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): June 9, 2023

Vor Biopharma Inc.
(Exact name of registrant as specified in its Charter)

Delaware	**001-39979**	**81-1591163**
(State or Other Jurisdiction of Incorporation)	**(Commission File Number)**	**(IRS Employer Identification No.)**

100 Cambridgepark Drive **Suite 101** **Cambridge, Massachusetts**	**02140**
(Address of Principal Executive Offices)	**(Zip Code)**

Registrant's telephone number, including area code: (617) 655-6580

Not Applicable
(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 Instructions 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(s)	Name of each exchange on which registered
Common Stock, $0.0001 par value per share	VOR	The Nasdaq Stock Market LLC

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 Disclosure.

On June 9, 2023, Vor Biopharma Inc. (the "Company") issued a press release announcing updated clinical data from patients treated in VBP101, its Phase 1/2a multicenter, open-label, first-in-human study of trem-cel (formerly VOR33) in patients with acute myeloid leukemia. A copy of the press release is attached hereto as Exhibit 99.1 and is incorporated herein by reference. These data were presented by Guenther Koehne, MD, PhD, Deputy Director and Chief of Blood & Marrow Transplant and Hematologic Oncology at Miami Cancer Institute of Baptist Health South Florida in a poster presentation at the European Hematology Association (EHA) 2023 Congress in Frankfurt, Germany. In connection with the announcement, the Company will host a call and webcast on June 9, 2023 at 8:30 a.m. ET. Call details are contained in the press release referenced above. Accompanying slides may be accessed through the "Investors" section of the Company's website at www.vorbio.com and are 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 the purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that Section, nor shall it be deemed incorporated by reference in any filing made by the Company under the Securities Act of 1933, as amended, or the Exchange Act, except as shall be expressly set forth by specific reference in such a filing.

Item 8.01 Other Events.

Clinical Update

On June 9, 2023, the Company presented updated clinical data from patients treated in VBP101, its Phase 1/2a multicenter, open-label, first-in-human study of trem-cel in patients with AML. The time for neutrophil engraftment in all five patients treated with trem-cel (10-11 days) was similar to unedited transplants, suggesting that CD33 may be biologically dispensable. All patients achieved high levels of myeloid donor chimerism by day 28.

After achieving timely neutrophil engraftment and platelet recovery, patient 2 experienced secondary graft failure coincident with a detected coronavirus hKU1 infection and following administration of antimicrobial agents including trimethoprim-sulfamethoxazole, both of which may be associated with graft failure. A backup graft was administered, and neutrophil engraftment and platelet recovery were observed.

Patient 3 achieved timely neutrophil engraftment at Day 10, however platelets are still recovering. A platelet-reactive antibody was identified, and the patient is being treated for autoimmune thrombocytopenia. Platelets are steadily increasing and are at 15,000 per µL independent of transfusions.

Patients 4 and 5 achieved normal neutrophil engraftment and platelet recovery, providing further confidence in the Company's platform and approach. These patients have not experienced any unexpected adverse events to date.

Cell doses for all five patients were successfully manufactured and met release criteria, and all had a high level of CD33 editing efficiency.

As previously reported, neutrophil and platelet cell counts were maintained in patient 1 who received multiple Mylotarg doses at 0.5 mg/m^2, suggesting protection from Mylotarg-induced hematotoxicity.

Regulatory Update

The Company also announced on June 9, 2023 that the U.S. Food and Drug Administration has cleared its Investigational New Drug application for VCAR33^{ALLO}, a T-cell therapy derived from allogeneic healthy donors using a chimeric antigen receptor specifically binding to CD33.

VCAR33^{ALLO} is planned to be studied in the VBP301 clinical trial, which will focus on patients who have relapsed following allogeneic stem cell transplant where T cells harvested from the original donor are used as starting material for the drug product.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

Exhibit No.	Description
99.1	Press release, dated June 9, 2023.
104	Cover Page Interactive Data File (embedded within the Inline XBRL document).

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.

Vor Biopharma Inc.

