

AMICUS THERAPEUTICS, INC.

FORM 8-K (Current report filing)

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**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): **December 14 , 2017**

AMICUS THERAPEUTICS, INC.

(Exact Name of Registrant as Specified in Its Charter)

Delaware

(State or Other Jurisdiction of
Incorporation)

001-33497

(Commission File Number)

71-0869350

(IRS Employer Identification No.)

1 Cedar Brook Drive, Cranbury, NJ

(Address of Principal Executive Offices)

08512

(Zip Code)

Registrant's telephone number, including area code: **(609) 662-2000**

(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))

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

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 8.01. Other Events.

On December 14, 2017, Amicus Therapeutics, Inc. (the “Company”) issued a press release announcing that the Company submitted a new drug application to the United States Food and Drug Administration to request approval of the oral precision medicine migalastat HCl (“migalastat”) for the treatment of patients 16 years and older with Fabry disease who have amenable mutations. A copy of this press release is attached hereto as Exhibit 99.1.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits: The Exhibit Index annexed hereto is incorporated herein by reference.

Exhibit No.	Description
99.1	Press Release dated December 14, 2017

EXHIBIT INDEX

Exhibit No.	Description
99.1	Press Release dated December 14, 2017

SIGNATURES

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: December 14, 2017

By: /s/ ELLEN S. ROSENBERG
Ellen S. Rosenberg
General Counsel and Corporate Secretary



Amicus Therapeutics Submits New Drug Application to U.S. FDA for Migalastat for Treatment of Fabry Disease

CRANBURY, NJ, December 14, 2017 — Amicus Therapeutics (Nasdaq: FOLD) submitted a new drug application (NDA) to the U.S. Food and Drug Administration (FDA) to request approval of the oral precision medicine migalastat HCl (“migalastat”) for the treatment of patients 16 years and older with Fabry disease who have amenable mutations. The NDA submission is based on existing clinical data, including reduction in disease-causing substrate (GL-3), as well as the totality of data from two Phase 3 pivotal studies in treatment-naïve (Study 011, or FACETS) and enzyme replacement therapy (ERT) switch patients (Study 012, or ATTRACT), as well as other completed clinical studies.

Migalastat previously received both Orphan Drug Designation and Fast Track designation from the U.S. FDA. The FDA has a 60-day filing review period to determine whether the NDA is complete and acceptable for filing, after which the Agency will notify the Company. Amicus plans to communicate following written receipt of the Agency’s decision.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc., stated, “Today marks the very first Amicus submission of a new drug application to the U.S. FDA. This important milestone is the culmination of a strong collaboration and commitment among the patients, physicians and Amicus employees who have spent more than a decade to advance migalastat. On the heels of our initial launch success for migalastat in Europe, our goal is to further expand access for more Fabry patients with amenable mutations in the U.S., Japan and other global geographies. We look forward to working with the FDA during the review process and to a potential U.S. approval of migalastat in 2018.”

An estimated 3,000 people in the U.S. are currently diagnosed with Fabry disease, more than any other country. Fabry disease is a progressive, inherited lysosomal storage disorder caused by deficiency of an enzyme called alpha-galactosidase A (alpha-Gal A), which is the result of mutations in the *GLA* gene. The disease causes accumulation of specific lipids, primarily GL-3, in tissues including the heart, kidneys, central nervous system, and skin. This abnormal accumulation can lead to debilitating consequences including pain, kidney failure, heart disease, and stroke.

Migalastat works by stabilizing the body’s own dysfunctional alpha-Gal A enzyme, so it can clear the accumulated disease substrate in patients who have amenable mutations (an estimated 35% to 50% of Fabry patients globally). A proprietary *in vitro* assay (Galafold Amenability Assay) has been used to classify more than 1,000 known *GLA* mutations as “amenable” or “not amenable” to treatment with Galafold. The European Commission (EC) granted full approval for migalastat, under the trade name Galafold™, as a first line therapy for long-term treatment of adults and adolescents aged 16 years and older with a confirmed diagnosis of Fabry disease (alpha-galactosidase A deficiency) and who have an amenable mutation. The EC approval was based on clinical data from two Phase 3 pivotal studies in both treatment-naïve (Study 011, or FACETS) and enzyme replacement therapy (ERT) switch patients (Study 012, or ATTRACT), as well as ongoing long-term extension studies.

Outside the EU, migalastat is approved in Switzerland, Israel, Australia and Canada, with regulatory submissions under review in Japan and additional geographies.

About Galafold™ and Amenable Mutations

Galafold™ (migalastat) is a first-in-class chaperone therapy approved in the European Union as a monotherapy for Fabry disease in patients with amenable mutations. Galafold works by stabilizing the body’s own dysfunctional enzyme, so it can clear the accumulation of disease substrate in patients who have amenable mutations. A proprietary *in vitro* assay (Galafold Amenability Assay) has been used to classify more than 1,000 known *GLA* mutations as “amenable” or “not amenable” to treatment with Galafold. The EU label includes 331 *GLA* mutations that have been identified and determined to be amenable based on the Galafold Amenability Assay, which represent between 35% and 50% of the currently diagnosed Fabry population.

