



Amicus Therapeutics Announces FDA Approval and Launch of New Treatment for Pompe Disease

September 28, 2023 at 10:41 AM EDT

**Pombiliti™ (cipaglucosidase alfa-atga) + Opfolda™ (miglustat) Approved in
ERT-Experienced Adults**

Pombiliti + Opfolda Logo

**First and Only Two-Component Therapy for Eligible Adults Living with Late-onset Pompe
Disease**



Amicus Therapeutics to Host Conference Call Today at 12:00 p.m. ET

PHILADELPHIA, Sept. 28, 2023 (GLOBE NEWSWIRE) -- [Amicus Therapeutics](#) (Nasdaq: FOLD) today announced that the U.S. Food and Drug Administration (FDA) has approved Pombiliti™ (cipaglucosidase alfa-atga) + Opfolda™ (miglustat) 65mg capsules. This two-component therapy is indicated for adults living with late-onset Pompe disease (LOPD) weighing ≥ 40 kg and who are not improving on their current enzyme replacement therapy (ERT).

Late-onset Pompe disease is a rare, debilitating, and life-threatening lysosomal disorder caused by a deficiency of the enzyme acid alpha-glucosidase (GAA). Reduced levels of GAA lead to the accumulation of the substrate glycogen in the lysosomes of muscle cells and glycogen buildup causes muscle damage. Disease severity ranges across a spectrum, with predominant manifestations such as skeletal muscle weakness and progressive respiratory involvement.¹

Pombiliti + Opfolda is a unique two-component therapy. Pombiliti is a recombinant human GAA enzyme (rhGAA) naturally expressed with high levels of bis-M6P (Mannose 6-Phosphate), designed for increased uptake into muscle cells. Once in the cell, Pombiliti can be properly processed into its most active and mature form to break down glycogen. Opfolda is an enzyme stabilizer designed to stabilize the enzyme in the blood.

"Today's FDA approval of Pombiliti and Opfolda is a testament to the power of science, medicine, and our passionate determination to improve the lives of people living with Pompe disease. This approval embodies our Amicus spirit, passion, and resilience and is a very meaningful step for the Pompe community. I am just so immensely proud of our team, and so very grateful to everyone who has worked to bring this medicine to this approval. Most especially to all of the people living with Pompe around the world," said John F. Crowley, Executive Chairman of Amicus Therapeutics, Inc.

Bradley Campbell, President and Chief Executive Officer of Amicus Therapeutics, Inc., stated, "The FDA approval of Pombiliti and Opfolda is a major milestone for Amicus. We are grateful to the Pompe community, particularly the patients, caregivers, families, researchers, and physicians who have contributed to the development process through their commitment to our clinical studies. Today's approval is also a testament to Team Amicus' extraordinary dedication to patients and our ability to execute on our vision to bring new therapies to the rare disease community. Our highly experienced team is ready to launch this medicine in the U.S., and we look forward to rapidly bringing this new treatment regimen to all eligible adults living with late-onset Pompe disease who are not improving on their current ERT."

The FDA approval was based on clinical data observed from the Phase 3 pivotal study (PROPEL), the only trial in LOPD to study ERT-experienced participants in a controlled setting.

"The Pompe community continues to face unmet need and limited treatment options. This two-component therapy is an important new treatment for those adults living with late-onset Pompe disease and not improving on current therapies. I am encouraged by the evidence generated over many years of clinical research studying this therapy for ERT-experienced patients living with late-onset Pompe disease," said Tahseen Mozaffar, MD, Director of the Division of Neuromuscular Diseases in the Department of Neurology at the School of Medicine at UC Irvine and Director of the UC Irvine ALS and Neuromuscular Center, as well as an investigator for the PROPEL study.

"Today's FDA approval is an extremely important step and acknowledges the potential of Pombiliti and Opfolda," said Priya Kishnani, MD, Professor of Pediatrics and Chief of Medical Genetics at Duke University School of Medicine and an investigator for the PROPEL study. "I am grateful that eligible patients with late-onset Pompe disease in the U.S. will now have access to additional treatment options."

"The FDA approval of Pombiliti and Opfolda represents a long-awaited day for people living with late-onset Pompe disease and advocating for additional therapeutic options," said Tiffany House, President, Acid Maltase Deficiency Association. "Amicus' long-standing commitment to the Pompe community and rare disease research has led to the development of an important therapy for the Pompe community because patients will now have options."

Amicus Therapeutics will launch Pombiliti + Opfolda immediately in the U.S. The FDA previously granted Breakthrough Therapy designation for Pombiliti + Opfolda. Pombiliti + Opfolda has also been approved for the treatment of adults with LOPD in the European Union and the United Kingdom.

Amicus Assist® provides support to patients and caregivers in the U.S. and can help patients access their medication and identify possible sources of financial assistance. For more information on Amicus Assist, visit the Amicus Assist website at amicusassist.com, or please call +1-833-AMICUS-A (+1-833-264-2872).

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. **CONTRADICTION:** 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 $\geq 5\%$ are headache, diarrhea, fatigue, nausea, abdominal pain, and pyrexia. **Please see full PRESCRIBING INFORMATION, including BOXED WARNING, for POMBILITI (cipaglucosidase alfa-atga) [LINK](#) and full Prescribing Information for OPFOLDA (miglustat) [LINK](#).**

Conference Call and Webcast

Amicus Therapeutics will host a conference call and audio webcast today, September 28, 2023, at 12:00 p.m. ET to discuss the FDA approval. Participants and investors interested in accessing the call by phone will need to register using the [online registration form](#). After registering, all phone participants will receive a dial-in number along with a personal PIN to access the event.

A live audio webcast and related presentation materials can also be accessed via the Investors section of the Amicus Therapeutics corporate website at [ir.amicusrx.com](#). Web participants are encouraged to register on the website 15 minutes prior to the start of the call. An archived webcast and accompanying slides will be available on the Company's website shortly after the conclusion of the live event.

About Pompe Disease

[Pompe disease](#) is an inherited lysosomal disorder caused by deficiency of the enzyme acid alpha-glucosidase (GAA). Reduced or absent levels of GAA lead to accumulation of glycogen in cells, which is believed to result in the clinical manifestations of Pompe disease. Pompe disease ranges from a rapidly deteriorating infantile form with significant impact to heart function, to a more slowly progressive, late-onset form primarily affecting skeletal muscle and progressive respiratory involvement. Late-onset Pompe disease can be severe and debilitating with progressive muscle weakness throughout the body that worsens over time, particularly skeletal muscles and muscles that control breathing.¹

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.

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).

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 metabolic diseases. Further information about the Company can be found at: [www.amicusrx.com](#), and can be followed 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 approval and commercialization plans for Pombiliti + Opfolda in the United States. 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, 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 we may not be successful in commercializing Pombiliti + Opfolda in the United States, the potential that public and commercial payors will not reimburse Pombiliti + Opfolda, the potential that we may not be able to manufacture or supply sufficient commercial products; and the potential that we will need additional funding to complete all of our commercialization and manufacturing activities. 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, 2022, as well as our Quarterly Report on Form 10-Q for the quarter ended June 30, 2023, filed with the Securities and Exchange Commission. 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.

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1. Kishnani PS, Steiner RD, Bali D, et al. Pompe disease diagnosis and management guideline. Genet Med 2006; 8: 267–88.

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A photo accompanying this announcement is available at <https://www.globenewswire.com/NewsRoom/AttachmentNg/a70f1858-5aa5-45ec-8e4b-f7092cff817a>