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): May 13, 2021

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 May 13, 2021, AVROBIO, Inc. (the "Company") issued a press release containing information about the Company's results of operations for the three months ended March 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 May 13, 2021.
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: May 13, 2021

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

Exhibit 99.1

AVROBIO Reports First Quarter 2021 Financial Results and Provides Business Update

Held meeting with the U.S. Food and Drug Administration (FDA) to discuss the regulatory path for AVR-RD-01 for Fabry disease

Dosing completion in ongoing FAB-GT Phase 2 trial reaches 50%, with three additional patients dosed in three months

CAMBRIDGE, Mass., May 13, 2021 — 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 quarter ended March 31, 2021 and provided a business update.

“During the first quarter, we met with the U.S. Food and Drug Administration (FDA) to discuss the regulatory path for AVR-RD-01, our investigational gene therapy for Fabry disease. We plan to engage with FDA in the second half of 2021 to seek feedback on our proposal for a registration trial,” said Geoff MacKay, president and CEO of AVROBIO. “In addition, we started the year with strong data updates from all three of our clinical programs – Fabry disease, Gaucher disease and cystinosis – at the WORLD*Symposium*™. These data show sustained improvements across important biomarkers and functional metrics, which we believe support the potential of our lentiviral gene therapy platform to treat life-limiting lysosomal disorders. We have been gratified by the strong interest in our clinical trials, and we’re pleased with our progress in enrolling and dosing new patients, including three patients dosed in just the last three months in our FAB-GT trial. As we advance AVR-RD-01, we plan to continue to execute across our pipeline, and we look forward to providing future updates.”

Program Updates and Milestones

Held meeting with FDA to discuss the regulatory path for AVR-RD-01 for Fabry disease in the first quarter of 2021

- Based on a recent U.S. regulatory development for Fabry disease therapies, AVROBIO intends to discuss with FDA a potential registration trial with a proposed primary efficacy surrogate endpoint of clearance of Gb3 inclusions in biopsied renal peritubular capillaries as the basis for potential full approval. See full update here.
- Subject to FDA feedback, AVROBIO intends to pursue full approval for investigational AVR-RD-01 as a first-line therapy for Fabry disease by conducting a single, head-to-

head registration trial versus Fabrazyme® (agalsidase beta)[1] using a kidney biopsy surrogate endpoint similar to the FAB-GT Phase 2 trial, where the company has seen 100% and 87% substrate reductions at one year post-gene therapy in the two patients with evaluable kidney biopsies. The company anticipates proposing to FDA a registration trial design with a scope, size and duration comparable to other gene therapy trials.

- Although FDA guidance provides that a surrogate endpoint that was the basis for approval in a particular clinical development program should not be assumed to be appropriate for use in a different program, AVROBIO believes Fabrazyme's full approval based on a similar kidney biopsy surrogate endpoint could potentially support the use of such an endpoint in a registration trial of AVR-RD-01.

Three additional patients dosed in three months and plans to amend FAB-GT trial protocol

- Three patients have now been dosed over the past three months in the FAB-GT Phase 2 trial. Seven patients of the up to 14 planned trial participants have been dosed to date.
- AVROBIO expects to amend the FAB-GT trial protocol in the second quarter of 2021 to help support the potential use of AVR-RD-01 in a broad Fabry disease population. The company plans to enroll female participants, eliminate antibody-status exclusions and add the collection of data on additional parameters that are recognized to be limitations of enzyme replacement therapy (ERT), such as endpoints to assess the gene therapy's potential ability to address cardiovascular and central nervous system manifestations.

Four data abstracts accepted for presentation at the 24th virtual Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT), May 11-14, 2021

- New data updates from an investigator-sponsored Phase 1/2 trial[2] in cystinosis and from AVROBIO's preclinical program in Pompe disease, as well as encore data from AVROBIO's clinical program in Gaucher disease type 1, have been presented.
- Encore data from AVROBIO's clinical program in Fabry disease will be presented on Friday, May 14, 2021.
- See more information here.

1 Fabrazyme® (agalsidase beta) is a registered trademark owned by Sanofi Genzyme.

2 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).

