# 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 16, 2020**

# 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 16, 2020, 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, 2019. 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 16, 2020.

**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 16, 2020

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

**Exhibit 99.1**

**AVROBIO Reports Fourth Quarter and Fiscal Year 2019 Financial Results and Provides Business Update**

*Presented positive data at the 16th Annual WORLDSymposium™ on AVR-RD-04 for cystinosis and AVR-RD-01 for Fabry disease, as well as early data on plato™-produced drug product and plasma enzyme activity level*

*Received orphan drug designation from U.S. Food and Drug Administration for AVR-RD-04; third AVROBIO investigational gene therapy to receive orphan status*

*Raised $100 million in gross proceeds from follow-on common stock offering in February 2020, extending cash runway into Q2 2022*

CAMBRIDGE, Mass., March 16, 2020 — 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, 2019 and provided a business update.

"For AVROBIO, 2019 was an important year, with positive data on a primary clinical endpoint from the first patient in our Fabry Phase 2 trial and the unveiling of our state-of-the-art gene therapy platform, plato™," said Geoff MacKay, AVROBIO's president and CEO. "This momentum has put us in a strong position for 2020. Our presence at WORLD*Symposium*™ in February included sharing supportive initial data for cystinosis and plato-produced drug product, as well as further data supporting efficacy and durability in Fabry disease, with our first Phase 1 patient now out to 32 months. We believe 2020 will be our breakout year, with important updates across our pipeline of investigational single-dose treatments for lysosomal disorders as well as our early-stage portfolio."

**Program Updates and Milestones**

*AVR-RD-01 in Fabry disease: Evidence of durability and tolerability across both Phase 1 and Phase 2 trials*

AVROBIO has two investigational gene therapy clinical trials for Fabry disease. Four patients have been dosed in the Phase 2 trial (FAB-201) and five patients have been dosed in the Phase 1 investigator-led trial of AVR-RD-01. Across both studies:

- For the first patient in the Phase 1 trial, data continue to demonstrate vector copy number (VCN) stability out to 32 months following treatment, supportive of long-term engraftment. The VCN data profiles were generally consistent across the seven other Phase 1 and Phase 2 trial participants out six to 24 months. The eighth participant has not yet reached the six-month post-treatment date.

- As of the safety data cut-off date of Nov. 26, 2019, there have been no safety events attributed to AVR-RD-01 drug product in either the Phase 1 or Phase 2 trial. Through the safety data cut-off date, two serious adverse events (SAEs) have been reported in the Phase 1 trial and four SAEs have been reported in the Phase 2 trial. The fourth Phase 2 patient, who was dosed after the safety data cut-off date, has reported an SAE, which was not attributed to AVR-RD-01 and subsequently resolved. Across both studies, each of the SAEs has been consistent with the underlying disease or pre-existing conditions, stem cell mobilization or conditioning regimen. Adverse events (AEs) reported did not suggest any unexpected safety signals or trends.

*AVR-RD-01 Phase 1 trial in Fabry disease: Interim data continue to support potential first-line use*

- Four of five patients had plasma lyso-Gb3 levels reduced between 26 and 47 percent compared to baseline levels measured while on enzyme replacement therapy (ERT). The fifth patient's plasma lyso-Gb3 level remains within the range for the Fabry disease patients on ERT observed in this study and he remains off ERT.
- Overall, three of the five Phase 1 patients have discontinued ERT and all three remain off ERT at six, 14 and 15 months after dosing.

*AVR-RD-01 Phase 2 trial in Fabry disease: Interim data support potential first line use*

- Data show increased leukocyte and plasma enzyme activity in the first three Phase 2 patients, suggesting production of an endogenous supply of functional alpha-galactosidase (AGA) enzyme sustained at nine, 12 and 18 months, respectively, after patient dosing.
- Two of these three patients have also demonstrated associated decreased plasma lyso-Gb3 levels, a key biomarker for monitoring Fabry disease, sustained below their baseline at six and 18 months, respectively, after dosing. As expected, the third Phase 2 patient, a cardiac variant who does not have classic Fabry disease and has very low baseline levels of lyso-Gb3, did not show a substantial decrease in plasma lyso-Gb3 levels.
- Cardiac and kidney function measures in the Phase 2 trial remained within the normal range for patients who had available 12-month data.

*AVR-RD-04 Phase 1/2 trial in cystinosis: Three-month data from first patient suggest positive trends across multiple measures*

- No safety events or SAEs attributed to the investigational drug product were reported as of the Jan. 27, 2020 safety data cut-off date. AEs reported did not suggest any unexpected safety signals or trends and was consistent with the conditioning regimen and the underlying disease.
- Three months following administration of the investigational gene therapy, the first patient had a peripheral blood VCN of 2.0.
- Average granulocyte cystine level – one of the trial's primary endpoints – decreased from 7.8 nmol half cystine/mg protein two weeks after cysteamine discontinuation to 1.5 nmol half cystine/mg protein at three months post-gene therapy.
- The U.S. Food and Drug Administration (FDA) granted orphan drug designation for AVR-RD-04 in March 2020.

*AVR-RD-02 Phase 1/2 trial in Gaucher disease: Expect to dose first patient in Q2 2020*

- Enrolled first patient.
- Received notice of clearance from the FDA regarding Investigational New Drug (IND) application for AVR-RD-02 for the treatment of Gaucher disease in January 2020.
- Recruiting participants in Australia and Canada, with additional U.S. sites planned.

