



Amicus Therapeutics Announces Presentations and Posters at the 19th Annual WORLDSymposium™ 2023

February 13, 2023

PHILADELPHIA, Feb. 13, 2023 (GLOBE NEWSWIRE) -- [Amicus Therapeutics](#) (Nasdaq: FOLD), today announced that two oral presentations and 11 posters across its development programs will be included at the [19th Annual WORLDSymposium™ 2023](#) being held February 22-26, 2023 in Orlando, FL.

Oral Platform Presentation:

Pompe Disease:

Abstract Title: *Long-term follow-up of cipaglucosidase alfa/miglustat in ambulatory patients with Pompe disease: An open-label phase I/II study (ATB200-02)*

- **Presenter:** Barry Byrne, MD, PhD, University of Florida Health, Gainesville, FL, U.S.A.
- **Date and time:** Friday, February 24, 10:30-11:30 a.m. ET
- **Location:** General Session Room

Abstract Title: *Long-term efficacy and safety of cipaglucosidase alfa/miglustat in ambulatory patients with Pompe disease: A phase III open-label extension study (ATB200-07)*

- **Presenter:** Benedikt Schoser, MD, Department of Neurology, Ludwig-Maximilians-University of Munich, Munich, Germany
- **Date and time:** Sunday, February 26, 9:00-10:00 a.m. ET
- **Location:** General Session Room

Poster Sessions:

Fabry Disease:

Abstract Title: *Estimation of arrhythmia risk in patients with Fabry disease using a machine learning model (Poster #9)*

- **Presenter:** Patricio Aguiar, MD, PhD, North Lisbon Hospital Center, Lisbon, Portugal
- **Date and time:** Thursday, February 23, 3:00-4:00 p.m. ET
- **Location:** Exhibit Hall, Kiosk 2-A

Abstract Title: *Estimation of stroke risk in patients with Fabry disease using a machine learning model (Poster #192)*

- **Presenter:** Staci Kallish, DO, Hospital of the University of Pennsylvania, Philadelphia, PA, U.S.A.
- **Date and time:** Thursday, February 23, 3:00-4:00 p.m. ET
- **Location:** Exhibit Hall, Kiosk 18-B

Abstract Title: *FollowME Fabry Pathfinders registry: Renal effectiveness in a multi-national, multi-center cohort of patients on migalastat treatment for at least three years (Poster #176)*

- **Presenter:** Derralynn Hughes, BMBCh, University College London, London, U.K.
- **Date and time:** Friday, February 24, 3:00-4:00 p.m. ET
- **Location:** Exhibit Hall, Kiosk 22-A

Abstract Title: *Clinical characteristics of female patients enrolled in the FollowME Fabry Pathfinders registry (Poster #190)*

- **Presenter:** Biliana Veleva-Rotse, PhD, Amicus Therapeutics, Philadelphia, PA, U.S.A.
- **Date and time:** Friday, February 24, 3:00-4:00 p.m. ET
- **Location:** Exhibit Hall, Kiosk 23-B

Abstract Title: *Gender heterogeneity in the diagnosis and treatment journey for patients with Fabry disease: A Japanese database analysis (Poster #81)*

- **Presenter:** Mio Tsuchiya, PhD, Amicus Therapeutics, Tokyo, Japan
- **Date and time:** Saturday, February 25, 3:00-4:00 p.m. ET
- **Location:** Exhibit Hall, Kiosk 10-B

Abstract Title: *High prevalence of cardiac findings in individuals with Fabry disease identified via no-charge sponsored testing programs* (Poster #363)

- **Presenter:** Biliana Veleva-Rotse, PhD, Amicus Therapeutics, Philadelphia, PA, U.S.A.
- **Date and time:** Saturday, February 25, 4:00-5:00 p.m. ET
- **Location:** Exhibit Hall, Kiosk 20-A

Pompe Disease:

Abstract Title: *Long-term follow-up of cipaglucosidase alfa/miglustat in ambulatory patients with Pompe disease: An open-label phase I/II study (ATB200-02)* (Poster #59)

- **Presenter:** Barry Byrne, MD, PhD, University of Florida Health, Gainesville, FL, U.S.A.
- **Date and time:** Friday, February 24, 3:00-4:00 p.m. ET
- **Location:** Exhibit Hall, Kiosk 3-B

Abstract Title: *Long-term efficacy and safety of cipaglucosidase alfa/miglustat in ambulatory patients with Pompe disease: A phase III open-label extension study (ATB200-07)*: (Poster #LB-59)

- **Presenter:** Benedikt Schoser, MD, Department of Neurology, Ludwig-Maximilians-University of Munich, Munich, Germany
- **Date and time:** Friday, February 24, 4:00-5:00 p.m. ET
- **Location:** Exhibit Hall, Kiosk 24-B

Abstract Title: *Disease burden, treatment patterns and healthcare resource utilization associated with Pompe disease in Sweden: A real-world evidence study* (Poster #257)

- **Presenter:** Alasdair MacCulloch, PhD, Amicus Therapeutics, Marlow, U.K.
- **Date and time:** Friday, February 24, 4:00-5:00 p.m. ET
- **Location:** Exhibit Hall, Kiosk 23-A

Abstract Title: *Indirect treatment comparison of three enzyme replacement treatments for late-onset Pompe disease: A network meta-analysis with patient-level and aggregate data* (Poster #63)

- **Presenter:** Jeff Castelli, PhD, Amicus Therapeutics, Philadelphia, PA, U.S.A.
- **Date and time:** Saturday, February 25, 3:00-4:00 p.m. ET
- **Location:** Exhibit Hall, Kiosk 10-A

Abstract Title: *Quality of life with late-onset Pompe disease: Qualitative interviews and general public utility estimation* (Poster #236)

- **Presenter:** Alasdair MacCulloch, PhD, Amicus Therapeutics, Marlow, U.K.
- **Date and time:** Saturday, February 25, 3:00-4:00 p.m. ET
- **Location:** Exhibit Hall, Kiosk 30-A

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.

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, and follow on [Twitter](https://twitter.com/AmicusRx) and [LinkedIn](https://www.linkedin.com/company/amicusrx).

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