



Amicus Therapeutics Announces First Quarter 2019 Financial Results and Corporate Updates

May 8, 2019

1Q19 Galafold® (migalastat) Strong Launch Trends Continue with Largest Quarterly Number of Net New Patients Added Globally (150+ Net New Galafold Patients in 1Q19)

1Q19 Galafold Revenue of \$34.0M and Early 2Q19 Momentum on Key Launch Metrics

Maintaining FY19 Revenue Guidance of \$160M-\$180M and 1,000+ Fabry Patients on Galafold Globally

AT-GAA in Pompe Disease Continues to Advance in PROPEL Pivotal Study with Full Enrollment on Track by Year-End

Gene Therapy Focus Advances with Positive Preclinical Pompe Gene Therapy Data and Significant Progress Across All Gene Therapy Programs - On Track to Report Additional Phase 1/2 Clinical Data in CLN6 Batten Disease in Mid-2019 (Q3)

Strong Balance Sheet with \$438.3M+ Cash

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

CRANBURY, N.J., May 08, 2019 (GLOBE NEWSWIRE) -- [Amicus Therapeutics](#) (Nasdaq: FOLD), a global biotechnology company focused on discovering, developing and delivering novel medicines for rare metabolic diseases, today announced financial results for the first quarter ended March 31, 2019. The Company also summarized recent program updates and reiterated its full-year 2019 guidance.

Corporate Highlights for 1Q19 and Early 2Q19

- **Global revenue for Galafold (migalastat) in the first quarter of 2019 was \$34.0M, in line with management expectations, and continues to track toward full-year 2019 revenue guidance of \$160M-\$180M and 1,000+ patients by year-end.** The first quarter revenue represented a year-over-year increase of 104% from total revenue of \$16.7 million in the first quarter of 2018.
- **Patient adoption and wide prescriber experience continues globally for Galafold.** Highest number since launch of net new patients added in 1Q19 (150+) with higher than anticipated prescriptions in EU, Japan and US, and continued global compliance and adherence rates exceeding 90%. Over 200 patient referral forms (PRFs) written now by over 90 physicians in the United States, with continued strong reimbursement from payors, since the launch in August 2018.
- **New intellectual property issued for Galafold.** The new patent covers a method of treatment and provides additional protection through 2038.
- **Positive Initial Proof-of-Concept for Preclinical Pompe Gene Therapy Data.** Positive preclinical [results](#) showed robust uptake and glycogen reduction in multiple tissues, including brain and spinal cord. Additional preclinical studies underway to select a clinical candidate in 2019. This program is significantly ahead of schedule reflecting a strong collaboration with the University of Pennsylvania.
- **Enrollment momentum in ongoing pivotal PROPEL study in Pompe disease.** The PROPEL study is now underway at 24 participating sites and is on track to achieve full enrollment of ~100 patients by year-end 2019. Significant enthusiasm from Pompe patient and physician community continues.
- **Additional ERT-Switch Patients (Cohort 4) Fully Enrolled in Pompe Phase 1/2 Clinical Study:** Initial 6-month data in Cohort 4, and complete 24-month data in Cohorts 1-3, anticipated in 2H19.
- **Additional data from Phase 1/2 CLN6 Batten disease study anticipated 3Q19.** A total of 12 patients have received a single administration of AAV9-CLN6 gene therapy (exposure ranging from ~5 to 39 months). The top-line data are expected to include two-year results in approximately half the patients in 3Q19 with more detail at a scientific congress later in 2019.
- **Low dose cohort fully enrolled in Phase 1/2 study in CLN3 Batten disease.** A total of three patients were dosed in the low dose cohort in the CLN3 Batten disease study with no serious adverse events to date following a single administration of AAV9-CLN3 gene therapy. The high dose cohort is expected to commence in the coming months.

