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 18, 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 March 18, 2021, 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, 2020. 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 18, 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: March 18, 2021

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

AVROBIO Reports Fourth Quarter and Fiscal Year 2020 Financial Results and Provides Business Update

100% kidney substrate reduction at 12 months post-gene therapy in the first patient dosed with the plato® gene therapy platform in FAB-GT[1] trial for Fabry disease

One new patient dosed and three new patients enrolled in Fabry disease clinical trial since January 2021

Orphan drug designation received from European Commission for AVR-RD-04 for cystinosis and AVR-RD-01 for Fabry disease

Balance sheet strengthened with a $75 million follow-on common stock offering extending anticipated cash runway into Q1 2023

CAMBRIDGE, Mass., March 18, 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 fourth quarter and year ended Dec. 31, 2020 and provided a business update.

“It has been a highly productive period for AVROBIO, with strong new data updates across all our clinical programs, underscoring the transformative potential of our ex vivo lentiviral gene therapies. Most dramatic was the 100% reduction in toxic substrate reported from the FAB-GT trial second evaluable biopsy, but we are equally pleased with the compelling data from other biomarkers and functional metrics across the cystinosis and Gaucher disease type 1 programs,” said Geoff MacKay, president and CEO of AVROBIO. “We also shared exciting preclinical data from our Hunter syndrome and Pompe disease programs that reinforced the potential of our second wave of programs, which aim to prevent these lysosomal disorders’ devastating neurological complications. These data bolster our confidence in the potential of our investigational gene therapies and inspire our continuing work that seeks to prevent, halt or even reverse lysosomal disorders with a single dose.”

Since January, the company has dosed a fifth patient and enrolled three additional patients in the FAB-GT trial for Fabry disease.

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1 Fabry Phase 2 trial, formerly known as FAB-201

“In 2021, we’re focused on operational execution as we prepare for a number of important milestones, including meeting with the U.S. Food and Drug Administration (FDA) to discuss the regulatory path for AVR-RD-01 for Fabry disease. We’re also progressing toward our goal of dosing 30 patients cumulatively across all clinical programs by the end of 2021. We look forward to providing additional updates as we advance our leading pipeline of lentiviral gene therapies that target the multi-billion-dollar lysosomal disorder market,” added MacKay.

Program Updates and Milestones

Received orphan drug designation (ODD) from the European Commission (EC) for AVR-RD-04 for cystinosis in March 2021

- Received ODD from the EC for AVR-RD-01 for Fabry disease in November 2020.
- All three AVROBIO investigational gene therapies in the clinic have received orphan designation from FDA and EC.

Provided robust data updates in 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 Phase 1 and Phase 2.
- For the first two patients dosed in the investigator-sponsored Phase 1/2 trial in cystinosis, showed improvement across multiple measures including 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 enzyme replacement therapy (ERT).
- Additional details on the data presented during the 17th annual WORLD*Symposium* can be found here.

Expanded management team with appointment of Diana M. Escolar, M.D., FAAN, chief medical officer, bringing expertise and experience in rare diseases, with a focus on lysosomal and neurogenetic disorders, in January 2021

Provided updates on ongoing clinical trials and second wave of programs in leading lysosomal disorder gene therapy pipeline at first Virtual R&D Day in November 2020

- Shared positive data out as far as 3.5 years across a broad lysosomal disorder gene therapy pipeline.

- Presented data on Bu90-target concentration intervention (TCI), the company's pioneering approach to personalized conditioning.
- Announced development plans for AVR-RD-06 in Gaucher disease type 3.
- Presented preclinical data from AVR-RD-05 for Hunter syndrome demonstrating normalization of multiple disease measures in a mouse model, including normalization of skeletal features such as the thickness of cheekbone and femur.
- Announced preclinical data for AVR-RD-03 for Pompe disease that showed the normalization of substrate levels in multiple hard-to-reach organs including brain and spinal cord.
- Additional details on the data presented at the Virtual R&D Day can be found here.

Strengthened balance sheet and extended anticipated cash runway into Q1 2023

- Raised gross proceeds of $75 million through a follow-on common stock offering in November 2020.
- Based on the company's current operating plan, AVROBIO expects its cash and cash equivalents as of Dec. 31, 2020, will enable the company to fund its operating expenses and capital expenditure requirements into Q1 2023.

Fourth Quarter and Year End 2020 Financial Results

AVROBIO reported a net loss of $28.1 million for the fourth quarter of 2020, and a net loss of $119.7 million for the year ended 2020, as compared to a net loss of $22.7 million and a net loss of $73.0 million for the comparable periods in 2019, respectively. These increases were due to increased research and development expenses, as well as increased general and administrative expenses.

Research and development expenses were $19.6 million for the fourth quarter of 2020, and $87.2 million for the year ended 2020, as compared to $17.2 million and $55.0 million for the comparable periods in 2019, respectively. These increases were driven by increased program development activities related to the advancement of the company's pipeline, including an $8.0 million expense incurred during the third quarter of 2020 related to a one-time, upfront fee we paid as consideration for in-licensing the Hunter syndrome program, 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 $8.5 million for the fourth quarter of 2020, and $33.0 million for the year ended 2020, as compared to $6.2 million and $20.8 million for the comparable periods in 2019, respectively. These increases were primarily due to an increase in employee headcount, which includes the impact of non-cash stock-based compensation, as well as professional fees and consulting costs.

As of Dec. 31, 2020, AVROBIO had $259.7 million in cash and cash equivalents, as compared to $187.0 million in cash and cash equivalents as of Dec. 31, 2019. This increase was primarily the result of the completion of the company's follow-on offerings completed in February 2020 and November 2020 that generated total net proceeds of $163.8 million. Based on the company's current operating plan, AVROBIO expects its cash and cash equivalents as of Dec. 31, 2020 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 current and prospective product candidates, 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, and product approvals, 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, including the use of a personalized and ultra-precision busulfan conditioning regimen, 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 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.

Investor Contact:
Christopher F. Brinzey
Westwicke, an ICR Company
339-970-2843
chris.brinzey@westwicke.com

Media Contact:
Stephanie Simon
Ten Bridge Communications
617-581-9333
stephanie@tenbridgecommunications.com

CONDENSED CONSOLIDATED BALANCE SHEETS
(In thousands)
(Unaudited)

	December 31, 2020	December 31, 2019
Cash and cash equivalents	$ 259,682	$ 187,043
Prepaid expenses and other current assets	7,560	8,658
Property and equipment, net	3,064	3,696
Other assets	928	1,117
Total assets	$ 271,234	$ 200,514
Accounts payable	$ 2,682	$ 3,949
Accrued expenses and other current liabilities	13,932	10,068
Deferred rent, net of current portion	276	484
Total liabilities	16,890	14,501
Total stockholders' equity	254,344	186,013
Total liabilities and stockholders' equity	$ 271,234	$ 200,514

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

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2020	2019	2020	2019
Operating expenses:				
Research and development	$ 19,587	$ 17,219	$ 87,236	$ 54,974
General and administrative	8,477	6,214	32,992	20,835
Total operating expenses	28,064	23,433	120,228	75,809
Loss from operations	(28,064)	(23,433)	(120,228)	(75,809)
Total other income (expense), net	(67)	771	516	2,844
Net loss	$(28,131)	$(22,662)	$(119,712)	$(72,965)
Net loss per share—basic and diluted	$ (0.73)	$ (0.72)	$ (3.31)	$ (2.66)
Weighted-average number of common shares outstanding—basic and diluted	38,528	31,629	36,206	27,432