UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, DC 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 12, 2021**

ABEONA THERAPEUTICS INC.
(Exact name of registrant as specified in its charter)

Delaware	**001-15771**	**83-0221517**
(State or other jurisdiction of incorporation)	(Commission File Number)	(I.R.S. Employer Identification No.)

1330 Avenue of the Americas, 33rd Floor,
New York, NY 10019
(Address of principal executive offices) (Zip Code)

(646) 813-4712
(Registrant's telephone number, including area code)

N/A
(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 (see General Instruction A.2. below):

☐ 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	Name of each exchange on which registered
Common Stock, $0.01 par value	**ABEO**	**Nasdaq Capital Markets**

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 7.01. Regulation FD.

On February 12, 2021, Abeona Therapeutics Inc. issued a press release entitled "New Positive Phase 1/2 Interim Data Presented at WORLDSymposium™ Shows Neurocognitive Development of Young MPS IIIA Patients Preserved up to Three Years Following Treatment with Abeona's ABO-102 Gene Therapy." The full text of the press release is included as Exhibit 99.1 to this Current Report on Form 8-K and is incorporated herein by reference.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits.

Exhibit No.	**Description**
99.1	Press release dated February 12, 2021
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

SIGNATURE

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.

Abeona Therapeutics Inc.
(Registrant)

By: */s/ Brendan M. O'Malley*
Name: Brendan M. O'Malley
Title: Corporate Secretary

Date: February 12, 2021

Exhibit 99.1

New Positive Phase 1/2 Interim Data Presented at WORLD*Symposium*™ Shows Neurocognitive Development of Young MPS IIIA Patients Preserved up to Three Years Following Treatment with Abeona's ABO-102 Gene Therapy

In addition to preservation of neurocognitive development with ABO-102 in MPS IIIA, new clinical results of ABO-102 in MPS IIIA and ABO-101 in MPS IIIB continue to show dose-dependent and sustained reductions in disease-specific biomarkers, denoting clear biologic effects

In addition, ABO-102 and ABO-101 continue to show favorable safety profile in ongoing studies

Abeona to host investor webinar on Tuesday, February 16, 2021 at 1:00 p.m. EST

NEW YORK and CLEVELAND, Feb. 12, 2021 – Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced new positive data from two ongoing Phase 1/2 clinical trials of the company's investigational AAV-based gene therapies ABO-102 and ABO-101 in MPS IIIA and MPS IIIB, respectively. The interim data was presented in late-breaking platform oral presentations at the 17th Annual WORLD*Symposium*™. The presentation slides are available on the company's website at www.abeonatherapeutics.com.

Michael Amoroso, Principal Executive and Chief Operating Officer of Abeona, stated, "We are excited to share updated positive efficacy and safety results that continue to suggest ABO-102 has the potential to be a life-altering treatment option for children with MPS IIIA, a rare, debilitating condition with no approved treatment that leads to progressive neurodevelopmental and physical decline, and often results in death early in life. We have requested a meeting with the FDA later this quarter to discuss the ABO-102 data and the potential path towards a Biologics License Application filing for ABO-102. In addition, the new results from the Transpher B study continue to support ABO-101's biologic activity in patients with MPS IIIB."

The updated results from the Transpher A study evaluating ABO-102 in Sanfilippo syndrome type A (MPS IIIA) demonstrated that neurocognitive development was preserved within normal range of a non-afflicted child for 2.5 years to 3 years (the latest time point measured) after treatment with ABO-102 ($3x10^{13}$ vg/kg) in three young patients in the high-dose cohort 3. The three young patients were treated with ABO-102 at ages 27 months, 19 months, and 12 months and are now at ages ranging from 3.5 years to 5+ years, the timepoint at which patients with MPS IIIA have already started to experience neurocognitive decline based on the natural history of disease progression. Dose-dependent and statistically significant reductions in cerebrospinal fluid heparan sulfate, denoting enzyme activity in the central nervous system (CNS), and liver volume were sustained for two years after treatment. ABO-102 has been well-tolerated with long-term safety remaining favorable 24-55 months following treatment. There have been no treatment-related severe adverse events and no clinically significant adverse events reported.

