Amicus Therapeutics Provides Full-Year 2018 Strategic Outlook and Financial Guidance

January 8, 2018

310+ People with Fabry Disease Treated with Reimbursed Galafold® at YE17

FY18 Galafold Revenue Guidance of $75M-$85M

Regulatory Agreement on Comparability of 1,000L Scale Engineering Material for Pompe

Additional Pompe Phase 1/2 Clinical Study Results to be Presented at WORLDSymposium™ 2018

CRANBURY, N.J., Jan. 08, 2018 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD), a global biotechnology company focused on discovering, developing and delivering novel cutting-edge medicines for rare metabolic diseases, today provided its full-year 2018 strategic outlook and financial guidance.

Key 2017 Accomplishments

- Exceeded “Target 300” goal with more than 310 people treated with reimbursed Galafold™ (migalastat) oral precision medicine for Fabry disease at year-end 2017. Full-year 2017 Galafold revenue totaled approximately $36 million.
- Completed global regulatory submissions for migalastat in Japan (J-NDA), the U.S. (NDA), and other key geographies
- Established important clinical proof-of-concept for novel, highly differentiated Pompe treatment regimen ATB200/AT2221 on safety, functional outcomes and key disease biomarkers
- Successfully scaled manufacture of Pompe biologic engineering batches at commercial scale (1,000L) with capacity plans to ensure that entire Pompe population can be served as quickly as possible
- Strengthened balance sheet with total cash, cash equivalents and marketable securities of $359 million at December 31, 2017 and cash runway into the second half of 2019

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. stated, “During 2017 we continued to build a leading global rare disease biotech company while advancing our vision to maximize the impact of our medicines for people living with rare diseases. We exceeded our EU launch and regulatory objectives for our Fabry precision medicine Galafold, and we reported clinical data from our Pompe clinical study that lays the foundation for a potential new treatment paradigm for this muscle disease. Throughout 2018 we are poised to create significant additional value for patients and shareholders across our key programs in Fabry and Pompe, and through our focused commitment to advancing and expanding a pipeline of novel medicines for rare metabolic diseases.”

Amicus is focused on five key strategic priorities in 2018:

- Double global revenue for Galafold ($75 million - $85 million)
- Secure approvals for migalastat in Japan and the U.S.
- Achieve clinical, manufacturing and regulatory milestones to advance ATB200/AT2221 toward global regulatory submissions and approvals as soon as possible
- Develop and expand preclinical pipeline to ensure at least one new clinical program in 2019
- Maintain a strong balance sheet

Mr. Crowley will discuss Amicus' corporate objectives and key milestones in a presentation at the 36th Annual J.P. Morgan Healthcare Conference on Tuesday, January 9, 2018 at 8:00 a.m. PT (11:00 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 2017 Financial Summary and 2018 Guidance

Amicus recorded approximately $36 million in full-year 2017 revenue from commercial sales and reimbursed expanded access programs for Galafold. For the full-year 2018 the Company anticipates total Galafold revenue of $75 million to $85 million.

Cash, cash equivalents, and marketable securities totaled approximately $359 million at December 31, 2017. As previously announced, the Company strengthened the balance sheet during 2017 with a $243.0 million in net proceeds from a follow on public offering in July 2017. The Company expects full-year 2018 net cash spend of between $230 million and $260 million. The current cash position is anticipated to fund ongoing operations into at least the second half of 2019.

Program Highlights

Migalastat for Fabry Disease
Migalastat is an oral precision medicine intended to treat Fabry disease in patients who have amenable genetic mutations. Regulatory authorities in the European Union, Switzerland, Israel, Canada, Australia, and South Korea have granted full approval for migalastat under the trade name Galafold. The EU approval may serve as the basis for regulatory approvals in more than two-thirds of the global Fabry market that is outside the U.S. In the

U.S., Amicus submitted a new drug application (NDA) to the FDA in December 2017 to seek approval of migalastat.

Amicus is committed to advancing the highest quality therapies for all people living with Fabry disease. For people with non-amenable mutations who are not eligible for migalastat as an oral precision medicine, the Company has established initial preclinical proof-of-concept for a novel Fabry ERT (ATB101) co-formulated with migalastat as part of our CHART® platform.

Global Fabry Updates:

- 310+ patients (naïve and ERT-switch) on reimbursed Galafold as of December 31, 2017
- Total full-year 2017 revenue of $36 million from global commercial sales and expanded access programs (EAPs)
- Pricing and reimbursement secured in 15 countries
- Approvals secured in EU, Australia, Canada, Israel, South Korea and Switzerland
- Approvals pending in Japan, U.S. and other key geographies

Anticipated Milestones:

- FDA acceptance of U.S. NDA for filing (1Q18)
- Regulatory decision on Japanese J-NDA (1H18)
- Total full-year 2018 revenue guidance of $75 million to $85 million
- ATB101 co-formulated with migalastat advancing toward the clinic in 2019

ATB200/AT2221 for Pompe Disease

ATB200/AT2221 is a novel treatment paradigm 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. Throughout 2017, Amicus presented a cascade of positive data from an ongoing global Phase 1/2 clinical study (ATB200-02) to evaluate safety, tolerability, pharmacokinetics (PK), and pharmacodynamics (PD) of ATB200/AT2221 across ambulatory ERT-switch patients (Cohort 1), non-ambulatory ERT-switch patients (Cohort 2), and ERT-naïve patients (Cohort 3).

Amicus continues to assemble the highest quality data package and to scale up manufacturing to meet the needs of the Pompe community. The Company is in the midst of a series of collaborative discussions with U.S. and EU regulators regarding the best and fastest pathway forward for this novel treatment option, and continues to anticipate a Pompe regulatory pathway update in the first half of 2018.

Pompe Program Updates:

- Data collection underway in a retrospective natural history study (POM-002)
- Prospective observational study (POM-003) initiated
- GMP production of ATB200 commenced at the large commercial scale (1,000 Liters)
- FDA agreement reached on comparability between 250L scale and 1000L engineering batches
- FDA agreement reached on testing strategy for demonstrating comparability between 250L scale and 1000L GMP batches

Anticipated Upcoming Pompe Program Milestones:

- Expansion of ongoing ATB200-02 clinical study to include four to six additional ambulatory ERT-switch patients
- Additional data from ATB200-02 clinical study at 14th Annual WORLD*Symposium*™ (February 5-9, 2018)
- Final regulatory agreement on manufacturing comparability between 1,000L and 250L GMP scale
- Completion and release for clinic of 1,000L GMP commercial scale material
- Pompe regulatory pathway update (1H18)
- Initiation of larger registration-directed study

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-centric biotechnology company focused on discovering, developing and delivering novel high-quality medicines for people living with rare metabolic diseases. The cornerstone of the Amicus portfolio is migalastat, an oral precision medicine for people living with Fabry disease who have amenable genetic mutations. Migalastat is currently approved under the trade name Galafold™ in the European Union, with additional approvals granted and pending in several geographies. The future value driver of the Amicus pipeline is ATB200/AT2221, a novel, late-stage, potential best-in-class treatment paradigm for Pompe disease. The Company is committed to advancing and expanding a robust pipeline of cutting-edge, first- or best-in-class medicines for rare metabolic diseases.

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, 2016 and Quarterly Report on Form 10-Q for the quarter ended September 30, 2017. 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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