# Amicus Therapeutics Presents New Long-term Data for both Galafold® (migalastat) and POMBILITI® (cipaglucosidase alfa-atga) + OPFOLDA® (miglustat) at the 22nd Annual WORLDSymposium™ 2026

February 3, 2026 at 8:00 AM EST

PRINCETON, N.J., Feb. 03, 2026 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD), today announced the presentation of new data from clinical and real-world studies of Galafold® (migalastat) in Fabry disease and POMBILITI® + OPFOLDA® (cipaglucosidase alfa plus miglustat) in late-onset Pompe disease. Data are being presented at the 22nd Annual WORLD*Symposium* ™ 2026

"Amicus continues to advance the science and understanding of both Fabry and Pompe diseases, and we are proud to showcase new data for our medicines at this year's WORLD *Symposium*. These new data add to the growing body of evidence supporting the important role of Galafold and Pombiliti + Opfolda," said Jeff Castelli, PhD, Chief Development Officer, Amicus Therapeutics, Inc. "We are immensely grateful to the patients, their families, and investigators whose partnership makes scientific research and advances possible."

**Oral Presentations:**

| Abstract Title | Disease | Presenter | Date and Time |
|---|---|---|---|
| *Real-world effectiveness of migalastat versus enzyme replacement therapy in previously treatment-naïve patients with Fabry disease: analyses of matched populations from the global followME Pathfinders registry* | Fabry | Peter Nordbeck, University of Wuerzburg, Wuerzburg, Germany | Thursday, February 5, 8:30 a.m. PST |
| *208-week outcomes of cipaglucosidase alfa plus miglustat in patients with late-onset Pompe disease treated from PROPEL baseline: muscle function and biomarkers* | Pompe | Tahseen Mozaffar, University of California, Irvine, Irvine, CA, USA | Friday, February 6, 1:20 p.m. PST |

**Poster Sessions**

**Fabry Disease:**

| Abstract Title | Presenter | Date and Time |
|---|---|---|
| *Exploring the lived experience of the Fabry community in Czechia* (Poster #378) | Christopher Wingrove, Amicus Therapeutics, Inc., Marlow, UK | Tuesday, February 3, 3:30 – 5:30 p.m. PST |
| *followME Fabry Pathfinders registry: cardiac and renal effectiveness in a multi-national, multi-center cohort of patients on migalastat treatment for 5 years* (Poster #266) | Peter Nordbeck, University of Wuerzburg, Wuerzburg, Germany | Thursday, February 5, 3:30 – 5:30 p.m. PST |
| *Real-world effectiveness of migalastat versus enzyme replacement therapy in previously treatment-naïve patients with Fabry disease: analyses of matched populations from the global followME Pathfinders registry* (Poster #267) | Peter Nordbeck, University of Wuerzburg, Wuerzburg, Germany | Thursday, February 5, 3:30 – 5:30 p.m. PST |
| *Adjusted migalastat dose regimens in patients with Fabry disease and amenable GLA variants with severe renal impairment, or with end-stage renal disease and receiving hemodialysis/hemodiafiltration (HD/HDF): pharmacokinetic (PK) and safety results from a protocol-specified interim analysis of the RENEW study* (Poster #187) | Franklin K. Johnson, Amicus Therapeutics, Inc., Princeton, NJ, USA | Thursday, February 5, 3:30 – 5:30 p.m. PST |
| *Trial in progress: an open-label study (AT1001-033; ASPIRE II) to evaluate the safety, pharmacokinetics, pharmacodynamics and efficacy of migalastat in pediatric patients with Fabry disease* (Poster #384) | Haichen Yang, Amicus Therapeutics, Inc., Philadelphia, PA, USA | Thursday, February 5, 3:30 – 5:30 p.m. PST |
| *A structured methodology for evaluating patient-reported outcomes across a treatment program: a comparative application in migalastat* (Poster #127) | Vera Gielen, Amicus Therapeutics, Inc., Marlow, UK | Thursday, February 5, 3:30 – 5:30 p.m. PST |
| *Clinical characterization and healthcare usage in Fabry disease using Swedish national registers* (Poster #118) | Emma Flordal Thelander, Amicus Therapeutics, Inc., Stockholm, Sweden | Thursday, February 5, 3:30 – 5:30 p.m. PST |
| *followME Fabry Pathfinders real-world registry in Spain and Portugal: cardiac and renal outcomes with migalastat in patients with Fabry disease* (Poster #22) | Olga Azevedo, Hospital Senhora da Oliveira, Guimarães, Portugal | Thursday, February 5, 3:30 – 5:30 p.m. PST |
| *Matching-adjusted indirect comparisons (MAICs) and network meta-analyses (NMAs) of the oral small-molecule chaperone migalastat versus intravenous enzyme replacement therapies (ERTs) for clinical measures in Fabry disease* (Poster #128) | Vera Gielen, Amicus Therapeutics, Inc., Marlow, UK | Thursday, February 5, 3:30 – 5:30 p.m. PST |