Kevin Flanigan, M.D., Director, Center for Gene Therapy at AWRI at Nationwide Children's and Transpher A study principal investigator, said, "The results presented today show a single intravenous dose of ABO-102 can help preserve neurocognitive development for up to 3 years following treatment in young MPS IIIA patients during early stages of their disease. The data shows ABO-102's ability to deliver a functional copy of the disease-causing *SGSH* gene to cells of the CNS and peripheral organs, as evidenced by the clinical benefits in neurocognition and biophysical measures and improvements in disease-specific biomarkers."

Results from the Transpher B study evaluating ABO-101 in Sanfilippo syndrome type B (MPS IIIB) showed that treatment with ABO-101 is associated with a dose-dependent and sustained improvement in central nervous system and systemic biomarkers, indicating the potent biologic effect of ABO-101 in patients with MPS IIIB. ABO-101 has been well-tolerated with no infusion related or early acute reactions and no clinically significant adverse events or laboratory abnormalities.

Maria Jose de Castro, M.D., Hospital Clínico Universitario Santiago de Compostela and Transpher B study investigator, said, "The results from the Transpher B study provide evidence of ABO-101's impact on disease biomarkers and potential to break down the accumulation of glycosaminoglycans that underlie MPS IIIB pathology. We look forward to continued follow-up to assess ABO-101's potential to preserve neurocognitive development in patients with MPS IIIB."

Investor Webinar on MPS III Gene Therapy Programs

Abeona management along with Dr. Flanigan and Dr. de Castro will host an investor webinar on February 16, 2021 at 1:00 p.m. EST. The live webinar, including audio and presentation slides, will be accessible at https://investors.abeonatherapeutics.com/events at the time of the meeting. To register in advance for the live webinar, clickhere.

An archived replay of the webinar will be available after the conclusion of the live event athttps://investors.abeonatherapeutics.com/events.

About the Annual WORLDSymposium™

The 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. For additional information on the 17th Annual WORLD*Symposium*™, please visit https://worldsymposia.org/.

About the Transpher A Study

The Transpher A Study (NCT02716246) is an ongoing, two-year, open-label, dose-escalation, Phase 1/2 global clinical trial assessing ABO-102 for the treatment of patients with Sanfilippo syndrome type A (MPS IIIA). The study, also known as ABT-001, is intended for patients 6 months to 2 years of age, or patients older than 2 years with a cognitive developmental quotient of 60% or above. ABO-102 gene therapy is delivered using AAV9 technology via a single-dose intravenous infusion. The study primary endpoints are neurodevelopment and safety, with secondary endpoints including behavior evaluations, quality of life, enzyme activity in cerebrospinal fluid (CSF) and plasma, heparan sulfate levels in CSF, plasma and urine, and brain and liver volume.

About the Transpher B Study

The Transpher B Study (NCT03315182) is an ongoing, two-year, open-label, dose-escalation, Phase 1/2 global clinical trial assessing ABO-101 for the treatment of patients with Sanfilippo syndrome type B (MPS IIIB). The study, also known as ABT-002, is intended for patients 6 months to 2 years of age, or patients older than 2 years with a cognitive developmental quotient of 60% or above. ABO-101 gene therapy is delivered using AAV9 technology via a single-dose intravenous infusion. The study primary endpoints are neurodevelopment and safety, with secondary endpoints including behavior evaluations, quality of life, enzyme activity in cerebrospinal fluid (CSF) and plasma, heparan sulfate levels in CSF, plasma and urine, and brain and liver volume.

About ABO-102

ABO-102 is a novel gene therapy in Phase 1/2 development for Sanfilippo syndrome type A (MPS IIIA), a rare lysosomal storage disease with no approved treatment that primarily affects the central nervous system (CNS). ABO-102 is dosed in a one-time intravenous infusion using a self-complementary AAV9 vector to deliver a functional copy of the SGSH gene to cells of the CNS and peripheral organs. The therapy is designed to address the underlying SGSH enzyme deficiency responsible for abnormal accumulation of glycosaminoglycans in the brain and throughout the body that results in progressive cell damage and neurodevelopmental and physical decline. In the U.S., Abeona holds Regenerative Medicine Advanced Therapy, Fast Track, Rare Pediatric Disease, and Orphan Drug designations for the ABO-102 clinical program. In the EU, the Company holds PRIME and Orphan medicinal product designations.

