Amicus Therapeutics Announces Presentations and Posters at 15th Annual WORLDSymposium™ 2019

January 18, 2019

CRANBURY, N.J., Jan. 18, 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 that four oral presentations and eight posters highlighting its development programs for Lysosomal Storage Disorders will be included at the 15th Annual WORLD*Symposium*™ 2019, to be held February 4-8, 2019 in Orlando, FL.

Oral Platform Presentations:

Fabry Disease:

- *A novel method for quantification of globotriaocylceramide (GL-3) inclusions in affected podocytes in females with Fabry disease shows progressive accumulation of GL-3 in podocytes with age and no cross-correction between affected and non-affected podocytes* – Behzad Najafian, MD, Department of Pathology, University of Washington Seattle (Tuesday, February 5, at 10:15 a.m. EST)

- *Oral pharmacological chaperone migalastat compared with enzyme replacement therapy in Fabry disease: 30-month results from the randomized phase 3 ATTRACT study* – Ulla Feldt-Rasmussen, MD, DMSc, Chief Physician, Medical Endocrinology and Metabolism, Rigshospitalet, Copenhagen, Denmark (Thursday, February 7 at 8:45 a.m. EST)

Pompe Disease:

- *Safety and efficacy of acid α-glucosidase (AT-GAA) in ERT-switch nonambulatory patients with Pompe disease: preliminary results from the ATB200-02 trial* – Paula Clemens, MD, Chief, Neurology Service, Pittsburgh VA Healthcare System (February 7 at 9:15 a.m. EST)

- *Preliminary patient-reported outcomes and safety of acid α-glucosidase (AT-GAA) in patients with Pompe disease from the ATB200-02 trial* – Mark Roberts, M.D. - Dept. of Neurology, Salford Royal NHS Foundation Trust (February 7 at 2:00 p.m. EST)

Poster Session: Tuesday, February 5, 4:30-6:30pm EST

Fabry Disease:

- *Effect of long-term migalastat treatment on plasma globotriaosylsphingosine (lyso-Gb3) levels in patients with Fabry disease previously treated with enzyme replacement therapy: results from ATTRACT and open-label extension studies* – Daniel Bichet, Department of Medicine, Université de Montréal (Poster #42)

- *Oral pharmacological chaperone migalastat compared with enzyme replacement therapy in Fabry disease: 30-month results from the randomized phase 3 ATTRACT study* – Ulla Feldt-Rasmussen, MD, DMSc, Chief Physician, Medical Endocrinology and Metabolism, Rigshospitalet, Copenhagen, Denmark (Poster #104)

- *Migalastat reduces globotriaosylceramide (GL-3) inclusions in renal peritubular capillaries in patients with Fabry disease and migalastat-amenable mutations: post hoc analyses from FACETS* – Laura Barisoni, MD, Department of Pathology, Duke University School of Medicine (Poster #32)

- *Migalastat pharmacokinetic (PK) exposure comparisons between race/ethnic groups and between males and females are similar* – Franklin Johnson, MS, Amicus Therapeutics, Inc.Cranbury, USA. (Poster #181)

Pompe Disease:

- *First-in-human study of acid α-glucosidase (AT-GAA) in patients with Pompe disease: Preliminary functional assessment results from the ATB200-02 trial* – Priya Kishnani, MBBS, Department of Pediatrics, Duke University School of Medicine (Poster #196)

- *Safety and efficacy of acid α-glucosidase (AT-GAA) in ERT-switch nonambulatory patients with Pompe disease: preliminary results from the ATB200-02 trial* –Paula Clemens, MD, Chief, Neurology Service of Pittsburgh VA Healthcare System (Poster #68)

Poster Session: Wednesday. February 6, 4:30-6:30 EST

Fabry Disease:

- *Clinical outcomes after switching to migalastat from agalsidase alfa or agalsidase beta in patients with Fabry disease: Post hoc analysis from ATTRACT* – Gere Sunder-Plassmann, MD, Department of Medicine III, Division of Nephrology and Dialysis, Medical University of Vienna, Vienna, Austria (Poster #347)

- *The effects of long-term migalastat treatment in Fabry disease patients previously treated with enzyme replacement therapy who have migalastat-amenable variants with low α-galactosidase A response in the in vitro migalastat amenability assay* – Kathleen Nicholls, MD, Department of Nephrology, Royal Melbourne Hospital, Melbourne, Australia (Poster #256)

- *Design of a prospective, multicenter, multinational, observational safety and outcomes registry in Fabry disease patients treated with migalastat and untreated patients* – Gere Sunder-Plassmann, MD, Department of Medicine III, Division of Nephrology and Dialysis, Medical University of Vienna, Vienna, Austria (Poster #346)

- *Occurrence of cerebrovascular events during long-term treatment with migalastat in patients with Fabry disease* – Gere Sunder-Plassmann, MD, Department of Medicine III, Division of Nephrology and Dialysis, Medical University of Vienna, Vienna, Austria (Poster #LB-49)

- *Fabry patients' needs and expectations regarding their treatment in France: Development of a Patients' Need Questionnaire (PNQ Fabry)* – Esther Noël, CHRU of Strasbourg, Medical Specialties – Ophthalmology-Hygiene and COREVIH (Poster #262)

Pompe Disease:

- *Preliminary patient-reported outcomes and safety of acid α-glucosidase (AT-GAA) in patients with Pompe disease from the ATB200-02 trial* – Mark Roberts, M.D. - Dept. of Neurology, Salford Royal NHS Foundation Trust

Batten Disease:

- Jill M. Weimer, PhD, Senior Director, Therapeutic Development at Sanford Research, will present data from preclinical studies in Batten disease conducted at Sanford Research, which are related to the Amicus gene therapy portfolio licensed from Nationwide Children's Hospital (NCH) - *Promise of AAV9 gene therapy in the treatment of Batten disease: Systematic approach in therapy design reduces pathological and behavioral deficits and prolongs survival in mouse models of CLN3-, CLN6-, and CLN8-Batten disease* (Poster #374)

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 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, and the reporting of results from preclinical studies and clinical trials. 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 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; and the potential that we will need additional funding to complete all of our studies. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results for any of our product candidates. 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.

CONTACTS:

Investors/Media:
Amicus Therapeutics
Sara Pellegrino, IRC
Vice President, Investor Relations and Corporate Communications
spellegrino@amicusrx.com
(609) 662-5044

Media:
Pure Communications
Jennifer Paganelli
jpaganelli@purecommunications.com
(347) 658-8290

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