By: /s/ Robert Ang
Robert Ang
Chief Executive Officer

Date: June 9, 2023

Successful Primary Engraftment of Trem-cel in First Five AML Patients Demonstrates Promise of Vor Bio's Platform

- *Patients transplanted with trem-cel achieved primary neutrophil engraftment and high levels of myeloid donor chimerism.*
- *Strong investigator enthusiasm and continued robust enrollment; additional data updates expected by year-end 2023.*
- *U.S. FDA clears VCAR33^{ALLO} IND.*
- *Conference call scheduled for today, June 9 at 8:30am ET.*

CAMBRIDGE, Mass., June 9, 2023 (GLOBE NEWSWIRE) — Vor Bio (Nasdaq: VOR), a clinical-stage cell and genome engineering company, today presented updated clinical data from patients treated in VBP101, its Phase 1/2a multicenter, open-label, first-in-human study of trem-cel (formerly VOR33) in patients with acute myeloid leukemia (AML). These data were presented by Guenther Koehne, MD, PhD, Deputy Director and Chief of Blood & Marrow Transplant and Hematologic Oncology at Miami Cancer Institute of Baptist Health South Florida in a poster presentation at the European Hematology Association (EHA) 2023 Congress in Frankfurt, Germany.

"Based on this interim update of five patients treated, we remain confident in the potential of our approach to enable targeted therapies post-transplant. Investigator enthusiasm is strong and proposal of patients for enrollment in the study currently exceeds the enrollment stagger. We look forward to sharing further engraftment and hematologic protection data from additional patients treated by year-end," said Eyal Attar, MD, Vor Bio Chief Medical Officer.

"The results being presented today are very encouraging. The unmet medical need, particularly for high-risk AML, is significant and this approach, if approved, could potentially transform outcomes for these patients," said Dr. Koehne.

The time for neutrophil engraftment in all five patients treated with trem-cel (10-11 days) was similar to unedited transplants, suggesting that CD33 may be biologically dispensable. All patients achieved high levels of myeloid donor chimerism by day 28.

After achieving timely neutrophil engraftment and platelet recovery, patient 2 experienced secondary graft failure coincident with a detected coronavirus hKU1 infection and following administration of antimicrobial agents including trimethoprim-sulfamethoxazole, both of which may be associated with graft failure. A backup graft was administered, and neutrophil engraftment and platelet recovery were observed.

Patient 3 achieved timely neutrophil engraftment at Day 10, however platelets are still recovering. A platelet-reactive antibody was identified, and the patient is being treated for autoimmune thrombocytopenia. Platelets are steadily increasing and are at 15,000 per µL independent of transfusions.

Patients 4 and 5 achieved normal neutrophil engraftment and platelet recovery, providing further confidence in the Company's platform and approach. These patients have not experienced any unexpected adverse events to date.

Cell doses for all five patients were successfully manufactured and met release criteria, and all had a high level of CD33 editing efficiency.

As previously reported, neutrophil and platelet cell counts were maintained in patient 1 who received multiple Mylotarg doses at 0.5 mg/m^2, suggesting protection from Mylotarg-induced hematotoxicity.

U.S. FDA clears VCAR33^{ALLO} IND

The Company also announced today that the U.S. Food and Drug Administration (FDA) has cleared its Investigational New Drug (IND) application for VCAR33^{ALLO}, a T-cell therapy derived from allogeneic healthy donors using a chimeric antigen receptor (CAR) specifically binding to CD33. VCAR33^{ALLO} is planned to be studied in the VBP301 clinical trial, which will focus on patients who have relapsed following allogeneic stem cell transplant where T cells harvested from the original donor are used as starting material for the drug product.

Conference Call & Webcast Information

Members of the Vor Bio management team, joined by Dr. Guenther Koehne, will conduct a live conference call and webcast today at 8:30 am Eastern Time.

Listeners can register for the webcast via this LINK.

Analysts wishing to participate in the Q&A session should use this LINK.

A replay of the webcast will be available via the investor section of the Company's website at www.vorbio.com approximately two hours after the call's conclusion.

