Amicus Therapeutics Announces Presentations and Posters at 16th Annual WORLDSymposium™ 2020

February 7, 2020

CRANBURY, N.J., Feb. 07, 2020 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD), a global, patient centric biotechnology company focused on discovering, developing and delivering novel medicines for rare diseases, today announced that one oral presentation and nine posters highlighting its development programs for Lysosomal Disorders will be included at the 16th Annual WORLD*Symposium* ™ 2020 to be held February 10-13, 2020 in Orlando, FL.

Oral Platform Presentations:
Batten Disease:

- *Single-dose AAV9-CLN6 gene transfer stabilizes motor and language function in CLN6-type Batten disease: interim results from the first clinical gene therapy trial* – Emily de los Reyes, MD, Pediatric Neurology, Nationwide Children's Hospital, Columbus, OH, USA (Thursday, February 13 at 8:30 a.m. EST)

Poster Session: Monday, February 10, 4:30-6:30 p.m. EST
Batten Disease:

- *Single-dose AAV9-CLN6 gene transfer stabilizes motor and language function in CLN6-type Batten disease: interim results from the first clinical gene therapy trial* – Emily de los Reyes, MD, Pediatric Neurology, Nationwide Children's Hospital, Columbus, OH, USA (Poster #92)

Poster Session: Tuesday, February 11, 4:30-6:30 p.m. EST
Fabry Disease:

- *Exposure-response of migalastat in support of extrapolation of efficacy from adults to children with Fabry disease* – Franklin Johnson, MS, Amicus Therapeutics, Inc. Cranbury, NJ, USA (Poster #190)

Poster Session: Wednesday, February 12, 4:30-6:30 p.m. EST
Fabry Disease:

- *Lyso-Gb3 is not a predictive biomarker of treatment response in migalastat-treated patients with migalastat-amenable variants* – Raphael Schiffmann, MD, Department of Neurology, Baylor Scott & White Research Institute, Dallas, TX, USA (Poster #372)
- *Migalastat has a low incidence rate of composite clinical outcomes at 4-year follow-up in patients with Fabry disease who previously received enzyme replacement therapy* – Daniel Bichet, MD, Department of Medicine, Université de Montréal, Montreal, Canada (Poster #LB-06)
- *Baseline patient characteristics of followME, a new, patient-centric, prospective, observational Fabry registry that evaluates migalastat, ERT, and a natural history cohort* – Gere Sunder-Plassmann, MD, Department of Medicine III, Division of Nephrology and Dialysis, Medical University of Vienna, Vienna, Austria (Poster #388)
- *The unmet need in Fabry disease: a retrospective analysis of healthcare claims in the United States reveals significant burden of illness in ERT-treated patients* – Eric Wallace, MD, Division of Nephrology, University of Alabama at Birmingham, Birmingham, AL, USA (Poster #409)
- *FABry disease Patient-Reported Outcome-Gastrointestinal (FABPRO-GI): a new Fabry disease-specific gastrointestinal outcomes instrument* – Juan Politei, Neurology Service, Dr Nestor Chamoles Laboratory of Neurochemistry, Buenos Aires, Argentina (Poster #331)
- *Extrapolation of migalastat tissue concentrations in mice as a predictor of human tissue concentrations* – Shirley Wu, Pharm.D. Pharmacokinetics & Pharmacodynamics, Nuventra Inc., Durham, NC, USA (Poster #427)

Pompe Disease:

- *Humoral immune responses to ATB200 in the first-in-human study of ATB200/AT2221 in patients with Pompe disease: preliminary results from the Phase 1/2 ATB200-02 trial* – Elfrida Benjamin, Ph.D., Amicus Therapeutics, Inc. Cranbury, NJ, USA (Poster #LB-04)

The goal of the WORLD*Symposium* is to provide an interdisciplinary forum to explore and discuss specific areas of interest, research, and clinical applicability related to lysosomal diseases. Each year, WORLD*Symposium* hosts a scientific meeting presenting the latest information from basic science, translational research, and clinical trials for lysosomal diseases. This symposium is designed to help researchers and clinicians to better manage and understand diagnostic options for patients with lysosomal diseases, identify areas requiring additional basic and clinical research, public policy and regulatory attention, and identify the latest findings in the natural history of lysosomal diseases. For more information please visit www.worldsymposia.org.

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 Statement

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 inclusion of forward-looking statements should not be regarded as a representation by us that any of our plans or projections 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 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 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 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. 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 press release to reflect events or circumstances after the date hereof.

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