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): March 17, 2022

AVROBIO, INC.
(Exact name of registrant as specified in its charter)

Delaware	**001-38537**	**81-0710585**
(State or other jurisdiction of incorporation)	**(Commission File Number)**	**(I.R.S. Employer Identification No.)**

One Kendall Square
Building 300, Suite 201
Cambridge, MA 02139
(Address of principal executive offices, including zip code)

(617) 914-8420
(Registrant's telephone number, including area code)

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:

☐ 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	**AVRO**	**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 2.02. Results of Operations and Financial Condition.

On March 17, 2022, AVROBIO, Inc. (the "Company") issued a press release containing information about the Company's results of operations for the three months and year ended December 31, 2021. A copy of this press release is furnished as Exhibit 99.1 to this Current Report on Form 8-K.

The information in this Form 8-K (including Exhibit 99.1) shall not be deemed "filed" for purposes 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 under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such a filing.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits

99.1	Press release issued by AVROBIO, Inc., dated March 17, 2022.
104	The cover page from this Current Report on Form 8-K, formatted in Inline XBRL.

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.

AVROBIO, INC.

Date: March 17, 2022

By: /s/ Geoff MacKay
Geoff MacKay
President and Chief Executive Officer

Exhibit 99.1

AVROBIO Reports Fourth Quarter and Fiscal Year 2021 Financial Results and Provides Business Update

Provided interim data at WORLDSymposium™ 2022 that showed sustained engraftment across first three patients 1+ year post-gene therapy in Phase 1/2 clinical trial[i] for cystinosis; all remain off oral cysteamine to date

Interim clinical data update of AVR-RD-02 in Gaucher disease type 1 planned for 2022; third patient dosed in Phase 1/2 clinical trial

Regulatory interactions planned in 2022 to inform clinical development and registration strategies for programs in Gaucher disease, cystinosis, Hunter syndrome and Pompe disease

Strong balance sheet with cash runway into Q1 2024

CAMBRIDGE, Mass., March 17, 2022 — AVROBIO, Inc. (Nasdaq: AVRO), a leading clinical-stage gene therapy company with a mission to free people from a lifetime of genetic disease, today reported financial results for the fourth quarter and year ended Dec. 31, 2021, and provided a business update.

"After a busy 2021 with new patients dosed across three clinical trials, we acted in early January to strategically position AVROBIO for long-term success by reprioritizing our leading lysosomal disorder pipeline. Four weeks after, we reinforced that position by releasing strong interim data from the Phase 1/2 collaborator-sponsored clinical trial for cystinosis at the WORLD*Symposium*™ 2022," said Geoff MacKay, president and CEO of AVROBIO. "We look forward to sharing new data on the three patients dosed to date in our Gaucher disease type 1 program in 2022 and providing updates for our other pipeline programs following meetings with regulatory agencies planned for later this year. We potentially have many exciting catalysts ahead of us and believe that our strong balance sheet will support continued progress through anticipated milestones and into the first quarter of 2024."

Program Updates

Presented interim data from Phase 1/2 clinical trial of AVR-RD-04 in cystinosis at the 18th Annual WORLD*Symposium*™ 2022:

- First three patients dosed to date remain off oral cysteamine with follow up durations ranging between 12- and 26-months post-gene therapy infusion. Sustained engraftment has been observed in each of these patients, as evidenced by stable vector copy number (VCN) levels.
- Reduction in number of cystine crystals as measured in skin and intestinal mucosa biopsies observed across these three patients.
- A fourth patient was infused in November 2021.
- No adverse events (AEs) related to the drug product have been reported in the four patients infused to date. All AEs observed have been attributed to myeloablative conditioning, stem cell mobilization, underlying disease or pre-existing conditions.

- Clinical proof-of-concept in adult patients lays the groundwork for potential AVROBIO-sponsored trial planned to begin in 2023.
- The company hosted a conference call providing a full data update (see here).

Dosed a third patient in our Phase 1/2 GUARD1 clinical trial in Gaucher disease type 1

Deprioritized Fabry disease program in January 2022 to focus pipeline on Gaucher disease programs, cystinosis, Hunter syndrome and Pompe disease programs, extending cash runway into the first quarter of 2024

Presented updated safety data on first 14 patients treated across two AVROBIO clinical trials at the virtual 28th Annual Congress of the European Society of Gene & Cell Therapy (ESGCT)

- No AEs or serious adverse events related to drug product in 14 patients dosed in Phase 1 and 2 Fabry disease trials and Phase 1/2 Gaucher disease trial. All AEs observed have been attributed to myeloablative conditioning, stem cell mobilization, underlying disease or pre-existing conditions.
- Post-gene therapy administration follow-up out more than 4 ½ years for first patient infused.
- AVROBIO shared new industry-leading techniques designed to better elucidate the safety profile of investigational gene therapies at cellular level.
- Full data can be accessed here.

