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): **February 8, 2021**

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

Delaware
(State or Other Jurisdiction of Incorporation)

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 8.01 – Other Events

On February 8, 2021, Amicus Therapeutics, Inc. (the "Company") issued press releases announcing positive initial clinical data for its CLN3 Batten disease gene therapy and positive preclinical data for its Fabry disease gene therapy. The results of each are featured in virtual poster presentations at the 17th Annual WORLDSymposium™ 2021, being held February 8-12, 2021. A copy of each press release is attached hereto as Exhibit 99.1 and Exhibit 99.2, respectively, and are incorporated herein by reference.

Item 9.01 Financial Statements and Exhibits

(d) Exhibits:

Exhibit No.	Description
99.1	CLN3 Press Release dated February 8, 2021
99.2	Fabry Gene Therapy Press Release dated February 8, 2021
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.

Date: February 8, 2021

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

Exhibit 99.1

Amicus Therapeutics Announces Positive Initial Clinical Data for CLN3 Batten Disease Gene Therapy at the 17th Annual WORLD*Symposium*™ 2021

Initial Data Suggest Early Signs of Disease Stabilization in Children with Fatal Neurologic Disease

Plan to Submit IND for Next Clinical Study in 2H2021

PHILADELPHIA, PA, February 8, 2021 – Amicus Therapeutics (Nasdaq: FOLD) today announced positive initial results from its first in-human study of its CLN3 Batten disease gene therapy program, AT-GTX-502. The results are featured in a virtual poster presentation at the 17th Annual WORLD*Symposium*™ 2021, being held February 8-12, 2021. The poster is also available in the Events and Presentations section of the Amicus Therapeutics corporate website at http://ir.amicusrx.com/events-and-presentations.

The Abigail Wexner Research Institute (AWRI) at Nationwide Children's Hospital is conducting the ongoing Phase 1/2 clinical study of a single one-time administration of AT-GTX-502 gene therapy for classic juvenile neuronal ceroid lipofuscinosis (JNCL), also known as CLN3 Batten disease. With no approved treatments, CLN3 Batten disease is a fatal neurologic disease that leads to blindness, motor impairment, learning difficulties, epilepsy and, ultimately, premature death.

Primary outcome measures are determined using the physical impairment subscale of the Unified Batten Disease Rating Scale (UBDRS), a clinical rating instrument developed specifically to assess disease progression in children with verified JNCL and includes evaluations of motor, behavioral, seizure and functional capabilities. UBDRS separately scores measures of vision, motor, speech, tone and abnormal movement over time. Higher scores indicate greater physical impairment.

Clinical Data Highlights:

Initial safety data are available for the first four children up to 15 months post-administration of the AAV-CLN3 gene therapy. Preliminary efficacy data are available for the first three children with CLN3 Batten disease in the low-dose cohort for up 15 months post-administration of the AAV-CLN3 gene therapy, as well as one participant with CLN3 Batten disease in the high-dose cohort for up to 3 months post-administration of the AAV-CLN3 gene therapy. Initial results of the study suggest that AT-GTX-502 was well tolerated and demonstrated potential early signs of disease stabilization compared to a natural history dataset.

- **Safety (n=4)**: Treatment with AT-GTX-502 was generally well tolerated. The majority of adverse events (AEs) were mild or moderate and unrelated to treatment. No pattern of AEs related to AAV or CLN3 immunogenicity were observed. Additional details are provided in the presentation.

- **Unified Batten Disease Rating Scale**: For the three subjects treated in the low-dose cohort (n=3), the mean yearly rate of change in UBDRS Physical Impairment scores was +0.07 over 12 months vs +2.86 in untreated subjects from published natural history (n=82).

Jeff Castelli, Ph.D., Chief Development Officer of Amicus Therapeutics, stated, "We are pleased to share this first set of clinical data for our intrathecal AAV gene therapy for CLN3 Batten disease and the second clinical program in our Batten portfolio. Preliminary results from this analysis suggest early signs of disease stabilization and has the potential to slow the neurological disease progression in children with CLN3 Batten disease. We are encouraged by the data and hope to make a meaningful impact for individuals living with CLN3 Batten disease, an ultra-rare, devastating neurodegenerative disease with no approved treatments."

Emily de los Reyes, M.D., Ph.D., Principal Investigator at Nationwide Children's and Professor of Clinical Pediatrics and Neurology at The Ohio State University College of Medicine is leading the CLN3 clinical trial at AWRI.