- **Robust gene therapy pipeline continues to advance toward important data milestones.** Additional preclinical proof-of-concept data in Pompe, initial preclinical data in Fabry, and further advancement of neurologic lysosomal disorder preclinical programs, expected throughout 2019.
- **Strong financial position to continue executing the Galafold launch, advance development programs and build leadership position in gene therapy science, development and manufacturing capabilities and capacity.** The current cash position of approximately \$438.3 million at March 31, 2019 is expected to fund ongoing operations into at least mid-2021.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. stated, "During the first quarter and into the second quarter, we have made tremendous progress in executing across our five key strategic priorities for 2019. With the success of the global Galafold launch and long-term opportunity in Fabry, our leadership position in Pompe disease with a late-stage Phase 3 program and now positive preclinical data in gene therapy, and a rapidly advancing gene therapy pipeline that includes two clinical-stage programs in Batten disease, we are marching closer toward our vision to treat 5,000+ patients and achieve \$1B+ in revenue by 2023. As we continue to build a leading global biotechnology company in rare metabolic diseases, specifically within the field of human genomic medicine, we remain more focused than ever on developing groundbreaking new medicines and hopefully one day cures, and delivering them to people living with these life-threatening conditions as quickly as possible."

First Quarter 2019 Financial Results

- Total revenue in the first quarter 2019 was \$34.0 million, a year-over-year increase of 104% from total revenue of \$16.7 million in the first quarter of 2018. This includes year-over-year operational revenue growth measured at constant currency exchange rates of 114.9%, offset by negative currency impact of \$1.8M, or 11.0%.
- Cash, cash equivalents, and marketable securities totaled \$438.3 million at March 31, 2019, compared to \$504.6 million at December 31, 2018.
- Total operating expenses of \$111.3 million for the first quarter of 2019 increased as compared to \$70.3 million in the first quarter of 2018, reflecting continued investments in the Galafold launch, Pompe program, and gene therapy pipeline.
- Net loss was \$120.3 million, or \$0.56 per share, compared to a net loss of \$49.9 million, or \$0.28 per share, for the first quarter 2018.

2019 Key Strategic Priorities

- Nearly double annual revenue for Galafold (FY19 guidance of \$160M-\$180M in worldwide revenue) with 1,000+ Fabry patients on Galafold by year end.
- Complete enrollment in pivotal study in Pompe disease and report additional Phase 2 data.
- Report additional two-year results from Phase 1/2 clinical study in CLN6 Batten disease and complete enrollment in ongoing CLN3 Batten disease Phase 1/2 study.
- Establish preclinical proof of concept for Fabry and Pompe gene therapies.
- Maintain a strong financial position.

2019 Financial Guidance

For the full-year 2019 the Company anticipates total Galafold revenue of \$160 million to \$180 million. Growth in 2019 is expected to be driven by continued growth in EU markets, further geographic expansion, and successful adoption in the first full year of launch in the U.S. and Japan. Consistent with Galafold adoption trends and ordering patterns seen in previous years, Amicus expects higher revenue growth in the second and fourth quarters of 2019.

Cash, cash equivalents, and marketable securities totaled \$438.3 million at March 31, 2019. The Company expects to end 2019 with approximately \$300 million in cash on hand. The current cash position is anticipated to fund ongoing operations into at least mid-2021.

Anticipated 2019 Milestones by Program

Galafold (migalastat) Oral Precision Medicine for Fabry Disease

- On track to meet full-year 2019 revenue guidance range of \$160 million to \$180 million.

AT-GAA for Pompe Disease

- Initial 6-month data in additional ERT-switch patients (Cohort 4) in Phase 1/2 ATB200-02 clinical study.
- Retrospective natural history study data in approximately 100 ERT-treated Pompe patients.
- Additional supportive studies, including an open-label study in pediatric patients.
- Full enrollment in Phase 3 PROPEL study.
- Advance agreed upon CMC requirements to support BLA.

Gene Therapy Pipeline:

- Additional two-year data from CLN6 Batten disease Phase 1/2 study.
- Full enrollment of ongoing CLN3 Batten disease Phase 1/2 study.
- Additional preclinical data including next-generation gene therapies for Fabry and Pompe
- Selection of Pompe AAV gene therapy clinical candidate to move into IND-enabling studies

Conference Call and Webcast

Amicus Therapeutics will host a conference call and audio webcast today, May 8, 2019 at 8:30 a.m. ET to discuss the first quarter 2019 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: 8358807.

A live audio webcast can also be accessed via the Investors section of the Amicus Therapeutics corporate website at <http://ir.amicusrx.com/>, and will be archived for 30 days. 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 8, 2019. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); conference ID: 8358807.

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 Australia, Canada, European Union, Israel, Japan, South Korea, Switzerland and the U.S.

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 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

Operational revenue growth excluding the impact of foreign currency exchange rates is a non-GAAP financial measure and should not be considered a replacement for, and should be read together with, the most comparable GAAP financial measure.

