

**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 16, 2023

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

Delaware
(State or Other Jurisdiction
of Incorporation)

001-39979
(Commission
File Number)

81-1591163
(IRS Employer
Identification No.)

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

02140
(Zip Code)

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

(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	Nasdaq Global Select Market

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 February 16, 2023, Vor Biopharma Inc. (the “Company”) issued a press release announcing additional clinical data from VBP101, its Phase 1/2a multicenter, open-label, first-in-human study of trem-cel (previously 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.

The information furnished under this Item 7.01, including Exhibit 99.1, shall not be deemed “filed” for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or subject to the liabilities of that section or Sections 11 and 12(a)(2) of the Securities Act of 1933, as amended. The information in this Item 7.01, including Exhibit 99.1, shall not be deemed incorporated by reference into any other filing with the U.S. Securities Exchange Commission made by the Company, whether made before or after the date hereof, regardless of any general incorporation language in such filing.

Item 8.01 Other Events

Clinical Update

On February 16, 2023, the Company announced additional clinical data from VBP101, its Phase 1/2a multicenter, open-label, first-in-human study of trem-cel (previously VOR33) in patients with acute myeloid leukemia. In the first patient, trem-cel maintained hematopoiesis through three cycles of Mylotarg (gemtuzumab ozogamicin), which was well-tolerated at the initial dose level of 0.5 mg/m². A second patient has successfully received a trem-cel transplant and engrafted normally.

Trem-cel Safety & Durability

Patient 1 maintained neutrophil and platelet counts approximately five months (147 days) after transplantation with trem-cel. Due to detectable measurable residual disease, Patient 1 was moved to other therapies following administration of the third dose of Mylotarg, subsequently relapsed, and remains on study for long-term follow-up.

Similar to Patient 1, Patient 2 successfully received a trem-cel transplant and showed robust cell recovery with neutrophil engraftment occurring at Day 11 and platelet recovery on Day 17. Trem-cel was well tolerated in both patients, with no related and no unexpected adverse events (“AEs”) reported.

No Hematological Toxicity Observed Through Repeated Doses of Mylotarg

In Patient 1, neutrophil and platelet cell counts were maintained following three sequential Mylotarg doses at 0.5 mg/m². This suggests potential protection from Mylotarg-related hematotoxicity. The only AE observed possibly related to Mylotarg through dose 3 was low grade nausea and vomiting, a known side-effect of Mylotarg. Mylotarg first-dose pharmacokinetics revealed 0.5 mg/m² achieved C_{max} and AUC parameters equivalent to 1-2 and 4-5 mg/m² accordingly, potentially due to the decreased CD33 antigen sink.

Evidence of Mylotarg Causing CD33-negative Donor Cell Enrichment

In Patient 1, CD33-negative donor hematopoiesis was enriched across hematopoietic cell types following Mylotarg administration. In addition, the CD33 deletion was observed in donor cells of myeloid and lymphoid origin which were both enriched following Mylotarg, suggesting that CD33 is expressed in early hematopoietic cells and that Mylotarg treatment enriches for edited donor cells.

Interest in enrollment in VBP101 continues to be strong with a high level of investigator enthusiasm at all nine study sites. The company is moving forward with dose escalation of Mylotarg per the 3+3 dose escalation schema in the protocol. The Company is also on-track to submit an IND in the first half of 2023 for VCAR33^{ALLO}, a CAR-T therapy using allogeneic healthy donor-derived cells, which it intends use in combination with trem-cel as a Treatment System.

Item 9.01 Financial Statements and Exhibits

<u>Exhibit No.</u>	<u>Description</u>
99.1	Press Release, dated February 16, 2023
104	Cover Page Interactive Data File (the cover page XBRL tags are 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: February 16, 2023



First AML Patient Transplanted with Vor Bio's Trem-cel Demonstrated Durable Engraftment through Multiple Mylotarg™ Cycles at Initial Dose Level

- *Trem-cel exhibited robust engraftment five months post-transplant through three cycles of Mylotarg*
- *Mylotarg treatment enriched CD33-negative donor hematopoiesis*
- *Second patient successfully received trem-cel transplant and achieved neutrophil engraftment and platelet recovery*

CAMBRIDGE, Mass., February 16, 2023 (GLOBE NEWSWIRE) — Vor Bio (Nasdaq: VOR), a clinical-stage cell and genome engineering company, presented clinical data from VBP101, its Phase 1/2a multicenter, open-label, first-in-human study of trem-cel (previously VOR33) in patients with acute myeloid leukemia (AML). In the first patient, trem-cel maintained hematopoiesis through three cycles of Mylotarg (gemtuzumab ozogamicin), which was well-tolerated at the initial dose level of 0.5 mg/m². A second patient has successfully received a trem-cel transplant and engrafted normally. These data were presented today by Miguel-Angel Perales, MD, Chief, Adult Bone Marrow Transplant Service, Memorial Sloan Kettering Cancer Center in a late-breaking [poster](#) at the 2023 Tandem Meetings (Transplantation & Cellular Therapy Meetings of ASTCT™ and CIBMTR®) in Orlando, FL.

“These data continue to demonstrate the potential of trem-cel as a next-generation hematopoietic stem cell transplant, which could transform treatment for patients with blood cancers such as AML,” said Dr. Eyal Attar, Vor Bio’s Chief Medical Officer. “We are also encouraged that a second patient successfully received a trem-cel transplant and look forward to learning more as we treat additional patients and dose escalate Mylotarg. In addition, we plan to file an IND for VCAR33^{ALLO}, a novel allogeneic CAR-T treatment which we believe could be more efficacious than Mylotarg in combination with a trem-cel transplant.”

Trem-cel Safety & Durability

Patient 1 maintained neutrophil and platelet counts approximately five months (147 days) after transplantation with trem-cel. Due to detectable measurable residual disease (MRD), Patient 1 was moved to other therapies following administration of the third dose of Mylotarg, subsequently relapsed, and remains on study for long-term follow-up.

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Interest in enrollment in VBP101 continues to be strong with a high level of investigator enthusiasm at all nine study sites. The company is moving forward with dose escalation of Mylotarg per the 3+3 dose escalation schema in the protocol. The Company is also on-track to submit an IND in the first half of 2023 for VCAR33^{ALLO}, a CAR-T therapy using allogeneic healthy donor-derived cells, which it intends use in combination with trem-cel as a Treatment System.

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 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 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/mm² 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,” “believe,” “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 feasibility of a trem-cel transplant to be successfully manufactured, to engraft normally, to maintain blood counts following treatment with Mylotarg following allogeneic hematopoietic cell transplant and to be well tolerated, the potential of Vor Bio’s platform, and timing expectations for additional regulatory filings. 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 the product candidate; 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. The interim data presented in this press release is based on two patients and future results for these patients or additional patients may not produce the same or consistent results. 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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