Regulatory interactions for AT-GTX-502 are ongoing and the Company expects to provide feedback on the clinical path forward later this year.

Amicus has exclusive rights under a license to the CLN3 gene therapy program developed at the Abigail Wexner Research Institute at Nationwide Children's Hospital.

About AT-GTX-502

AT-GTX-502 is a novel gene therapy in Phase 1/2 development for CLN3 Batten disease, a rare, fatal, inherited lysosomal disorder with no approved treatment that primarily affects the nervous system. AT-GTX-502 is dosed in a one-time infusion to deliver a functional copy of the CLN3 gene to cells of the central nervous system. The therapy is designed to address the underlying enzyme deficiency that results in progressive cell damage and neurodevelopmental and physical decline. In the U.S., AT-GTX-502 was granted Fast Track Designation and Rare Pediatric Designation by the United States Food and Drug Administration. AT-GTX-502 also holds Orphan Drug designations in both the U.S. and in the EU.

About Batten Disease

Batten disease is the common name for a broad class of rare, fatal, inherited disorders of the nervous system also known as neuronal ceroid lipofuscinoses, or NCLs. In these disorders, a defect in a specific gene triggers a cascade of problems that interferes with a cell's ability to recycle certain molecules. Each gene is called CLN (ceroid lipofuscinosis, neuronal) and given a different number designation as its subtype. There are 13 known forms of Batten disease often referred to as CLN1-8; 10-14. The various types of Batten disease have similar features and symptoms but vary in severity and age of onset.

Most forms of Batten disease/NCLs usually begin during childhood. The clinical course often involves progressive loss of independent adaptive skills such as mobility, feeding and communication. Affected children may also experience vision loss, personality changes, behavioral problems, learning impairment and seizures. Children living with Batten disease typically experience progressive loss of motor function and eventually become wheelchair-bound, are then bedridden and die prematurely.

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 metabolic diseases. With extraordinary patient focus, Amicus Therapeutics is committed to advancing and expanding a robust pipeline of cutting-edge, first- or best-in-class medicines for rare metabolic diseases. For more information please visit the company's website at www.amicusrx.com, and follow 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 preclinical and clinical development of our product candidates, the timing and reporting of results from preclinical studies and clinical trials and the prospects and timing of the potential regulatory approval of our product candidates. In particular, this press release relates to interim data from an ongoing Phase 1/2 study to investigate intrathecal administration of AAV-CLN3 gene therapy. The inclusion of forward-looking statements arising from this interim data, ongoing study and natural history preliminary data 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, with respect to statements regarding the goals, progress, timing, and outcomes of discussions with regulatory authorities, and in particular the potential goals, progress, timing, and results of preclinical studies and clinical trials, 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 results of clinical or preclinical studies indicate that the product candidates are unsafe or ineffective; the potential that it may be difficult to enroll patients in our clinical trials; the potential that regulatory authorities, including the FDA, EMA, and PMDA, may not grant or may delay approval for our product candidates; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; and the potential that we will need additional funding to complete all of our studies. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. The interim data and Phase 1/2 study discussed herein is inherently preliminary and early in the study, derived from a limited patient set, and later trial results with this patient set or others may not be consistent with these preliminary results. 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, 2019 and Quarterly Report on Form 10-Q for the quarter ended September 30, 2020. 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.

CONTACTS:

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

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

FOLD–G

Amicus Therapeutics Presents Positive Preclinical Fabry Disease Gene Therapy Data at the 17th Annual WORLD*Symposium*™ 2021

Amicus Optimized Transgene Show Greater Substrate Reduction than Wild Type Construct Across All Tissues and Doses

Further Validates Combining Amicus-Engineered Transgenes with Penn's AAV Gene Therapy Technologies to Develop Next Generation Gene Therapies

PHILADELPHIA, PA, February 8, 2021 – Amicus Therapeutics (Nasdaq: FOLD) today announced initial preclinical data from its investigational adeno-associated viral (AAV) gene therapy program for Fabry disease in mice. The results are featured in a virtual poster presentation at the 17th Annual WORLD*Symposium*™ 2021, being held February 8-12, 2021. The poster is also available in the Events and Presentations section of the Amicus Therapeutics corporate website.