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, 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. 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, 2018. 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, | |
|-----------------------------------------------------------|-------------------------------------|-------------|
| | 2019 | 2018 |
| Revenue: | | |
| Net product sales | \$ 34,046 | \$ 16,696 |
| Cost of goods sold | 4,055 | 2,615 |
| Gross profit | 29,991 | 14,081 |
| Operating expenses: | | |
| Research and development | 64,593 | 40,798 |
| Selling, general and administrative | 44,303 | 27,396 |
| Changes in fair value of contingent consideration payable | 1,383 | 1,100 |

| | | | |
|-----------------------------------------------------------------------------------|--------------------|---------------------|----------|
| Depreciation and amortization | 991 | 969 | |
| Total operating expenses | 111,270 | 70,263 | |
| Loss from operations | (81,279 |) (56,182 |) |
| Other income (expense): | | | |
| Interest income | 2,639 | 1,737 | |
| Interest expense | (6,454 |) (4,488 |) |
| Loss on exchange of convertible notes | (36,123 |) — | |
| Change in fair value of derivatives | — | 4,861 | |
| Other income | 1,086 | 2,764 | |
| Loss before income tax | (120,131 |) (51,308 |) |
| Income tax (expense) benefit | (168 |) 1,392 | |
| Net loss attributable to common stockholders | \$ (120,299 |) \$ (49,916 |) |
| Net loss attributable to common stockholders per common share — basic and diluted | \$ (0.56 |) \$ (0.28 |) |
| Weighted-average common shares outstanding — basic and diluted | 213,519,287 | 175,977,700 | |

TABLE 2

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

| | March 31, 2019 | December 31, 2018 |
|--------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|---------------------------|----------------------------------|
| Assets | | |
| Current assets: | | |
| Cash and cash equivalents | \$ 96,349 | \$ 79,749 |
| Investments in marketable securities | 341,978 | 424,403 |
| Accounts receivable | 22,960 | 21,962 |
| Inventories | 8,167 | 8,390 |
| Prepaid expenses and other current assets | 13,592 | 16,592 |
| Total current assets | 483,046 | 551,096 |
| Operating and finance lease right-of-use assets, less accumulated amortization of \$2,111 and \$0 at March 31, 2019 and December 31, 2018, respectively | 36,308 | — |
| Property and equipment, less accumulated depreciation of \$15,794 and \$15,671 at March 31, 2019 and December 31, 2018, respectively | 13,286 | 11,375 |
| In-process research & development | 23,000 | 23,000 |
| Goodwill | 197,797 | 197,797 |
| Other non-current assets | 11,265 | 6,683 |
| Total Assets | \$ 764,702 | \$ 789,951 |
| Liabilities and Stockholders' Equity | | |
| Current liabilities: | | |
| Accounts payable, accrued expenses, and other current liabilities | \$ 60,596 | \$ 80,625 |
| Deferred reimbursements | 2,750 | 5,500 |
| Operating and finance lease liabilities | 2,523 | — |
| Total current liabilities | 65,869 | 86,125 |
| Deferred reimbursements | 11,406 | 10,156 |
| Convertible notes | 22,052 | 175,006 |
| Senior secured term loan | 146,766 | 146,734 |
| Contingent consideration payable | 20,767 | 19,700 |
| Deferred income taxes | 6,465 | 6,465 |
| Operating and finance lease liabilities | 36,100 | — |
| Other non-current liabilities | 3,609 | 2,853 |
| Total liabilities | 313,034 | 447,039 |
| Commitments and contingencies | | |
| Stockholders' equity: | | |
| Common stock, \$0.01 par value, 500,000,000 shares authorized, 230,180,714 and 189,383,924 shares issued and outstanding at March 31, 2019 and December 31, 2018, respectively | 2,347 | 1,942 |
| Additional paid-in capital | 1,970,607 | 1,740,061 |
| Accumulated other comprehensive loss: | | |
| Foreign currency translation adjustment | (1,309 |) 495 |

| | | |
|---------------------------------------------------------|-------------------|-------------------|
| Unrealized gain (loss) on available-for-sale securities | 157 | (427) |
| Warrants | 12,387 | 13,063 |
| Accumulated deficit | (1,532,521) | (1,412,222) |
| Total stockholders' equity | 451,668 | 342,912 |
| Total Liabilities and Stockholders' Equity | \$ 764,702 | \$ 789,951 |



Source: Amicus Therapeutics, Inc.