Amicus Therapeutics Provides Full-Year 2019 Strategic Outlook and Financial Guidance

January 7, 2019

Full-Year 2018 Galafold Revenue of ~$91M Exceeds $80M-$90M Guidance

2019 Galafold Revenue Expected to Nearly Double – with Guidance of $160M-$180M

Pompe Phase 3 PROPEL Study Expected to Complete Enrollment and Additional Phase 2 Pompe Data in 2019

Additional 2-Year Data from Phase 1/2 CLN6 Batten Disease Clinical Study Anticipated Mid-Year 2019

Ongoing Phase 1/2 CLN3 Batten Disease Study Expected to Complete Enrollment in 2019

Preclinical Proof of Concept for Fabry and Pompe Gene Therapy Programs Expected in 2019

Strong Balance Sheet with $500M+ Cash

CRANBURY, N.J., Jan. 07, 2019 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD), a global biotechnology company focused on discovering, developing and delivering novel medicines for rare metabolic diseases, today provided unaudited preliminary Galafold revenue for the full-year 2018 and introduced its full-year 2019 strategic outlook and financial guidance.

During 2018 Amicus met or exceeded all five key strategic priorities:

- **More than doubled global revenue for Galafold (migalastat)**. Revenue grew from $36.9 million in full-year 2017 to approximately $91 million (preliminary and unaudited) in full-year 2018, exceeding the high end of the full-year 2018 guidance range of $80 million to $90 million.
- **Successfully secured approvals for migalastat in the U.S. and Japan, with strong initial adoption**. As of December 31, 2018, 149 patients in the U.S. have been prescribed Galafold since the August launch.
- **Achieved clinical, manufacturing and regulatory milestones to advance AT-GAA toward global regulatory submissions and approvals**. Highlights included positive 12- and 18-month data from the ongoing Phase 1/2 clinical study, manufacturing scale up (1000L), and first patient dosed in the PROPEL pivotal study.
- **Pipeline expanded to include 14 new gene therapy programs, including two clinical programs in Batten disease**, **exceedingtarget of least one new clinical program in 2019**. Target enrollment has been achieved in the Phase 1/2 study in CLN6 Batten disease, and the first patient has been dosed in the Phase 1/2 study in CLN3 Batten disease.
- **Maintained and strengthened the balance sheet**. The current cash position of approximately $505 million (preliminary and unaudited) at December 31, 2018 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 2018 we advanced several steps closer to our 2023 vision to treat at least 5,000 patients and achieve $1 billion in global revenue. Following the new approvals for our Fabry precision medicine Galafold in the U.S. and Japan, and continued growth momentum in international markets, we have exceeded our 2018 guidance. Patients are also now being treated in multiple Amicus clinical studies, including our Phase 1/2 and pivotal studies of AT-GAA for Pompe disease, as well as Phase 1/2 studies of our investigational gene therapies for CLN3 and CLN6 Batten disease. Today we are in a stronger position than ever to become a leading global biotechnology company focused on transforming the lives of people living with these rare, life-threatening conditions and creating significant value for our shareholders.”

Amicus is focused on the following five key strategic priorities in 2019:

- Nearly double again 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 CLN-3 Batten disease Phase 1/2 study
- Establish preclinical proof of concept for Fabry and Pompe gene therapies
- Maintain a strong financial position

Mr. Crowley will discuss Amicus' corporate objectives and key milestones in a presentation at the 37th Annual J.P. Morgan Healthcare Conference on Tuesday, January 8, 2019 at 8:30 a.m. PT (11:30 a.m. ET). A live webcast of the presentation can be accessed through the Investors section of the Amicus Therapeutics corporate web site at http://ir.amicusrx.com/events.cfm, and will be archived for 90 days.

Full-Year 2018 Financial Summary and 2019 Guidance

Amicus recorded approximately $91 million (preliminary and unaudited) in full-year 2018 revenue from commercial sales and reimbursed expanded

access programs for Galafold. For the full-year 2019 the Company anticipates total Galafold revenue of $160 million to $180 million. Prescription growth in 2018 was largely driven by EU and other countries outside the U.S. and Japan. Growth in 2019 is expected to be driven by continued growth in EU markets, further geographic expansion, and further success from the first full year of launch in the U.S. and Japan.

Cash, cash equivalents, and marketable securities totaled just over $500 million (preliminary and unaudited) at December 31, 2018. 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.