About AML

AML is the most common type of acute leukemia in adults and one of the deadliest and most aggressive blood cancers, affecting 20,000 newly diagnosed patients each year in the United States. Approximately half of patients with AML who receive a hematopoietic cell transplant (HCT) suffer a relapse of their leukemia, with two-year survival rates of less than 20%, and relapse rates are higher for patients with certain adverse risk features. The fragility of engrafted hematopoietic stem cells prevents treatment following transplant, giving the cancer a chance to return.

About the VBP101 Clinical Trial

VBP101 is a Phase 1/2a, multicenter, open-label, first-in-human study of trem-cel (VOR33) in participants with AML who are undergoing human leukocyte antigen (HLA)-matched allogeneic HCT. Trem-cel is an allogeneic CRISPR/Cas9 genome-edited hematopoietic stem and progenitor cell (HSPC) therapy product, lacking the CD33 protein. It is being investigated for participants with CD33$^+$ AML at high risk for relapse after HCT to allow post-HCT targeting of residual CD33$^+$ acute AML cells using Mylotarg (gemtuzumab ozogamicin) without toxicity to engrafted cells. Participants undergo a myeloablative HCT with matched related or unrelated donor CD34-selected HSPCs engineered to remove CD33 expression (trem-cel drug product). Mylotarg is given after engraftment for up to four cycles. The primary endpoint is the incidence of successful engraftment, defined as the first day of 3 consecutive days of absolute neutrophil count (ANC) ≥500 cells/mm^2 by day 28. Part 1 of this study is evaluating the safety of escalating Mylotarg dose levels to determine the maximum tolerated dose (MTD) and recommended Phase 2 dose. Part 2 will expand the number of participants to evaluate the Mylotarg recommended Phase 2 dose. For more information, visit: https://clinicaltrials.gov/ct2/show/NCT04849910

About Trem-cel

Tremtelectogene empogeditemcel (trem-cel), formerly VOR33, is a genome-edited hematopoietic stem and progenitor allogeneic donor product candidate where CD33 has been deleted using genome engineering. Transplant with trem-cel is designed to replace standard of care transplants for patients suffering from AML and potentially other blood cancers. Trem-cel has the potential to enable powerful targeted therapies in the post-transplant setting including CD33-targeted CAR-T cells.

About Vor Bio

Vor Bio is a clinical-stage cell and genome engineering company that aims to change the standard of care for patients with blood cancers by engineering hematopoietic stem cells to enable targeted therapies post-transplant. For more information, visit: www.vorbio.com.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. The words “aim,” “anticipate,” “can,” “continue,” “could,” “design,” “enable,” “expect,” “initiate,” “intend,” “may,” “on-track,” “ongoing,” “plan,” “potential,” “should,” “target,” “update,” “will,” “would,” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Forward-looking statements in this press release include Vor Bio’s statements regarding the potential of its product candidates to positively impact quality of life and alter the course of disease in the patients it seeks to treat, the timing and pace of patient enrollment in clinical trials and the availability of data therefrom, the expected safety profile of its product candidates, the potential of trem-cel to enable targeted therapies in the post-transplant setting including Mylotarg and CD33-targeted CAR-Ts. Vor Bio may not actually achieve the plans, intentions, or expectations disclosed in these forward-looking statements, and you should not place undue reliance on these forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in these forward-looking statements as a result of various factors, including: uncertainties inherent in the initiation and completion of preclinical studies and clinical trials and clinical development of Vor Bio’s product candidates; availability and timing of results from preclinical studies and clinical trials; whether interim results from a clinical trial will be predictive of the final results of the trial or the results of future trials; whether successful engraftment and platelet recovery will ultimately lead to efficacy of trem-cel; expectations for regulatory approvals to conduct trials or to market products; the success of Vor Bio’s in-house manufacturing capabilities and efforts; and availability of funding sufficient for its foreseeable and unforeseeable operating expenses and capital expenditure requirements. These and other risks are described in greater detail under the caption “Risk Factors” included in Vor Bio’s most recent annual or quarterly report and in other reports it has filed or may file with the Securities and Exchange Commission. Any forward-looking statements contained in this press release speak only as of the date hereof, and Vor Bio expressly disclaims any obligation to update any forward-looking statements, whether because of new information, future events or otherwise, except as may be required by law.

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