Business Updates

- Appointed Sean O'Bryan, who brings a wealth of experience in regulatory strategy and product development for cell and gene therapies, to chief regulatory officer in February 2022.
- Appointed Azadeh Golipour, Ph.D., who has filled multiple roles with increasing responsibility during her five-year career at AVROBIO, to chief technology officer in January 2022.
- Appointed Essra Ridha, M.D., MRCP, FFPM, who was previously clinical development lead at AVROBIO and has extensive experience in cell and gene therapy development gained at Sangamo Therapeutics and GlaxoSmithKline, to chief medical officer in October 2021.

Anticipated Milestones Over the Next 12 Months:

- *AVR-RD-04 in cystinosis:* Plan to engage with regulatory agencies to discuss clinical development and regulatory strategy with the intent of initiating a company-sponsored clinical trial in 2023, subject to regulatory clearance.
- *Advancing our Gaucher disease franchise:*
  - *AVR-RD-02 in Gaucher disease type 1:* Plan to provide an interim clinical data update in 2022.
  - *AVR-RD-06 in Gaucher disease type 3:* Plan to engage with regulatory agencies on a Phase 2/3 clinical development strategy.
- *AVR-RD-05 in Hunter syndrome:* Subject to regulatory clearance, collaborators at the University of Manchester plan to initiate a Phase 1/2 clinical trial in 2023.

- *AVR-RD-03 in Pompe disease:* Plan to engage with regulatory agencies on the clinical development strategy and plan to initiate a clinical trial in 2023, subject to regulatory clearance.

Fourth Quarter and Year End 2021 Financial Results

AVROBIO reported a net loss of $28.2 million for the fourth quarter of 2021, and a net loss of $119.1 million for the year ended 2021, as compared to a net loss of $28.1 million and a net loss of $119.7 million for the comparable periods in 2020, respectively.

Research and development expenses were $19.0 million for the fourth quarter of 2021, and $83.1 million for the year ended 2021, as compared to $19.6 million and $87.2 million for the comparable periods in 2020, respectively. These decreases were driven by decreased program development expenses, including $9.1 million in non-recurring license fees incurred in 2020, which included an $8.0 million expense related to a one-time, upfront fee paid as consideration for in-licensing the Hunter syndrome program, which were partially offset by an increase in personnel-related costs.

General and administrative expenses were $9.0 million for the fourth quarter of 2021, and $35.7 million for the year ended 2021, as compared to $8.5 million and $33.0 million for the comparable periods in 2020, respectively. These increases were primarily due to an increase in personnel-related costs and non-cash stock-based compensation, which were partially offset by a decrease in professional fees, legal fees and facilities costs.

As of Dec. 31, 2021, AVROBIO had $189.6 million in cash and cash equivalents, as compared to $259.7 million in cash and cash equivalents as of Dec. 31, 2020. Based on the company's current operating plan, AVROBIO expects its cash and cash equivalents as of Dec. 31, 2021 will enable the company to fund its operating expenses and capital expenditure requirements into the first quarter of 2024.

About AVROBIO

Our vision is to bring personalized gene therapy to the world. We aim to prevent, halt and/or reverse disease throughout the body with a single dose of gene therapy designed to drive durable expression of therapeutic protein, even in hard-to-reach tissues and organs including brain, muscle and bone. AVROBIO's pipeline is powered by our industry-leading plato® gene therapy platform, our foundation designed to deliver gene therapy worldwide. It includes clinical programs in cystinosis and Gaucher disease type 1, as well as preclinical programs in Gaucher disease type 3, Hunter syndrome and Pompe disease. We are headquartered in Cambridge, Mass. For additional information, visit avrobio.com, and follow us on Twitter and LinkedIn.

