Amicus Therapeutics Announces Presentations and Posters at the 17th Annual WORLDSymposium™ 2021

January 21, 2021

CRANBURY, N.J., Jan. 21, 2021 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD), a patient-dedicated global biotechnology company focused on discovering, developing and delivering novel medicines for rare diseases, today announced that three oral presentations and ten posters highlighting its development programs for Lysosomal Disorders will be included at the 17th Annual WORLDSymposium ™ 2021 to be held virtually February 8-12, 2021.

Oral Platform Presentations:

- *Direct intercellular cross-correction of α-galactosidase-A deficiency in Fabry disease podocytes through tunneling nanotubes in a mixed cell culture model* – Behzad Najafian, MD, Department of Pathology, University of Washington, Seattle, WA, USA (Monday, February 8 at 1:12 p.m. EST)

- *Single-dose AAV9-CLN6 gene transfer slows the decline in motor and language function in variant late infantile neuronal ceroid lipofuscinosis 6: Interim results from phase 1/2 trial* – Emily de los Reyes, MD, Pediatric Neurology, Nationwide Children's Hospital, Columbus, OH, USA (Wednesday, February 10 at 11:24 a.m. EST)

- *Utilization of artificial intelligence to identify undiagnosed Fabry disease patients: Development of a validated machine learning model* – John Jefferies, MD, MPH, Division of Cardiovascular Disease, University of Tennessee Health Science Center, Memphis, TN, USA (Thursday, February 11 at 1:00 p.m. EST)

ePoster Sessions:

Monday, February 8, 2:30-3:30 p.m. EST

- *Direct intercellular cross-correction of α-galactosidase-A deficiency in Fabry disease podocytes through tunneling nanotubes in a mixed cell culture model* – Behzad Najafian, MD, Department of Pathology, University of Washington, Seattle, WA, USA (Poster #169)

Tuesday, February 9, 2:30-3:30 p.m. EST

- *Functional analysis and clinical curation of human acid alpha glucosidase (GAA) variants of unknown significance (VUS) screened from infants diagnosed with Pompe disease via newborn screening (NBS)* – Shelly Goomber, PhD, Division of Medical Genetics, Duke University Medical Center, Durham, NC, USA (Poster #87)

- *Development of a Fabry disease screening tool for chronic pain patients - step 1: Categorization based on phenotypic risk profiles* – Michael A. Ueberall, MD, Pediatric Neurology, Institute of Neurological Sciences, Nurnberg, Germany (Poster #256)

Wednesday, February 10, 2:30-3:30 p.m. EST

- *Long-term treatment with migalastat 150 mg every other day is associated with sustained cardiac efficacy and is well tolerated* – Ulla Feldt-Rasmussen MD, DMSc, Department of Endocrinology, Rigshospitalet, Copenhagen University Hospital, Copenhagen, Denmark (Poster #70)

- *Migalastat 150 mg every other day achieves bioequivalent exposures in adolescent and adult patients with Fabry disease* – Uma Ramaswami, MD, Lysosomal Storage Disease Unit, Royal Free London NHS Foundation Trust, London, UK (Poster #210)

Thursday, February 11, 2:30-3:30 p.m. EST

- *Utilization of artificial intelligence to identify undiagnosed Fabry disease patients: Development of a validated machine learning model* – John Jefferies, MD, MPH, Division of Cardiovascular Disease, University of Tennessee Health Science Center, Memphis, TN, USA (Poster #107)

- *Migalastat clinical dose is highly extracted by hemodialysis and hemodiafiltration* – Franklin Johnson, MS, Clinical Pharmacology and Pharmacokinetics, Amicus Therapeutics, Inc., Cranbury, NJ, USA (Poster #108)

- *Development of a novel gene therapy for Fabry disease: Engineered alpha-galactosidase A transgene for improved stability* – Tobias Willer, Principal Investigator, Amicus Therapeutics, Inc., Philadelphia, PA, USA (Poster #272)

Friday, February 12, 2:30-3:30 p.m. EST

- *An open-label, phase 1/2a, AAV9-CLN3 gene transfer clinical trial for juvenile neuronal ceroid lipofuscinosis* – Emily de los Reyes, MD, Pediatric Neurology, Nationwide Children's Hospital, Columbus, OH, USA (Poster #LB-10)

- *Long-term renal efficacy and incidence of Fabry-associated clinical events in treatment-naive and enzyme replacement therapy-experienced female patients receiving migalastat for Fabry disease up to 8.5 years* – Heather A. Lau, MD, Division of Neurogenetics, New York University, NY, USA (Poster #LB-28)

The goal of 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 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 and the Quarterly Report filed on Form 10-Q for the quarter ended September 30, 2020. 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.

CONTACTS:

Investors:

Amicus Therapeutics
Andrew Faughnan
Director, Investor Relations
afaughnan@amicusrx.com
(609) 662-3809

Media:
Amicus Therapeutics
Diana Moore

Head of Global Corporate Communications
dmoore@amicusrx.com
(609) 662-5079

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