Provided robust data updates for Fabry disease, cystinosis and Gaucher disease type 1 during WORLD*Symposium*™ in February 2021

- Announced 100% kidney substrate reduction at 12 months post-gene therapy in the first patient dosed with the plato® platform in the FAB-GT trial, together with additional data from multiple biomarkers and functional cardiac measurements across both the Phase 1 and Phase 2 Fabry trials.
- Showed improvement across multiple measures for first two patients dosed in the investigator-sponsored Phase 1/2 trial in cystinosis, and substantial improvement in photophobia in the first patient at 12 months post-gene therapy.
- Presented early data from the Phase 1/2 clinical trial in Gaucher disease type 1 showing plasma chitotriosidase levels decreased 49% and toxic metabolite lyso-Gb1 levels decreased 44% in the first patient at six months post-gene therapy, compared to the patient's pre-treatment levels while on ERT.
- Additional details on the data presented during the 17th annual WORLD*Symposium*™ can be found here.

First Quarter 2021 Financial Results

AVROBIO reported a net loss of $26.9 million for the first quarter of 2021 as compared to a net loss of $26.0 million for the comparable period in 2020. This increase was driven by increased research and development expenses and a decrease in other (expense) income, net.

Research and development expenses were $18.5 million for the first quarter of 2021 as compared to $18.3 million for the comparable period in 2020. This increase was driven by increased program development activities related to the advancement of the company's pipeline, as well as increased personnel-related costs resulting from an increase in employee headcount, which includes the impact of non-cash stock-based compensation.

General and administrative expenses were $8.4 million for the first quarter of 2021 as compared to $8.3 million for the comparable period in 2020. This increase was primarily due to an increase in employee headcount, which includes the impact of non-cash stock-based compensation, which was offset by a decrease in consulting costs and professional fees.

Other (expense) income, net was $0.02 million in expense for the first quarter of 2021 as compared to other (expense) income, net of $0.6 million in income for the comparable period in 2020. This decrease was driven by a reduction in interest income.

As of March 31, 2021, AVROBIO had $233.0 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 March 31, 2021, will enable the company to fund its operating expenses and capital expenditure requirements into the first quarter of 2023.

About AVROBIO

Our vision is to bring personalized gene therapy to the world. We aim to prevent, halt 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. Our ex vivo lentiviral gene therapy pipeline includes clinical programs in Fabry disease, Gaucher disease type 1 and cystinosis, as well as preclinical programs in Hunter syndrome, Gaucher disease type 3 and Pompe disease. AVROBIO is powered by our industry leading plato® gene therapy platform, our foundation designed to deliver gene therapy worldwide. We are headquartered in Cambridge, Mass., with an office in Toronto, Ontario. For additional information, visit avrobio.com, and follow us on Twitter and LinkedIn.

Forward-Looking Statements

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 product candidates, the design, commencement, enrollment and timing of ongoing or planned clinical trials, clinical trial results, product approvals and regulatory pathways, our plans and expectations with respect to the development of AVR-RD-01, including timing and design of our potential registration trial, the intended use of such trial as our registration trial for this product candidate, anticipated interactions with regulatory agencies and the planned use of surrogate endpoints in future development of AVR-RD-01, anticipated benefits of our gene therapy platform including potential impact on our commercialization activities, 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, the expected safety profile of our investigational gene therapies, and statements regarding our financial and cash position and expected cash runway. 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 regulatory agencies may disagree with our anticipated development approach for our product candidates such as AVR-RD-01, including that we may not be able to utilize our planned registration trial of AVR-RD-01 for full approval but instead be required to conduct additional testing, that we may be required to conduct our planned testing in a more time-consuming, expensive, challenging or otherwise different manner than we envision or have conducted for our existing trials, particularly in light of the FDA's preference for clinical trials to be double-blinded and potentially include sham controls, the risk that we may not be able to utilize our envisioned surrogate endpoint to support full approval of AVR-RD-01 but instead be required to measure a different endpoint such as a clinical outcome, 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, 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)

	March 31, 2021	December 31, 2020
Cash and cash equivalents	$233,027	$ 259,682
Prepaid expenses and other current assets	9,037	7,560
Property and equipment, net	2,930	3,064
Other assets	918	928
Total assets	$245,912	$ 271,234
Accounts payable	$ 2,698	$ 2,682
Accrued expenses and other current liabilities	10,070	13,932
Deferred rent, net of current portion	214	276
Total liabilities	$ 12,982	$ 16,890
Total stockholders' equity	232,930	254,344
Total liabilities and stockholders' equity	$245,912	$ 271,234

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

	Three Months Ended March 31,	
	2021	2020
Operating expenses:		
Research and development	$ 18,527	$ 18,274
General and administrative	8,357	8,315
Total operating expenses	26,884	26,589
Loss from operations	(26,884)	(26,589)
Other (expense) income, net	(15)	616
Net loss	$(26,899)	$(25,973)
Net loss per share — basic and diluted	$ (0.65)	$ (0.77)
Weighted-average number of common shares outstanding — basic and diluted	41,618	33,667