Fabry disease is an inherited lysosomal disorder caused by deficiency of the enzyme alpha-galactosidase A (GLA). Reduced or absent levels of GLA lead to accumulation of disease substrate leading to cellular disfunction and organ damage, which results in the clinical manifestations of Fabry disease. Amicus, in collaboration with the Gene Therapy Program of the Perelman School of Medicine at the University of Pennsylvania (Penn), is developing a novel gene therapy for Fabry disease that combines the Amicus protein-engineering expertise and deep knowledge and experience in Fabry disease with Penn's adeno associated virus (AAV) gene transfer technologies.

This initial preclinical study assessed a range of single doses of AAV in *Gla* knockout (KO) mice with either natural unmodified hGLA ("wildtype hGLA") or Amicus/Penn engineered hGLA transgenes ("engineered hGLA"). The Amicus/Penn engineered hGLAs are designed for improved stability which is believed to provide a larger window for the enzyme to stay active while in circulation prior to being taken up into the target tissues and for additional stabilization after cell uptake. The lead Amicus/Penn engineered hGLA declared as an IND candidate is designated as AT-GTX-701.

Preclinical Poster Highlights for Amicus/Penn AAV Gene Therapy for Fabry Disease:

- **Improved for stability:** In vitro characterization of two stabilized alpha-Gal A constructs with engineered disulfide bonds demonstrated stable homodimer formation, enhanced temperature, plasma, and neutral pH stability compared to wildtype.
- **Dose dependent response**: The lowest tested dose of AT-GTX-701 in *Gla* KO mice showed partial substrate reduction, while highest tested dose resulted in near complete substrate reduction.
- **Significantly greater substrate reduction vs. wildtype transgene**: AT-GTX-701 demonstrated significantly greater lyso-Gb3/GL-3 substrate reduction across all Fabry disease relevant tissues including the dorsal root ganglia (DRG), kidney, and heart, with reductions at low dose being equal to or greater than the reductions observed at higher doses with wildtype transgene.
- **First evidence of dorsal route ganglia storage reduction**: DRG are affected in Fabry disease and associated with neuropathic pain. AT-GTX-701's stabilized transgene provided the first evidence for DRG storage reduction in a Fabry mouse model treated with an AAV gene therapy.
- **Amicus/Penn Gene Therapy Platform:** Further validates the potential of this platform to design constructs that enhance proteins across multiple lysosomal disorders.
- Additional preclinical studies, IND enabling studies, and GMP manufacturing process development are underway.

Hung Do, PhD, Chief Science Officer of Amicus Therapeutics, stated, "These very important preclinical results validate our capabilities to develop engineered proteins via a gene therapy that can result in superior substrate reduction compared with a wildtype transgene. This is the second program in our collaboration with Penn that has demonstrated the potential advantages of optimizing the target protein in these disorders, and may be applicable to other lysosomal disorders as we continue to combine our understanding of the molecular basis of these diseases and expertise in protein engineering, together with Penn's vector engineering expertise, to develop novel gene therapies."

Amicus is currently developing AAV gene therapies in collaboration with Penn for Pompe disease, Fabry disease, CDD, CLN1, MPS IIIB, a next generation program in MPS IIIA, as well as Angelman Syndrome. The agreement between Amicus and Penn is a Research, Collaboration and License Agreement, providing funding to Penn to advance the preclinical research programs in the Wilson Lab and to license certain technologies invented under the funded Research Collaboration.

About Fabry Disease

Fabry disease is an inherited lysosomal 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 irreversible organ damage regardless of the time of symptom onset.

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 metabolic diseases. With extraordinary patient focus, Amicus Therapeutics is committed to advancing and expanding a robust pipeline of cutting-edge, first- or best-in-class medicines for rare metabolic diseases. For more information please visit the company’s website at www.amicusrx.com, and follow us 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, including statements relating to initial preclinical data from its investigational adeno-associated viral (AAV) gene therapy program for Fabry disease in mice and the potential implications of these data for the future advancement and development of a gene therapy for Fabry disease and other lysosomal disorders and development of potential platform technologies. 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 preliminary data reported before completion of the study will not be predictive of future results, that results of additional preliminary data or data from the completed study or any future study will not yield results that are consistent with the preliminary data presented, that later study results will not support further development, or even if such later results are favorable, that the Company will not be able to successfully complete the development of, obtain regulatory approval for, or successfully commercialize. 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, 2019 and the Quarterly Report filed on Form 10-Q for the quarter ended September 30, 2020. 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:
Andrew Faughnan
Sr. Director, Investor Relations
afaughnan@amicusrx.com
(609) 662-3809

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

FOLD–G