Forward-Looking Statement

This press release contains forward-looking statements, including statements made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995. These statements may be identified by words and phrases such as "aims," "anticipates," "believes,"

“could,” “designed to,” “estimates,” “expects,” “forecasts,” “goal,” “intends,” “may,” “plans,” “possible,” “potential,” “seeks,” “will,” and variations of these words and phrases or similar expressions that are intended to identify forward-looking statements. These forward-looking statements include, without limitation, statements regarding our business strategy for and the potential therapeutic benefits of our current and prospective product candidates, the expected safety profile of our investigational gene therapies, results of preclinical studies, the design, commencement, enrollment and timing of ongoing or planned clinical trials, clinical trial results, product approvals and regulatory pathways, the timing of patient recruitment and enrollment activities, our plans and expectations with respect to interactions with regulatory agencies, timing and likelihood of success, the expected benefits and results of our implementation of the plato® platform in our clinical trials and gene therapy programs and its potential impact on our manufacturing and commercialization activities, and statements regarding our financial and cash position and expected cash runway, including impact on anticipated milestones. Any such statements in this press release that are not statements of historical fact may be deemed to be forward-looking statements. Results in preclinical or early-stage clinical trials may not be indicative of results from later stage or larger scale clinical trials and do not ensure regulatory approval. You should not place undue reliance on these statements, or the scientific data presented.

Any forward-looking statements in this press release are based on AVROBIO’s current expectations, estimates and projections about our industry as well as management’s current beliefs and expectations of future events only as of today and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties include, but are not limited to, the risk that any one or more of AVROBIO’s product candidates will not be successfully developed or commercialized, the risk of cessation or delay of any ongoing or planned clinical trials of AVROBIO or our collaborators, the risk that AVROBIO may not successfully recruit or enroll a sufficient number of patients for our clinical trials, the risk that AVROBIO may not realize the intended benefits of our gene therapy platform, including the features of our plato® platform, the risk that our product candidates or procedures in connection with the administration thereof will not have the safety or efficacy profile that we anticipate, the risk that prior results, such as signals of safety, activity or durability of effect, observed from preclinical or clinical trials, will not be replicated or will not continue in ongoing or future studies or trials involving AVROBIO’s product candidates, the risk that we will be unable to obtain and maintain regulatory approval for our product candidates, the risk that the size and growth potential of the market for our product candidates will not materialize as expected, risks associated with our dependence on third-party suppliers and manufacturers, including sole source suppliers, risks regarding the accuracy of our estimates of expenses and future revenue, risks relating to our capital requirements and needs for additional financing, risks relating to clinical trial and business interruptions resulting from the COVID-19 outbreak or similar public health crises, including that such interruptions may materially delay our enrollment and development timelines and/or increase our development costs or that data collection efforts may be impaired or otherwise impacted by such crises, and risks relating to our ability to obtain and maintain intellectual property protection for our product candidates.

For a discussion of these and other risks and uncertainties, and other important factors, any of which could cause AVROBIO's actual results to differ materially and adversely from those contained in the forward-looking statements, see the section entitled "Risk Factors" in AVROBIO's most recent Annual or Quarterly Report, as well as discussions of potential risks, uncertainties and other important factors in AVROBIO's subsequent filings with the Securities and Exchange Commission. AVROBIO explicitly disclaims any obligation to update any forward-looking statements except to the extent required by law.

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CONDENSED CONSOLIDATED BALANCE SHEETS
(In thousands)
(Unaudited)

	December 31, 2021	December 31, 2020
Cash and cash equivalents	$ 189,567	$ 259,682
Prepaid expenses and other current assets	9,578	7,560
Property and equipment, net	4,126	3,064
Other assets	566	928
Total assets	$ 203,837	$ 271,234
Accounts payable	$ 3,486	$ 2,682
Accrued expenses and other current liabilities	15,900	13,932
Note payable, net of discount	14,945	—
Deferred rent, net of current portion	30	276
Total liabilities	34,361	16,890
Total stockholders' equity	169,476	254,344
Total liabilities and stockholders' equity	$ 203,837	$ 271,234

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
(In thousands, except per share data)
(Unaudited)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2021	2020	2021	2020
Operating expenses:				
Research and development	$ 19,000	$ 19,587	$ 83,114	$ 87,236
General and administrative	8,962	8,477	35,727	32,992
Total operating expenses	27,962	28,064	118,841	120,228
Loss from operations	(27,962)	(28,064)	(118,841)	(120,228)
Total other income (expense), net	(265)	(67)	(285)	516
Net loss	$(28,227)	$(28,131)	$(119,126)	$(119,712)
Net loss per share—basic and diluted	$ (0.65)	$ (0.73)	$ (2.78)	$ (3.31)
Weighted-average number of common shares outstanding—basic and diluted	43,648	38,528	42,854	36,206

[i] Collaborator-sponsored Phase 1/2 clinical trial of AVR-RD-04 is funded in part by grants to UCSD from the California Institute for Regenerative Medicine (CIRM), Cystinosis Research Foundation (CRF) and National Institutes of Health (NIH)