



Amicus Therapeutics Announces Presentations and Posters at the 18th Annual WORLDSymposium™ 2022

February 7, 2022

PHILADELPHIA, Feb. 07, 2022 (GLOBE NEWSWIRE) -- [Amicus Therapeutics](#) (Nasdaq: FOLD), a patient-dedicated global biotechnology company focused on developing and commercializing novel medicines for rare diseases, today announced that two oral presentations and 11 posters across its development programs will be included at the [18th Annual WORLDSymposium™ 2022](#) being held February 7-11, 2022 in San Diego, CA.

Oral Platform Presentation:

Pompe Disease:

Wednesday, February 9, 10:30 a.m. PST

- **Abstract Title:** *Immunogenicity of cipaglucosidase alfa/miglustat versus alglucosidase alfa/placebo in late-onset Pompe disease (LOPD): A phase III, randomized study (PROPEL)*
Presenter: Tahseen Mozaffar, MD, University of California, Irvine, CA, U.S.A.
Location: Exhibit Hall

Friday, February 11, 10:15 a.m. PST

- **Abstract Title:** *Liver-directed and systemic AAV gene transfer approaches for Pompe disease therapy*
Presenter: Naresh Kumar Meena, PhD, National Institutes of Health, Bethesda, MD, U.S.A.
Location: Exhibit Hall

Poster Sessions:

Fabry Disease:

Wednesday, February 9, 3:00-5:00 p.m. PST

- **Abstract Title:** *Baseline demographics and clinical characteristics of patients enrolled in the followME Fabry Pathfinders Registry (Poster #216)*
Presenter: Peter Nordbeck, MD, University Hospital Wuerzburg, Wuerzburg, Germany
Location: Exhibit Hall
- **Abstract Title:** *Long-term multisystemic efficacy with migalastat in ERT-naive and ERT-experienced patients with amenable GLA variants (Poster #123)*
Presenter: Robert Hopkin, MD, Cincinnati Children's Hospital Medical Center, Cincinnati, OH, U.S.A.
Location: Exhibit Hall
- **Abstract Title:** *Migalastat HCl 150 mg every other day is well-tolerated and efficacious in adolescent patients with Fabry disease (Poster #258)*
Presenter: Uma Ramaswami, MD, Royal Free London NHS Foundation Trust, London, U.K.
Location: Exhibit Hall
- **Abstract Title:** *Insights into patients' expectations and treatment preferences based on the patient needs questionnaire: Interim results from the SATIS-Fab study in Fabry disease (ePoster #174)*
Presenter: Olivier Lidove, MD, Deaconess Hospital Group Croix Saint-Simon, Paris, France
Location: Virtual Site

Thursday, February 10, 3:00-5:00 p.m. PST

- **Abstract Title:** *First results from The Spanish Fabry Women Study: A retrospective observational study describing the phenotype of female carrying genetic variants associated to Fabry disease (ePoster #269)*
Presenter: Rosario Sánchez, MD, General University Hospital of Alicante, Alicante, Spain
Location: Virtual Site
- **Abstract Title:** *Fabry disease, symptom burden, health-related quality of life burden and treatment satisfaction (ePoster #186)*

Presenter: Alasdair MacCulloch, PhD, Amicus Therapeutics UK LTD, Marlow, U.K.

Location: Virtual Site

- **Abstract Title:** *Treatment preferences in Fabry disease, a discrete choice experiment in the UK and Denmark* (ePoster #187)

Presenter: Alasdair MacCulloch, PhD, Amicus Therapeutics UK LTD, Marlow, U.K.

Location: Virtual Site

Pompe Disease:

Wednesday, February 9, 3:00-5:00 p.m. PST

- **Abstract Title:** *Immunogenicity of cipaglucosidase alfa/miglustat versus alglucosidase alfa/placebo in late-onset Pompe disease (LOPD): A phase III, randomized study (PROPEL)* (Poster #47)

Presenter: Tahseen Mozaffar, MD, University of California, Irvine, CA, USA

Location: Exhibit Hall

- **Abstract Title:** *Cipaglucosidase alfa/miglustat versus alglucosidase alfa/placebo in late-onset Pompe disease (LOPD): PROPEL study subgroup analyses* (Poster #39)

Presenter: Barry Byrne, MD, PhD, University of Florida Health, Gainesville, FL, U.S.A.

Location: Exhibit Hall

- **Abstract Title:** *Living with Pompe disease in the UK: Characterizing the patient journey and burden on physical, emotional and social quality of life* (ePoster #130)

Presenter: Derralynn Hughes, BMBCh, University College London, London, U.K.

Location: Virtual Site

Thursday, February 10, 3:00-5:00 p.m. PST

- **Abstract Title:** *Plasma total GAA protein PK profiles differ between cipaglucosidase alfa/miglustat and alglucosidase alfa* (ePoster #143)

Presenter: Franklin Johnson, MS, Amicus Therapeutics, Inc., Philadelphia, PA, U.S.A.

Location: Virtual Site

About WORLDSymposium

The goal of WORLDSymposium is to provide an interdisciplinary forum to explore and discuss specific areas of interest, research and clinical applicability related to lysosomal diseases. Each year, WORLDSymposium 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](#).

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