*plato gene therapy platform debut: Presented one-month data for fourth patient in Phase 2 Fabry trial (FAB-201)*

- Shared preliminary data from the first drug product produced using plato.
    - Enzyme activity and transduction efficiency were 2.2 times higher than the mean of these measures for the drug product for the first three patients dosed in FAB-201.
    - VCN was 1.8 times higher than the mean of this measure for the drug product for the first three patients dosed in FAB-201.
- At one month following administration of the plato-produced investigational gene therapy, plasma AGA enzyme activity level was 4.0 times higher for the first patient dosed using the plato platform in the Company's FAB-201 clinical trial than the mean plasma enzyme activity level of the first three patients in the same trial at the same timepoint.

*Strengthened balance sheet and extended anticipated cash runway into Q2 2022*

- In February 2020, the company raised gross proceeds of $100 million through a follow-on common stock offering.

- Based on the company's current operating plan, AVROBIO expects its cash and cash equivalents as of Dec. 31, 2019, together with the net proceeds from the February 2020 follow-on common stock offering, will enable the company to fund its operating expenses and capital expenditure requirements into Q2 2022.

**Fourth Quarter and Fiscal Year 2019 Financial Results**

AVROBIO reported a net loss of $22.7 million for the fourth quarter of 2019, and a net loss of $73.0 million for the year ended 2019, as compared to a net loss of $16.0 million and a net loss of $46.4 million for the comparable periods in 2018, respectively. These increases were due to increased research and development expenses, as well as increased general and administrative expenses.

Research and development expenses were $17.2 million for the fourth quarter of 2019, and $55.0 million for the year ended 2019, as compared to $12.8 million and $35.1 million for the comparable periods in 2018, respectively. These increases were 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. Also, during the fourth quarter of 2019, the company made a milestone payment of $2.0 million to GenStem Therapeutics, Inc., in connection with the dosing of the first patient in the Phase 1/2 clinical trial of AVR-RD-04 in cystinosis in the United States.

General and administrative expenses were $6.2 million for the fourth quarter of 2019, and $20.8 million for the year ended 2019, as compared to $3.9 million and $11.1 million for the comparable periods in 2018, respectively. These increases were primarily due to an increase in employee headcount, expenses associated with being a publicly traded company and the impact of non-cash stock-based compensation.

As of Dec. 31, 2019, AVROBIO had $187.0 million in cash and cash equivalents, as compared to $126.3 million in cash and cash equivalents as of Dec. 31, 2018. This increase was primarily the result of the completion of the company's follow-on common stock offering completed in July 2019, which raised net proceeds of $129.5 million. Based on the company's current operating plan, AVROBIO expects its cash and cash equivalents as of Dec. 31, 2019, together with the net proceeds from the February 2020 follow-on common stock offering, will enable the company to fund its operating expenses and capital expenditure requirements into Q2 2022.

**About AVROBIO**

Our mission is to free people from a lifetime of genetic disease with a single dose of gene therapy. We aim to halt or reverse disease throughout the body by driving durable expression of functional protein, even in hard-to-reach tissues and organs including the brain, muscle and bone. Our clinical-stage programs include Fabry disease, Gaucher disease and cystinosis and we also are advancing a program in Pompe disease. AVROBIO is powered by the plato™ gene therapy platform, our foundation designed to scale 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 prospective product candidates, the design, commencement, enrollment and timing of ongoing or planned clinical trials, clinical trial results, product approvals and regulatory pathways, 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 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 business interruptions resulting from the coronavirus disease (COVID-19) outbreak or similar public health 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, 2019 | December 31, 2018 |
|---|---|---|
| Cash and cash equivalents | $ 187,043 | $ 126,302 |
| Prepaid expenses and other current assets | 8,658 | 3,718 |
| Property and equipment, net | 3,696 | 2,634 |
| Other assets | 1,117 | 825 |
| Total assets | $ 200,514 | $ 133,479 |
| Accounts payable | $ 3,949 | $ 2,784 |
| Accrued expenses and other current liabilities | 10,068 | 7,822 |
| Deferred rent, net of current portion | 484 | 689 |
| Total liabilities | 14,501 | 11,295 |
| Total stockholders' equity | 186,013 | 122,184 |
| Total liabilities and stockholders' equity | $ 200,514 | $ 133,479 |

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

| | Three Months Ended December 31, | | Twelve Months Ended December 31, | |
|---|---|---|---|---|
| | 2019 | 2018 | 2019 | 2018 |
| Operating expenses: | | | | |
| Research and development | $ 17,219 | $ 12,809 | $ 54,974 | $ 35,095 |
| General and administrative | 6,214 | 3,867 | 20,835 | 11,148 |
| Total operating expenses | 23,433 | 16,676 | 75,809 | 46,243 |
| Loss from operations | (23,433) | (16,676) | (75,809) | (46,243) |
| Total other income (expense), net | 771 | 655 | 2,844 | (118) |
| Net loss | $ (22,662) | $ (16,021) | $ (72,965) | $ (46,361) |
| Reconciliation of net loss to net loss attributed to common stockholders: | | | | |
| Net loss | $ (22,662) | $ (16,021) | $ (72,965) | $ (46,361) |
| Accretion of issuance costs on redeemable convertible preferred stock | | — | — | (2,243) |
| Net loss attributable to common stockholders — basic and diluted | $ (22,662) | $ (16,021) | $ (72,965) | $ (48,604) |
| Net loss per share attributable to common stockholders — basic and diluted | $ (0.72) | $ (0.67) | $ (2.66) | $ (3.62) |
| Weighted-average number of common shares used in computing net loss per share attributable to common stockholders — basic and diluted | 31,628,930 | 23,791,495 | 27,432,489 | 13,435,478 |