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| *Long-term safety and effectiveness of migalastat in patients with Fabry disease: results from the Korean post-marketing surveillance* (Poster #168) | Geu-Ru Hong, Severance Cardiovascular Hospital, Seoul, Korea | Thursday, February 5, 3:30 – 5:30 p.m. PST |

**Pompe Disease:**

| **Abstract Title** | **Presenter** | **Date and Time** |
|---|---|---|
| *208-week outcomes of cipaglucosidase alfa plus miglustat in patients with late-onset Pompe disease treated from PROPEL baseline: pulmonary function* (Poster #205) | Priya S. Kishnani, Duke University, Durham, NC, USA | Thursday, February 5, 3:30 – 5:30 p.m. PST |
| *208-week outcomes of cipaglucosidase alfa plus miglustat in patients with late-onset Pompe disease treated from PROPEL baseline: Muscle function and biomarkers* (Poster #257) | Tahseen Mozaffar, University of California, Irvine, Irvine, CA, USA | Thursday, February 5, 3:30 – 5:30 p.m. PST |
| *90-month muscle function and biomarker outcomes with cipaglucosidase alfa plus miglustat (cipa+mig) in adults with Pompe disease in ATB200-02, an open-label phase I/II study* (Poster #313) | Benedikt Schoser, Friedrich-Baur-Institute, Ludwig-Maximilians-University, Munich, Germany | Thursday, February 5, 3:30 – 5:30 p.m. PST |
| *90-month pulmonary function outcomes with cipaglucosidase alfa plus miglustat (cipa+mig) in adults with Pompe disease in ATB200-02, an open-label phase I/II study* (Poster #206) | Priya S. Kishnani, Duke University, Durham, NC, USA | Thursday, February 5, 3:30 – 5:30 p.m. PST |
| *90-month physician and patient-reported outcomes with cipaglucosidase alfa plus miglustat (cipa+mig) in adults with Pompe disease in ATB200-02, an open-label phase I/II study* (Poster #299) | Mark Roberts, Salford Royal NHS Foundation Trust, Salford, UK | Thursday, February 5, 3:30 – 5:30 p.m. PST |
| *From frustration to function: reimagining registry value with individual monitoring dashboards to visualize disease progression in Pompe disease* (Poster #247) | Paul McIntosh, University of Pennsylvania, Philadelphia, PA, USA | Thursday, February 5, 3:30 – 5:30 p.m. PST |
| *ROSSELLA: an ongoing open-label, multicenter, global trial to study next-generation treatment of infantile-onset Pompe disease combining enzyme replacement with a stabilizing iminosugar* (Poster #244) | Thorsten Marquardt, University of Münster, Münster, Germany | Thursday, February 5, 3:30 – 5:30 p.m. PST |
| *Impact of mobility-aid use on late-onset Pompe disease (LOPD) patient experience: insights from patient interviews* (Poster #178) | Derralynn Hughes, Royal Free London NHS Foundation Trust, University College London, London, UK | Thursday, February 5, 3:30 – 5:30 p.m. PST |
| *Delayed diagnosis and missed opportunities: results from an analysis of the diagnostic journey for LOPD in the UK* (Poster #82) | Patrick Deegan, Cambridge University Hospitals NHS Foundation Trust (CUH), Cambridge, UK | Thursday, February 5, 3:30 – 5:30 p.m. PST |

**About Galafold**

Galafold® (migalastat) 123 mg capsules is an oral pharmacological chaperone of alpha-Galactosidase A (alpha-Gal A) for the treatment of Fabry disease in adults who have amenable galactosidase alpha gene *(GLA)* variants. In these patients, Galafold works by stabilizing the body's own dysfunctional enzyme so that it can clear the accumulation of disease substrate. Globally, Amicus Therapeutics estimates that approximately 35 to 50 percent of people living with Fabry disease may have amenable *GLA* variants, though amenability rates within this range vary by geography. Galafold is approved in more than 40 countries around the world, including the U.S., EU, U.K., and Japan.