Program Highlights

Galafold (Migalastat) Oral Precision Medicine for Fabry Disease
Galafold is an oral precision medicine for Fabry disease approved in the EU and other geographies to treat Fabry disease in patients 16 years or older who have amenable genetic mutations. The U.S. FDA approved Galafold under Subpart H for the treatment of adult patients with a confirmed diagnosis of Fabry disease and an amenable genetic variant. An estimated 35% to 50% of the global Fabry population may be suitable for treatment with Galafold on the basis of their genetic mutations, or variants. For patients who are not suitable for treatment with Galafold on the basis of their genetic mutations, or variants, Amicus is advancing a next-generation gene therapy.

Global Galafold Updates:

- 650+ patients (naïve and ERT-switch) on reimbursed Galafold worldwide as of December 31, 2018.
- Approvals secured in eight geographies including Australia, Canada, EU, Israel, Japan, South Korea, Switzerland, and United States and pending in Taiwan and several additional markets.
- U.S. launch exceeded internal expectations with 149 new patient prescriptions, also known as patient referral forms (PRFs), as of December 31, 2018. Time to shipment was up to 60 days, limiting 2018 revenue impact but providing a strong foundation for 2019.
- Pricing and reimbursement secured in 24 countries.
- Registry and other Phase 4 supportive studies underway.

AT-GAA for Pompe Disease
AT-GAA is a novel treatment paradigm in Phase 3 development that consists of ATB200, a unique recombinant human acid alpha-glucosidase (rhGAA) enzyme with optimized carbohydrate structures, particularly mannose 6-phosphate (M6P), to enhance uptake, co-administered with AT2221, a pharmacological chaperone. Positive results from a global Phase 1/2 clinical study (ATB200-02) have shown consistent and durable responses across key measures of safety, functional outcomes and biomarkers in both ERT-switch and ERT-naïve Pompe patients following up to 18 months of treatment with AT-GAA.

The Company's strategy is to enhance the body of clinical data for AT-GAA in ongoing clinical studies, including the pivotal study (PROPEL, also referred to as ATB200-03) to deliver this potential new therapy to as many people living with Pompe disease as soon as possible. Based on regulatory feedback from both the U.S. FDA and European Medicines Agency (EMA), the PROPEL study is expected to support approval for a broad indication, including ERT-switch and treatment-naïve patients.
Pompe Program Updates:

- 1000L scale material released for pivotal PROPEL study.
- Dosing initiated in PROPEL study.
- WuXi partnership strengthened with 5-year supply agreement.

Anticipated Pompe Program Milestones in 2019:

- New data from the Phase 1/2 ATB200-02 clinical study, including final 24-month data in Cohorts 1-3, and initial 6-month data in additional ERT-switch patients (Cohort 4).
- 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 Programs for Rare Metabolic Diseases
During the third quarter and early fourth quarter of 2018, Amicus expanded its pipeline and future growth platform [link here] to include 14 new gene therapy programs and future growth platform for rare metabolic diseases, including 10 preclinical and clinical stage adeno associated virus 9 (AAV9) programs (intrathecal delivery) for neurologic lysosomal storage disorders (LSDs). Together these 10 programs have the potential to address 10,000+ people living with these neurologic LSDs and represent a $1 billion recurring revenue opportunity. Amicus is also developing four next-generation AAV gene therapies for Fabry disease, Pompe disease, CDKL5 deficiency disorder (CDD) and one additional undisclosed rare metabolic disorder.

In Batten disease, compelling proof-of-concept has been demonstrated in preclinical studies in CLN6, CLN3, and CLN8, as well as initial clinical safety and efficacy in a Phase 1/2 study in patients with CLN6. The Company has also shown early proof-of-principle for Amicus DNA constructs for optimized gene therapies for Fabry and Pompe diseases.

Gene Therapy Program Updates:

- First patient treated in CLN3 Batten disease Phase 1/2 study with no serious adverse events reported to date.
- Target enrollment achieved in CLN6 Batten disease Phase 1/2 study, with 12 patients receiving a single administration of

gene therapy (exposure ranging from ~1 to 34 months).

Anticipated Gene Therapy Pipeline Milestones in 2019:

- Additional two-year data from CLN6 Batten disease Phase 1/2 study.
- Full enrollment of ongoing CLN3 Batten disease Phase 1/2 study.
- Preclinical data for next-generation gene therapies for Fabry, Pompe and CDD.
- Preclinical work across additional neurologic LSDs.

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 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 us on Twitter and LinkedIn.

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 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, 2017 as well as our Quarterly Report on Form 10-Q for the quarter September 30, 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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