Amicus Therapeutics Announces Second Quarter 2020 Financial Results and Corporate Updates

August 10, 2020

Galafold 2Q2020 Revenue of $62.4 Million, On-Track to Achieve 2020 Revenue Guidance of $250M-$260M

AT-GAA Phase 3 PROPEL Study Readout and Rolling BLA Submission to U.S. FDA on Schedule

Advancing Industry-Leading Rare Disease Gene Therapy Portfolio

Path to Profitability Achieved without the Need for Any Future Dilutive Financings

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

CRANBURY, N.J. , Aug. 10, 2020 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD), a global, patient-dedicated biotechnology company focused on discovering, developing and delivering novel medicines for rare diseases, today announced financial results for the second quarter ended June 30, 2020. The Company also summarized recent program updates and reiterated its full-year 2020 guidance.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc., stated, “During the second quarter, we made tremendous progress advancing our mission for patients and are on track to achieve our 2020 key strategic priorities, including our global Fabry launch, Pompe late-stage development program, and advancing our industry-leading gene therapy pipeline. Following our strategic financing in July, Amicus is firmly on a path to profitability without the need to access the equity markets. Through these efforts, we remain strongly positioned 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 second quarter of 2020 was $62.4M**. Second quarter revenue represented a year-over-year increase of 41% from total revenue of $44.1 million in the second quarter of 2019. On a constant currency basis, second quarter 2020 total revenue was $63.3 million, representing operational revenue growth measured at constant currency exchange rates of 43%, which was offset by a negative currency impact of $1.0 million, or 2%.
- **Strong second quarter revenue represents the continued performance across the global business, including new patient starts from switch and naïve patients throughout the quarter in all major regions, including that hardest hit by COVID-19.** Performance driven largely by strong patient demand. Global compliance and adherence rates continue to exceed 90%.
- **1,000+ mutations added to the EU Galafold label**. This update includes a number of new mutations identified by creating and testing all possible single-base pair point mutations in the GLA gene. Including this list will help physicians assess treatment options in an expeditious manner. Amenable mutations continue to represent up to half of all people living with Fabry disease.
- **Global Phase 3 PROPEL clinical study of AT-GAA in late-onset Pompe disease (LOPD) remains on track for top line data in 1H21.** To date, 97%+ of the 2,810 planned infusions and assessments for the ongoing PROPEL study have been completed on schedule. The Company plans to initiate a rolling BLA for AT-GAA in the second half of 2020, completing final submission in the first half of 2021.
- **Expanded Access Program for infantile-onset Pompe patients underway.** Amicus has initiated an expanded access program for its investigational medicine AT-GAA for young children living with infantile-onset Pompe disease (IOPD).
- **Additional Phase 1/2 CLN6 data to be presented this year at the Child Neurology Society Annual Meeting in October.** Regulatory interactions are ongoing and the Company expects to provide feedback on the path forward in early 2021.
- **Initial data from the Phase 1/2 CLN3 study expected in early 2021 based on changes in medical conference schedules.** Regulatory interactions are ongoing and concurrent with the data, the Company expects to provide feedback on the regulatory pathway.
- **Positive preclinical Pompe gene therapy data presented at American Society of Cell and Gene Therapy meeting in May.** Positive results showed the Amicus engineered hGAA having better targeting and clearance of glycogen storage in Pompe mice. Preliminary data in non-human primates suggested therapeutically relevant expression levels in target organs.
- **Cash position sufficient to achieve self-sustainability without the need for any future dilutive financings.** The previously announced debt facility places Amicus firmly on a path to profitability, while the Company continues to carefully manage expenses and investments, while executing on the Galafold launch and advancing development programs.

2020 Key Strategic Priorities

- Achieve $250 million to $260 million of global product revenue for Galafold
- Complete Pompe Phase 3 PROPEL study, enroll pediatric studies and advance manufacturing to support 2021 BLA and MAA
- Advance clinical development, manufacturing and regulatory discussions for CLN6 and CLN3 Batten programs
- Progress Pompe gene therapy towards Investigational New Drug (IND) application and disclose up to two additional IND candidates
- Maintain strong financial position

Second Quarter 2020 Financial Results

- Total revenue in the second quarter 2020 was $62.4 million, a year-over-year increase of 41% from total revenue of $44.1 million in the second quarter of 2019. On a constant currency basis, second quarter 2020 total revenue was $63.3 million, representing operational revenue growth measured at constant currency exchange rates of 43%, which was offset by a negative currency impact of $1.0 million, or 2%.
- Cash, cash equivalents, and marketable securities totaled $309.6 million at June 30, 2020, compared to $452.7 million at December 31, 2019.
- Total GAAP operating expenses of $107.0 million for the second quarter 2020 decreased as compared to $115.2 million for the second quarter 2019, reflecting decreased travel and third-party costs, offset by continued investments in our gene therapy pipeline.
- Total non-GAAP operating expenses of $95.9 million for the second quarter of 2020 decreased as compared to $103.6 million in the second quarter of 2019, reflecting decreased travel and third-party costs, offset by continued investments in our gene therapy pipeline.[1]
- Net loss was $52.5 million, or $0.20 per share, compared to a net loss of $84.6 million, or $0.36 per share, for the second quarter 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.

2020 Financial Guidance

- For the full-year 2020, the Company anticipates total Galafold revenue of $250 million to $260 million based on the average exchange rates for 2019.
- Non-GAAP operating expense guidance for the full-year 2020 is $410 million to $420 million, driven by continued investment in the global Galafold launch, AT-GAA clinical studies, and advancing our gene therapy pipeline.[2]
- Cash, cash equivalents, and marketable securities totaled $309.6 million at June 30, 2020. Based on current operating models, the Company believes that the current cash position, along with the net proceeds from the 2020 Senior Secured Term Loan and expected revenues, is sufficient to fund the Company's operations and ongoing research programs through to profitability.