About ABO-101

ABO-101 is a novel gene therapy in Phase 1/2 development for Sanfilippo syndrome type B (MPS IIIB), a rare lysosomal storage disease with no approved therapy that primarily affects the central nervous system (CNS). ABO-101 is dosed in a one-time intravenous infusion using a self-complementary AAV9 vector to deliver a functional copy of the NAGLU gene to cells of the CNS and peripheral tissues. The therapy is designed to address the underlying NAGLU enzyme deficiency responsible for abnormal accumulation of glycosaminoglycans in the brain and throughout the body that results in progressive cell damage and neurodevelopmental and physical decline. In the U.S., Abeona holds Fast Track and Rare Pediatric Disease designations for ABO-101 and Orphan Drug designation in both the U.S. and EU.

About Sanfilippo Syndrome Type A (MPS IIIA)

Sanfilippo syndrome type A (MPS IIIA) is a rare, fatal lysosomal storage disease with no approved treatment that primarily affects the CNS and is characterized by rapid neurodevelopmental and physical decline. Children with MPS IIIA present with progressive language and cognitive decline and behavioral abnormalities. Other symptoms include sleep problems and frequent ear infections. Additionally, distinctive facial features with thick eyebrows or a unibrow, full lips and excessive body hair for one's age, and liver/spleen enlargement are also present in early childhood. MPS IIIA is caused by genetic mutations that lead to a deficiency in the SGSH enzyme responsible for breaking down glycosaminoglycans, which accumulate in cells throughout the body resulting in rapid health decline associated with the disorder.

About Sanfilippo syndrome type B (MPS IIIB)

Sanfilippo syndrome type B (MPS IIIB) is a rare and fatal lysosomal storage disease with no approved therapy that primarily affects the central nervous system and is characterized by rapid neurodevelopmental and physical decline. Children with MPS IIIB present with progressive language and cognitive decline and behavioral abnormalities. Other symptoms include sleep problems and frequent ear infections. Additionally, distinctive signs such as facial features with thick eyebrows or a unibrow, full lips and excessive body hair for one's age and liver/spleen enlargement are also present. The underlying cause of MPS IIIB is a deficiency in the NAGLU enzyme responsible for breaking down glycosaminoglycans, which accumulate throughout the body resulting in rapid decline associated with the disorder.

About Abeona Therapeutics

Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing gene and cell therapies for serious diseases. Abeona's clinical programs include EB-101, its autologous, gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa in Phase 3 development, as well as ABO-102 and ABO-101, novel AAV-based gene therapies for Sanfilippo syndrome types A and B (MPS IIIA and MPS IIIB), respectively, in Phase 1/2 development. The Company's portfolio also features AAV-based gene therapies for ophthalmic diseases with high unmet medical needs. Abeona's novel, next-generation AIM™ capsids have shown potential to improve tropism profiles for a variety of devastating diseases. Abeona's fully functional, gene and cell therapy GMP manufacturing facility produces EB-101 for the pivotal Phase 3 VIITAL™ study and is capable of clinical and commercial production of AAV-based gene therapies. For more information, visit www.abeonatherapeutics.com.

Forward-Looking Statements

This press release contains certain statements that are forward-looking within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, and that involve risks and uncertainties. These statements include statements about the Company exploring all strategic options, including the sale of some or all of its assets or sale of the Company. We have attempted to identify forward-looking statements by such terminology as "may," "will," "believe," "estimate," "expect," and similar expressions (as well as other words or expressions referencing future events, conditions or circumstances), which constitute and are intended to identify forward-looking statements. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, numerous risks and uncertainties, including but not limited to the potential impacts of the COVID-19 pandemic on our business, operations, and financial condition, the outcome of the strategic review, continued interest in our rare disease portfolio, our ability to enroll patients in clinical trials, the outcome of any future meetings with the U.S. Food and Drug Administration or other regulatory agencies, the impact of competition, the ability to secure licenses for any technology that may be necessary to commercialize our products, the ability to achieve or obtain necessary regulatory approvals, the impact of changes in the financial markets and global economic conditions, risks associated with data analysis and reporting, and other risks disclosed in the Company's most recent Annual Report on Form 10-K and subsequent quarterly reports on Form 10-Q and other periodic reports filed with the Securities and Exchange Commission. The Company undertakes no obligation to revise the forward-looking statements or to update them to reflect events or circumstances occurring after the date of this press release, whether as a result of new information, future developments or otherwise, except as required by the federal securities laws.

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