



## Amicus Therapeutics Announces Presentations and Posters at the 20th Annual WORLDSymposium™ 2024

February 1, 2024 at 7:00 AM EST

PRINCETON, N.J., Feb. 01, 2024 (GLOBE NEWSWIRE) -- [Amicus Therapeutics](#) (Nasdaq: FOLD), today announced that one oral presentation and 11 posters across its development programs will be included at the [20th Annual WORLDSymposium™ 2024](#) being held February 4-9, 2024 in San Diego, CA.

### Oral Platform Presentation:

#### Fabry Disease:

**Abstract Title:** *Trial in progress: An open-label study (AT1001-025) to evaluate the safety and pharmacokinetics of migalastat in patients with Fabry disease and amenable GLA variants and severe renal impairment or end-stage renal disease treated with hemodialysis*

- **Presenter:** Franklin Johnson, MS, Amicus Therapeutics, Inc., Princeton, NJ, U.S.A.
- **Date and time:** Thursday, February 8, 9:12 – 9:24 a.m. PT

### Poster Sessions:

#### Fabry Disease:

**Abstract Title:** *Multiorgan involvement in females with Fabry disease: Results from two Phase III trials and the followME registry (Poster #246)*

- **Presenter:** Peter Nordbeck, MD, University Hospital Würzburg, Würzburg, Germany
- **Date and time:** Wednesday, February 7, 3:00 – 5:00 p.m. PT
- **Location:** Exhibit Hall, Kiosk 41-F

**Abstract Title:** *Prevalence of migalastat-amenable mutations in patients with Fabry disease from Brazil, Argentina and Colombia (Poster #262)*

- **Presenter:** Juan Politei, MD, Foundation for the Study of Neurometabolic Diseases, Buenos Aires, Argentina
- **Date and time:** Wednesday, February 7, 3:00 – 5:00 p.m. PT
- **Location:** Exhibit Hall, Kiosk 44-F

**Abstract Title:** *Treatment-related benefit and satisfaction in patients with Fabry disease: Insight into patients' expectations and preferences from the SATIS-Fab study (Poster #189)*

- **Presenter:** Didier Lacombe, MD, Bordeaux Hospital University Center, Bordeaux, France
- **Date and time:** Wednesday, February 7, 3:00 – 5:00 p.m. PT
- **Location:** Exhibit Hall, Kiosk 34-B

**Abstract Title:** *FollowME Fabry Pathfinders registry: Renal effectiveness in a cohort of patients on migalastat treatment for at least three years (Poster #339)*

- **Presenter:** Michael West, MD, Dalhousie University, Halifax, Canada
- **Date and time:** Wednesday, February 7, 3:00 – 5:00 p.m. PT
- **Location:** Exhibit Hall, Kiosk 49-D

**Abstract Title:** *Exploring the experience of females living with Fabry disease in North America (Poster #8)*

- **Presenter:** Julia Alton, Executive Director, Canadian Fabry Association, Ontario, Canada
- **Date and time:** Wednesday, February 7, 3:00 – 5:00 p.m. PT
- **Location:** Exhibit Hall, Kiosk 2-F

**Abstract Title:** *Trial in progress: An open-label study (AT1001-025) to evaluate the safety and pharmacokinetics of migalastat in patients with Fabry disease and amenable GLA variants and severe renal impairment or end-stage renal disease treated with hemodialysis (Poster #165)*

- **Presenter:** Franklin Johnson, MS, Amicus Therapeutics, Inc., Princeton, NJ, U.S.A.
- **Date and time:** Thursday, February 8, 3:00 – 5:00 p.m. PT
- **Location:** Exhibit Hall, Kiosk 18-D

**Abstract Title:** *Cardiac biomarkers in Fabry disease* (Poster #241)

- **Presenter:** Pooja Nandi, Scientific Product Manager, Koneksa Health, New York, NY, U.S.A.
- **Date and time:** Thursday, February 8, 3:00 – 5:00 p.m. PT
- **Location:** Exhibit Hall, Kiosk 25-D

**Abstract Title:** *Exploring the journey of patients with Fabry disease in Brazil* (Poster #346)

- **Presenter:** Christopher Wingrove, Amicus Therapeutics UK LTD, Marlow, U.K.
- **Date and time:** Thursday, February 8, 3:00 – 5:00 p.m. PT
- **Location:** Exhibit Hall, Kiosk 31-H

#### **Pompe Disease:**

**Abstract Title:** *Effect size analysis of cipaglucosidase alfa plus miglustat versus alglucosidase alfa in ERT-experienced adults with late-onset Pompe disease in PROPEL* (Poster #232)

- **Presenter:** Tahseen Mozaffar, MD, University of California, Irvine, CA, U.S.A.
- **Date and time:** Wednesday, February 7, 3:00 – 5:00 p.m. PT
- **Location:** Exhibit Hall, Kiosk 39-B

**Abstract Title:** *Switching treatment to cipaglucosidase alfa plus miglustat positively affects motor function and quality of life in patients with late-onset Pompe disease* (Poster #320)

- **Presenter:** Antonio Toscano, MD, University of Messina, Messina, Italy
- **Date and time:** Wednesday, February 7, 3:00 – 5:00 p.m. PT
- **Location:** Exhibit Hall, Kiosk 45-D

**Abstract Title:** *Baseline demographics of the UK Early Access to Medicines Scheme registry for cipaglucosidase alfa plus miglustat in enzyme replacement therapy-experienced adults with late-onset Pompe disease* (Poster #279)

- **Presenter:** Mark Roberts, MD, Salford Royal NHS Foundation Trust, Greater Manchester Neurosciences Centre, Manchester, U.K.
- **Date and time:** Wednesday, February 7, 3:00 – 5:00 p.m. PT
- **Location:** Exhibit Hall, Kiosk 46-F

**Abstract Title:** *Minimal clinically important differences in six-minute walking distance in late-onset Pompe disease* (Poster #293)

- **Presenter:** Benedikt Schoser, MD, Ludwig Maximilian University of Munich, Munich, Germany
- **Date and time:** Wednesday, February 7, 3:00 – 5:00 p.m. PT
- **Location:** Exhibit Hall, Kiosk 50-F

#### **About WORLDSymposium**

WORLDSymposium™ is an annual research conference dedicated to lysosomal diseases. WORLD is an acronym that stands for We're Organizing Research on Lysosomal Diseases. 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](http://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 diseases. With extraordinary patient focus, Amicus Therapeutics is committed to advancing and expanding a pipeline of cutting-edge, first- or best-in-class medicines for rare diseases. For more information please visit the company's website at [www.amicusrx.com](http://www.amicusrx.com), and follow on [Twitter](https://twitter.com/amicusrx) and [LinkedIn](https://www.linkedin.com/company/amicusrx).

CONTACTS:

#### **Investors:**

Amicus Therapeutics  
Andrew Faughnan

Vice President, Investor Relations  
[afaughnan@amicusrx.com](mailto:afaughnan@amicusrx.com)  
(609) 662-3809

#### **Media:**

Amicus Therapeutics  
Diana Moore

Vice President, Corporate Communications

[dmoore@amicusrx.com](mailto:dmoore@amicusrx.com)

(609) 662-5079

FOLD-G