[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 2020 Milestones by Program

Galafold (migalastat) Oral Precision Medicine for Fabry Disease

- On track to meet full-year 2020 revenue guidance range of $250 million to $260 million
- Continued geographic expansion
- Registry and other Phase 4 studies underway

AT-GAA for Pompe Disease

- Plans to initiate a Rolling Biologics License Application (BLA) for AT-GAA in 2020, with addition of complete clinical results for PROPEL in 1H2021 to support full approval
- Retrospective natural history study data in approximately 100 Pompe Patients treated with enzyme replacement therapy
- Additional supportive studies, including an open-label study in 12- to <18-year-olds living with Pompe

Gene Therapy Portfolio

- Report further safety and efficacy data in the CLN6 Batten disease Phase 1/2 study and advance regulatory discussions to finalize clinical and regulatory path
- Initial data from the CLN3 Batten disease Phase 1/2 study expected in early 2021 and advance regulatory discussions to finalize clinical and regulatory path

- Continue IND-enabling toxicology work in Pompe disease and progress towards IND
- Additional preclinical data expected across multiple programs with disclosure of up to two additional IND candidates
- Manufacturing advancements across portfolio

Conference Call and Webcast
Amicus Therapeutics will host a conference call and audio webcast today, August 10, 2020 at 8:30 a.m. ET to discuss the second quarter 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: 4949075.

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 10, 2020. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); conference ID: 4949075.

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 *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, 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, 2019, the Quarterly Report filed on Form 10-Q for the quarter ended March 31, 2020, and the Quarterly Report filed on Form 10-Q 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 June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Net product sales	$ 62,353	$ 44,130	$ 122,878	$ 78,176
Cost of goods sold	6,676	5,367	13,228	9,422
Gross profit	55,677	38,763	109,650	68,754
Operating expenses:				
Research and development	69,611	70,981	158,731	135,574
Selling, general, and administrative	34,657	42,578	74,872	86,881
Changes in fair value of contingent consideration payable	715	480	1,646	1,863
Depreciation and amortization	2,039	1,154	3,803	2,145
Total operating expenses	107,022	115,193	239,052	226,463
Loss from operations	(51,345)	(76,430)	(129,402)	(157,709)
Other income (expense):				
Interest income	865	2,599	2,380	5,238
Interest expense	(3,635)	(4,625)	(7,364)	(11,079)
Loss on exchange of convertible notes	—	(4,501)	—	(40,624)
Other income (expense)	5,326	(877)	(2,990)	209
Loss before income tax	(48,789)	(83,834)	(137,376)	(203,965)
Income tax expense	(3,703)	(717)	(4,064)	(885)
Net loss attributable to common stockholders	$ (52,492)	$ (84,551)	$ (141,440)	$ (204,850)
Net loss attributable to common stockholders per common share — basic and diluted	$ (0.20)	$ (0.36)	$ (0.55)	$ (0.91)
Weighted-average common shares outstanding — basic and diluted	257,973,329	238,089,824	257,548,623	225,848,013

TABLE 2

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

	June 30, 2020	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$ 164,573	$ 142,837
Investments in marketable securities	145,017	309,903
Accounts receivable	43,040	33,284
Inventories	12,979	14,041
Prepaid expenses and other current assets	18,275	20,008
Total current assets	383,884	520,073
Operating lease right-of-use assets, less accumulated amortization of $6,219 and $5,342 at June 30, 2020 and December 31, 2019, respectively	23,949	33,315
Property and equipment, less accumulated depreciation of $21,194 and $17,604 at June 30, 2020 and December 31, 2019, respectively	46,945	47,705
In-process research & development	23,000	23,000
Goodwill	197,797	197,797
Other non-current assets	25,876	28,317
Total Assets	$ 701,451	$ 850,207
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 14,306	$ 21,722
Accrued expenses and other current liabilities	85,478	99,901
Operating lease liabilities	8,516	7,189
Total current liabilities	108,300	128,812
Deferred reimbursements	8,906	8,906
Convertible notes	2,203	2,131
Senior secured term loan	147,834	147,374
Contingent consideration payable	20,027	22,681
Deferred income taxes	5,051	5,051
Operating lease liabilities	43,666	53,531
Other non-current liabilities	4,511	5,296
Total liabilities	340,498	373,782

Commitments and contingencies		
Stockholders' equity:		
Common stock, $0.01 par value, 500,000,000 shares authorized, 258,223,842 and 255,417,869 shares issued and outstanding at June 30, 2020 and December 31, 2019, respectively	2,614	2,598
Additional paid-in capital	2,250,849	2,227,225
Accumulated other comprehensive loss:		
Foreign currency translation adjustment	4,865	2,785
Unrealized gain on available-for-sale securities	288	40
Warrants	12,387	12,387
Accumulated deficit	(1,910,050)	(1,768,610)
Total stockholders' equity	360,953	476,425
Total Liabilities and Stockholders' Equity	$ 701,451	$ 850,207

TABLE 3

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	**2019**	**2020**	**2019**
Total operating expenses - as reported GAAP	**$ 107,022**	**$ 115,193**	**$ 239,052**	**$ 226,463**
Research and development:				
Share-based compensation	3,362	3,952	8,615	8,984
Selling, general and administrative:				
Share-based compensation	5,046	5,983	12,389	13,695
Changes in fair value of contingent consideration payable	715	480	1,646	1,863
Depreciation and amortization	2,039	1,154	3,803	2,145
Total operating expense adjustments to reported GAAP	11,162	11,569	26,453	26,687
Total operating expenses - as adjusted	$ 95,860	$ 103,624	$ 212,599	$ 199,776