UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 8-K

CURRENT REPORT PURSUANT TO
SECTION 13 OR 15(d) OF THE
SECURITIES EXCHANGE ACT OF 1934

Date of Report (Date of earliest event reported): **April 26, 2023**

AMICUS THERAPEUTICS, INC.
(Exact Name of Registrant as Specified in Its Charter)

Delaware	001-33497	71-0869350
(State or Other Jurisdiction of Incorporation)	**(Commission File Number)**	**(I.R.S. Employer Identification No.)**

3675 Market Street, Philadelphia, PA 19104
(Address of Principal Executive Offices, and Zip Code)

215-921-7600
Registrant's Telephone Number, Including Area Code

(Former Name or Former Address, if Changed Since Last Report.)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock Par Value $0.01	FOLD	NASDAQ

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (17 CFR §230.405) or Rule 12b-2 of the Securities Exchange Act of 1934 (17 CFR §240.12b-2). Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Item 7.01 – Regulation FD Disclosure.

On April 26, 2023, Amicus Therapeutics, Inc. (the "Company") issued a press release announcing that the Company has received a positive CHMP opinion for Opfolda® (miglustat) for late-onset Pompe disease. A copy of this press release is attached hereto as Exhibit 99.1 and incorporated herein by reference.

The information in this Item 7.01, including Exhibit 99.1, is being furnished and shall not be deemed "filed" for purposes of Section 18 of the Act, or otherwise subject to the liabilities of that Section. The information in this Item 7.01, including Exhibit 99.1, shall not be incorporated by reference into any registration statement or other document pursuant to the Act.

Item 9.01 Financial Statements and Exhibits

(d) Exhibits:

Exhibit No.	Description
99.1	April 26, 2023 Press Release
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

Signature Page

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

AMICUS THERAPEUTICS, INC.

Date: April 26, 2023

By: /s/ Ellen S. Rosenberg
Name: Ellen S. Rosenberg
Title: Chief Legal Officer and Corporate Secretary

Amicus Therapeutics Receives Positive CHMP Opinion for Opfolda® (miglustat) for Late-Onset Pompe Disease

European Commission Approval of Opfolda and Commercial Launch of Pombiliti® + Opfolda® Anticipated in 3Q 2023

Upon Approval, Pombiliti + Opfolda will be the First Two-Component Therapy in the European Union for the Treatment of Adults Living with Late-Onset Pompe Disease

PHILADELPHIA, PA, April 26, 2022 – Amicus Therapeutics (Nasdaq: FOLD) today announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) adopted a positive opinion recommending marketing authorization of miglustat, the enzyme stabilizer component of AT-GAA. A decision for miglustat from the European Commission is expected in the third quarter of 2023, after which the two-component therapy will be fully approved, and the Company will begin the country-by-country reimbursement and launch process. Miglustat will be commercialized under the brand name Opfolda. The biologic component of the two-component therapy, Pombiliti (cipaglucosidase alfa), was approved by the European Commission (EC) in March 2023.

Upon approval, Pombiliti + Opfolda will be the first two-component therapy available in the European Union (EU) for the treatment of adults living with late-onset Pompe disease (LOPD). The indication for Opfolda is an enzyme stabilizer of cipaglucosidase alfa long-term enzyme replacement therapy in adults with late-onset Pompe disease (acid α glucosidase [GAA] deficiency).

“With today’s positive CHMP opinion of Opfolda, we are now one step away from bringing this much-needed new treatment to adults living in Europe with late-onset Pompe disease. This was the realization of the hard work and efforts of so many individuals dedicated to the mission of improving the lives of people living with Pompe disease,” said John F. Crowley, Executive Chairman of Amicus Therapeutics, Inc.

"I am immensely proud of our team that has worked tirelessly over the past decade to develop this innovative therapy. We are grateful for the commitment and support from the Pompe community who have helped advance this therapy, especially the patients, families, and physicians who participated in our clinical studies. Based on the strength of the label and our launch readiness, once fully approved we believe there is significant opportunity to bring Pombiliti and Opfolda as the first two-component therapy for adult LOPD patients in Europe, and to establish this novel treatment combination as a potential new standard of care in Pompe disease,” said Bradley Campbell, President and Chief Executive Officer of Amicus Therapeutics, Inc.

“This significant milestone moves Pombiliti and Opfolda closer to the LOPD community, where there is a high medical need for novel treatment options,” said Prof. Benedikt Schoser, Professor of Neurology at Ludwig-Maximillians-University of Munich LMU Department of Neurology. “In clinical studies, Pombiliti and Opfolda has exhibited clinically meaningful and positive changes in the key manifestations of this challenging disease. The CHMP positive opinion and recommended indication reflect this robust body of evidence and gives me further hope for the potential of this innovative therapy for people living with LOPD.”

The CHMP based its positive opinion on clinical data from the Phase 3 PROPEL pivotal study, the only randomized, controlled trial in LOPD to include patients in the high unmet need ERT-experienced population, in addition to ERT-naïve patients.

About Pombiliti® + Opfolda®

Pombiliti + Opfolda, also known as AT-GAA, is an investigational two-component therapy that consists of cipaglucosidase alfa, 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 minimize loss of enzyme activity in the blood. In clinical studies, AT-GAA was associated with demonstrated improvements in both musculoskeletal and respiratory measures.