**U.S. INDICATIONS AND USAGE**

Galafold is indicated for the treatment of adults with a confirmed diagnosis of Fabry disease and an amenable galactosidase alpha gene (*GLA*) variant based on *in vitro* assay data.

This indication is approved under accelerated approval based on reduction in kidney interstitial capillary cell globotriaosylceramide (KIC GL-3) substrate. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials.

**U.S. IMPORTANT SAFETY INFORMATION**

**ADVERSE REACTIONS:** The most common adverse reactions reported with Galafold (≥10%) were headache, nasopharyngitis, urinary tract infection, nausea and pyrexia.

**USE IN SPECIFIC POPULATIONS:** There is insufficient clinical data on Galafold use in pregnant women to inform a drug-associated risk for major birth defects and miscarriage. Advise women of the potential risk to a fetus. It is not known if Galafold is present in human milk. Therefore, the developmental and health benefits of breastfeeding should be considered along with the mother's clinical need for Galafold and any potential adverse effects on the breastfed child from Galafold or from the underlying maternal condition. Galafold is not recommended for use in patients with severe renal impairment or end-stage renal disease requiring dialysis. The safety and effectiveness of Galafold have not been established in pediatric patients. To report Suspected Adverse Reactions, contact Amicus Therapeutics at 1-877-4AMICUS or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch. **For additional information about Galafold, please see the full U.S. Prescribing Information**.

**About Pombiliti + Opfolda**

Pombiliti + Opfolda, is a two-component therapy that consists of cipaglucosidase alfa-atga, a bis-M6P-enriched rhGAA that facilitates high-affinity uptake through the M6P receptor while retaining its capacity for processing into the most active form of the enzyme, and the oral enzyme stabilizer, miglustat, that's designed to reduce loss of enzyme activity in the blood.

**U.S. INDICATIONS AND USAGE**
POMBILITI in combination with OPFOLDA is indicated for the treatment of adult patients with late-onset Pompe disease (lysosomal acid alpha-glucosidase [GAA] deficiency) weighing ≥40 kg and who are not improving on their current enzyme replacement therapy (ERT).

**SAFETY INFORMATION**

**HYPERSENSITIVITY REACTIONS INCLUDING ANAPHYLAXIS: Appropriate medical support measures, including cardiopulmonary resuscitation equipment, should be readily available. If a severe hypersensitivity reaction occurs, POMBILITI should be discontinued immediately and appropriate medical treatment should be initiated. INFUSION-ASSOCIATED REACTIONS (IARs): If severe IARs occur, immediately discontinue POMBILITI and initiate appropriate medical treatment. RISK OF ACUTE CARDIORESPIRATORY FAILURE IN SUSCEPTIBLE PATIENTS: Patients susceptible to fluid volume overload, or those with acute underlying respiratory illness or compromised cardiac or respiratory function, may be at risk of serious exacerbation of their cardiac or respiratory status during POMBILITI infusion. See PI for complete Boxed Warning. CONTRAINDICATION:** POMBILITI in combination with Opfolda is contraindicated in pregnancy. **EMBRYO-FETAL TOXICITY:** May cause embryo-fetal harm. Advise females of reproductive potential of the potential risk to a fetus and to use effective contraception during treatment and for at least 60 days after the last dose. **Adverse Reactions:** Most common adverse reactions ≥ 5% are headache, diarrhea, fatigue, nausea, abdominal pain, and pyrexia. **Please see full PRESCRIBING INFORMATION, including BOXED WARNING, for POMBILITI (cipaglucosidase alfa-atga) and full PRESCRIBING INFORMATION for OPFOLDA (miglustat).**

**About WORLD*Symposium***
WORLD*Symposium* is designed for basic, translational and clinical researchers, patient advocacy groups, clinicians, and all others who are interested in learning more about the latest discoveries related to lysosomal diseases and the clinical investigation of these advances. Each year, WORLD*Symposium* presents the latest information from basic science, translational research, and clinical trials for lysosomal diseases. For more information, please visit www.worldsymposia.org.

**About Amicus Therapeutics**
Amicus Therapeutics (Nasdaq: FOLD) is a leading, global biotechnology company with a clear and compelling mission: to develop and deliver transformative medicines for people living with rare diseases. With extraordinary patient focus, Amicus strives to redefine expectations in rare disease. For more information please visit the company's website at www.amicusrx.com, and follow on LinkedIn.

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