AT-GTX-501.** The Fast Track program facilitates the development and accelerates the review of new drugs for serious conditions, which have the potential to address unmet medical needs. A drug development program with Fast Track designation is afforded greater access to the FDA for the purpose of expediting the drug's development, review and potential approval**.**

- **Initial clinical data from the Phase 1/2 CLN3 gene therapy study were presented at the 17th Annual WORLD*Symposium*™ 2021.**Results suggest early signs of disease stabilization and the potential to slow the neurological disease progression in children living with CLN3 Batten disease. Regulatory interactions are ongoing and the Company expects to submit the protocol for the next clinical study in the second half of this year.

- **Preclinical data from the Company’s Fabry disease gene therapy clinical candidate, AT-GTX-701, presented at the 17th Annual WORLD*Symposium*™ 2021.**As part of the research collaboration with the Gene Therapy Program of the Perelman School of Medicine at the University of Pennsylvania (Penn), initial data from the Fabry AAV gene therapy with an engineered GLA transgene improved for stability demonstrated greater substrate reduction than wild type constructs across all tissues and doses. These findings further validate the synergies of combining Amicus-engineered transgenes

with Penn's AAV technologies to develop next generation gene therapies.

- **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.

Full Year 2020 Financial Results

- Total revenue in the full year 2020 was $260.9 million, a year-over-year increase of 43% from total revenue of $182.2 million in the full year of 2019. On a constant currency basis, full year 2020 total revenue was $258.6 million, representing operational revenue growth measured at constant currency exchange rates of 42%. Reported revenue was aided by a positive currency impact of $2.3 million, or 1%.
- Cash, cash equivalents, and marketable securities totaled $483.3 million at December 31, 2020, compared to $452.7 million at December 31, 2019.
- Total GAAP operating expenses of $476.8 million for the full year 2020 increased as compared to $464.3 million for the full year 2019, reflecting continued investments in our pipeline offset by decreased travel and third-party costs.
- Total non-GAAP operating expenses of $415.7 million for the full year of 2020 increased as compared to $411.8 million in the full year of 2019, reflecting continued investments in our pipeline offset by decreased travel and third-party costs.[1]
- Net loss was $276.9 million, or $1.07 per share, compared to a net loss of $356.4 million, or $1.48 per share, for the full year 2019.

[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 at least $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 $483.3 million at December 31, 2020. Based on current operating models, the Company believes that the current cash position, which includes the net proceeds from the 2020 Senior Secured Term Loan, 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

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

AT-GAA for Pompe Disease

- Complete the BLA submission in 1H2021 and the EU MAA submission to be completed in 2H2021
- Ongoing supportive studies, including pediatric and extension studies

Gene Therapy Portfolio

- Advance manufacturing and regulatory discussions for the CLN6 Batten disease gene therapy program and begin dosing additional patients with GMP grade material
- Report initial data from the CLN3 Batten disease Phase 1/2 study, advance manufacturing and regulatory discussions, and submit protocol for next clinical study
- Continue to progress IND-enabling work in both Pompe and Fabry gene therapy
- 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, March 1, 2021 at 8:30 a.m. ET to discuss the full year 2020 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: 6959755.

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 March 1, 2021. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); conference ID: 6959755.

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 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,		
	2020	**2019**	**2018**
Net product sales	$ 260,886	$ 182,237	$ 91,245
Cost of goods sold	31,044	21,963	14,404
Gross profit	229,842	160,274	76,841
Operating expenses:			

Research and development	308,443	286,378	270,902
Selling, general, and administrative	156,407	169,861	127,200
Changes in fair value of contingent consideration payable	3,144	3,297	3,300
Depreciation and amortization	8,846	4,775	4,216
Total operating expenses	476,840	464,311	405,618
Loss from operations	(246,998)	(304,037)	(328,777)
Other (expense) income:			
Interest income	3,226	10,249	10,461
Interest expense	(22,425)	(18,872)	(22,402)
Loss on exchange of convertible notes	—	(40,624)	—
Loss on extinguishment of debt	(7,276)	—	—
Change in fair value of derivatives	—	—	(2,739)
Other expense	(781)	(2,626)	(5,632)
Loss before income tax	(274,254)	(355,910)	(349,089)
Income tax (expense) benefit	(2,598)	(478)	94
Net loss attributable to common stockholders	$ (276,852)	$ (356,388)	$ (348,995)
Net loss attributable to common stockholders per common share — basic and diluted	$ (1.07)	$ (1.48)	$ (1.88)
Weighted-average common shares outstanding — basic and diluted	258,867,380	240,421,001	185,790,021

TABLE 2

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

	December 31,	
	2020	**2019**
Assets		
Current assets:		
Cash and cash equivalents	$ 163,240	$ 142,837
Investments in marketable securities	320,029	309,903
Accounts receivable	46,923	33,284
Inventories	19,556	14,041
Prepaid expenses and other current assets	29,721	20,008
Total current assets	579,469	520,073
Operating lease right-of-use assets, less accumulated amortization of $7,574 and $5,342 at December 31, 2020 and December 31, 2019, respectively	23,296	33,315
Property and equipment, less accumulated depreciation of $14,487 and $17,604 at December 31, 2020 and December 31, 2019, respectively	43,863	47,705
In-process research & development	23,000	23,000
Goodwill	197,797	197,797
Other non-current assets	19,095	28,317
Total Assets	$ 886,520	$ 850,207
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 17,063	$ 21,722
Accrued expenses and other current liabilities	96,841	99,901
Contingent consideration payable	8,900	—
Operating lease liabilities	6,872	7,189
Total current liabilities	129,676	128,812
Deferred reimbursements	7,406	8,906
Long-term debt	389,254	149,505
Contingent consideration payable	16,925	22,681
Deferred income taxes	4,896	5,051
Operating lease liabilities	45,604	53,531
Other non-current liabilities	6,379	5,296
Total liabilities	600,140	373,782
Commitments and contingencies		
Stockholders' equity:		

Common stock, $0.01 par value, 500,000,000 shares authorized, 262,063,461 and 255,417,869 shares issued and outstanding at December 31, 2020 and December 31, 2019, respectively	2,650	2,598
Additional paid-in capital	2,308,578	2,227,225
Accumulated other comprehensive loss:		
Foreign currency translation adjustment	8,412	2,785
Unrealized (loss) gain on available-for securities	(185)	40
Warrants	12,387	12,387
Accumulated deficit	(2,045,462)	(1,768,610)
Total stockholders' equity	286,380	476,425
Total Liabilities and Stockholders' Equity	$ 886,520	$ 850,207

TABLE 3

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

	December 31		
	2020	**2019**	**2018**
Total operating expenses - as reported GAAP	**$ 476,840**	**$ 464,311**	**$ 405,618**
Research and development:			
Share-based compensation	20,817	17,575	11,740
Asset acquisition related expenses for in-process R&D	-	-	100,000
Selling, general and administrative:			
Share-based compensation	28,334	26,855	17,520
Changes in fair value of contingent consideration payable	3,144	3,297	3,300
Depreciation and amortization	8,846	4,775	4,216
Total operating expense adjustments to reported GAAP	61,141	52,502	136,776
Total operating expenses - as adjusted	$ **415,699**	$ **411,809**	**$ 268,842**