Healthcare providers in the EU may access the website www.Galafoldamenabilitytable.com to quickly and accurately identify which mutations are categorized as “amenable” or “not amenable” to Galafold. Amicus expects to submit additional updates to the EU label as additional *GLA* mutations are identified and tested in the Galafold Amenability Assay.

EU Important Safety Information

Treatment with Galafold should be initiated and supervised by specialists experienced in the diagnosis and treatment of Fabry disease. Galafold is not recommended for use in patients with a non-amenable mutation.

- Galafold is not intended for concomitant use with enzyme replacement therapy.
- Galafold is not recommended for use in patients with Fabry disease who have severe renal impairment ($<30 \text{ mL/min/1.73 m}^2$). The safety and efficacy of Galafold in children 0—15 years of age have not yet been established.
- No dosage adjustments are required in patients with hepatic impairment or in the elderly population.
- There is very limited experience with the use of this medicine in pregnant women. If you are pregnant, think you may be pregnant, or are planning to have a baby, do not take this medicine until you have checked with your doctor, pharmacist, or nurse.
- While taking Galafold, effective birth control should be used. It is not known whether Galafold is excreted in human milk.
- Contraindications to Galafold include hypersensitivity to the active substance or to any of the excipients listed in the PRESCRIBING INFORMATION.
- It is advised to periodically monitor renal function, echocardiographic parameters and biochemical markers (every 6 months) in patients initiated on Galafold or switched to Galafold.
- OVERDOSE: General medical care is recommended in the case of Galafold overdose.
- The most common adverse reaction reported was headache, which was experienced by approximately 10% of patients who received Galafold. For a complete list of adverse reactions, please review the SUMMARY OF PRODUCT CHARACTERISTICS.
- Call your doctor for medical advice about side effects.

For further important safety information for Galafold, including posology and method of administration, special warnings, drug interactions and adverse drug reactions, please see the European SmPC for Galafold available from the EMA website at www.ema.europa.eu.

About Fabry Disease

Fabry disease is an inherited lysosomal storage disorder caused by deficiency of an enzyme called alpha-galactosidase A (alpha-Gal A), which is the result of mutations in the GLA gene. The primary biological function of alpha-Gal A is to degrade specific lipids in lysosomes, including globotriaosylceramide (referred to here as GL-3 and also known as Gb₃). Lipids that can be degraded by the action of alpha-Gal A are called “substrates” of the enzyme. Reduced or absent levels of alpha-Gal A activity lead to the accumulation of GL-3 in the affected tissues, including the central nervous system, heart, kidneys, and skin. Progressive accumulation of GL-3 is believed to lead to the morbidity and mortality of Fabry disease, including pain, kidney failure, heart disease, and stroke. The symptoms can be severe, differ from patient to patient, and begin at an early age. All Fabry disease is progressive and may lead to organ damage regardless of the time of symptom onset.

About Amicus Therapeutics

Amicus Therapeutics (Nasdaq: FOLD) is a global biotechnology company at the forefront of therapies for rare and orphan diseases. The Company has a robust pipeline of advanced therapies for a broad range of human genetic diseases. Amicus’ lead programs in development include the small molecule pharmacological chaperone migalastat as a monotherapy for Fabry disease, as well as novel enzyme replacement therapy (ERT) and biologic products for Fabry disease, Pompe disease, and other rare and devastating diseases.

Forward-Looking Statements

This press release contains “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995 relating to the clinical development, regulatory approval pathway, and prospects and timing of regulatory submission and approval of our product candidates for the treatment of Fabry disease. Any express or implied statements contained in this press release that are not statements of historical fact, including interpretation of guidance given by the U.S. FDA may be deemed forward-looking statements. The inclusion of forward-looking statements should not be regarded as a representation by us that any of our plans will be achieved in a timely manner or at all. 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, with respect to statements regarding the goals, progress, timing, and outcomes of discussions with regulatory authorities, 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, changes in FDA guidance for regulatory approval, risks regarding the FDA’s interpretation of our clinical trial results, including the risk that results from completed clinical trials that supported approval by regulators in other jurisdictions will not be sufficient for U.S. FDA purposes, the risk that the FDA will require additional studies or data, the risk that the timing of an NDA will be delayed or not be accepted by the FDA, the potential that regulatory authorities, including the FDA, EMA, and PMDA, may not grant or may delay approval for our product candidate and the potential that we may not be successful in commercializing our product candidates for Fabry disease in Europe or any other country in which approval

is ultimately obtained, if any. 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, 2016 and the Quarterly Report for the quarter ended September 30, 2017. The FDA guidance described in this release was given as of a specific date and the FDA could change its position on the clinical end points or other standards for review and/or approval. 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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