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 fatal infantile form with significant impacts 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, including progressive muscle weakness throughout the body, particularly the skeletal muscles and muscles controlling breathing, that worsens over time.

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 and LinkedIn.

Important Safety Information

Pombiliti (cipaglucosidase alfa) Important Safety Information

Posology and Method of Administration: Pombiliti must be used in combination with miglustat 65 mg hard capsules. The recommended dose of Pombiliti is 20 mg/kg of body weight every other week. The Pombiliti infusion should start 1 hour after taking miglustat capsules. ***Paediatric population:*** The safety and efficacy of Pombiliti in combination with miglustat therapy in paediatric patients less than 18 years old have not yet been established. No data are available. ***Contraindications*****:** Life-threatening hypersensitivity to the active substance, or to any of the excipients. Contraindication to miglustat. ***Anaphylaxis and infusion-associated reactions (IARs):*** Serious anaphylaxis and IARs have occurred in some patients during infusion and following infusion with Pombiliti. Premedication with oral antihistamine, antipyretics, and/or corticosteroids may be administered to assist with signs and symptoms related to IARs experienced with prior enzyme replacement therapy (ERT) treatment. Reduction of the infusion rate, temporary interruption of the infusion, symptomatic treatment with oral antihistamine, or antipyretics, and appropriate resuscitation measures should be considered to manage serious IARs. If anaphylaxis or severe allergic reactions occur, infusion should be immediately paused, and appropriate medical treatment should be initiated. The current medical standards for emergency treatment of anaphylactic reactions are to be observed and cardiopulmonary resuscitation equipment should be readily available. The risks and benefits of re-administering Pombiliti following anaphylaxis or severe allergic reaction should be carefully considered, and appropriate resuscitation measures made available. ***Risk of acute cardiorespiratory failure in susceptible patients:*** Patients with acute underlying respiratory illness or compromised cardiac and/or respiratory function may be at risk of serious exacerbation of their cardiac or respiratory compromise during infusions. Appropriate medical support and monitoring measures should be readily available during Pombiliti infusion. ***Immune complex-related reactions:*** Immune complex-related reactions have been reported with other ERTs in patients who had high IgG antibody titres, including severe cutaneous reactions and nephrotic syndrome. If immune complex-related reactions occur, discontinuation of the administration of Pombiliti should be considered and appropriate medical treatment should be initiated. The risks and benefits of re-administering Pombiliti following an immune complex-related reaction should be reconsidered for each individual patient. ***Contraception in females:*** Reliable contraceptive measures must be used by women of childbearing potential during treatment with Pombiliti in combination with miglustat, and for 4 weeks after discontinuing treatment. ***Pregnancy:*** Pombiliti in combination with miglustat therapy is not recommended during pregnancy. ***Breast feeding:*** It is not known if Pombiliti and miglustat are secreted in human breast milk. A decision must be made whether to discontinue breast-feeding or to discontinue/abstain from Pombiliti in combination with miglustat therapy taking into account the benefit of breast-feeding for the child and the benefit of therapy for the woman. ***Summary of the safety profile:*** The most commonly reported adverse reactions only attributable to Pombiliti were chills (4.0%), dizziness (2.6%), flushing (2.0%), somnolence (2.0%), chest discomfort (1.3%), cough, (1.3%), infusion site swelling (1.3%), and pain (1.3%). Reported serious adverse reactions only attributable to Pombiliti were urticaria (2.0%), anaphylaxis (1.3%), pyrexia (0.7%), presyncope (0.7%), dyspnoea (0.7%), pharyngeal oedema (0.7%), wheezing (0.7%), and hypotension (0.7%). Refer to SmPC for full list.

Forward-Looking Statements

This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including statements relating to data from a global Phase 3 study to investigate AT-GAA for the treatment of late-onset Pompe disease, the potential implications on these data for the future advancement and development of AT-GAA, expectations regarding the regulatory process in the US and Europe, and the outcome of those regulatory reviews. There can be no assurance that the EMA will grant full approval for Opfolda or when any such approvals may occur. Words such as, but not limited to, "look forward to," "believe," "expect," "anticipate," "estimate," "intend," "confidence," "encouraged," "potential," "plan," "targets," "likely," "may," "will," "would," "should" and "could," and similar expressions or words identify forward-looking statements. The forward-looking statements included in this press release are based on management's current expectations and belief's which are subject to a number of risks, uncertainties and factors, including that the Company will not be able to successfully complete the development of, obtain full regulatory approval for, or successfully manufacture and commercialize AT-GAA once fully approved. In addition, all forward looking statements are subject to the other risks and uncertainties detailed in our Annual Report on Form 10-K for the year ended December 31, 2022. As a consequence, actual results may differ materially from those set forth in this press release. You are cautioned not to place undue reliance on these forward-looking statements, which speak only of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, and we undertake no obligation to revise this press release to reflect events or circumstances after the date hereof.

CONTACTS:

Investors:
Amicus Therapeutics
Andrew Faughnan
Vice President, Investor Relations
afaughnan@amicusrx.com
(609) 662-3809

Media:
Amicus Therapeutics
Diana Moore
Head of Global Corporate Communications
dmoore@amicusrx.com
(609) 662-5079

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