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): October 5, 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 8.01 Other Events.

Press Release

On October 5, 2020, AVROBIO, Inc. (the "Company") issued a press release announcing that it has entered into an agreement (the "License") with The University of Manchester, England ("UoM"), whereby UoM granted to the Company an exclusive worldwide license under certain patent and other intellectual property rights, subject to certain retained rights, to develop, commercialize and sell an *ex vivo* lentiviral gene therapy for use in the treatment of Hunter syndrome, or mucopolysaccharidosis type II (MPSII).

As consideration for the License, the Company has agreed to pay UoM an upfront, one-time fee of $8.0 million. The Company is also required to make milestone payments up to an aggregate of $80.0 million upon the achievement of specified development and regulatory milestones, to pay royalties, on a product-by-product and country-by-country basis, of a mid-single digit percentage based on net sales of products licensed under the License, and to pay a low double-digit percentage of any sublicense fees received by the Company.

Unless terminated earlier, the License expires upon the later of 15 years from the effective date or the expiration of the last valid claim of the licensed patents, subject to certain surviving rights and obligations. UoM and the Company can each terminate the agreement in the event of the bankruptcy or insolvency of the other party, or a material breach by the other party and failure to cure such breach within a certain period of time. UoM has the right to terminate the agreement in the event of certain actions relating to challenge or opposition to the licensed intellectual property brought by the Company or its affiliates or sublicensees.

Concurrently with the License, the Company entered into a collaborative research funding agreement with UoM (the "CRFA"). Under the CRFA, the Company has agreed to fund the budgeted costs of an investigator-sponsored Phase 1/2 clinical trial to be sponsored by UoM in connection with the development activities under the License, which are currently estimated to equal approximately £9.1 million in the aggregate.

A copy of the press release is filed as Exhibit 99.1 to this Current Report on Form 8-K and incorporated into this Item 8.01 by reference.

Financial Position

The Company continues to expect its existing cash and cash equivalents will enable the Company to fund its operating expenses and capital expenditure requirements into the second half of 2022, which is based on the Company's current operating plan and takes into account the upfront payment due under the License and the budgeted costs under the CRFA that are expected to become due during such period.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits

99.1	Press release issued by AVROBIO, Inc., dated October 5, 2020.
104	The cover page from this Current Report on Form 8-K, formatted in Inline XBRL

Forward Looking Statements

This Current Report on Form 8-K and certain of the materials furnished or filed herewith 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 the Company's financial position and cash runway guidance, as well as statements regarding the expected costs of the investigator-sponsored Phase 1/2 clinical trial for Hunter syndrome/MPSII. Any such statements that are not statements of historical fact may be deemed to be forward-looking statements.

Any forward-looking statements are based on the Company's current expectations, estimates and projections only as of the date of this release 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, risks regarding the accuracy of the Company's estimates of expenses and future revenue, risks relating to the Company's capital requirements and needs for additional financing, as well as risks that the costs of the investigator-sponsored Phase 1/2 clinical trial for Hunter syndrome/MPSII may exceed the Company's estimates. For a discussion of these and other risks and uncertainties, and other important factors, any of which could cause the Company's actual results to differ materially and adversely from those contained in the forward-looking statements, see the section entitled "Risk Factors" in the Company's Quarterly Report on Form 10-Q for the quarter ended June 30, 2020, as well as discussions of potential risks, uncertainties, and other important factors in the Company's other filings with the Securities and Exchange Commission. The Company explicitly disclaims any obligation to update any forward-looking statements except to the extent required by law.

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: October 5, 2020

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

Exhibit 99.1

AVROBIO Expands Lentiviral Gene Therapy Pipeline with Program for Hunter Syndrome

Investigational gene therapy licensed from The University of Manchester, UK aims to address both physical and CNS manifestations of Hunter syndrome

Expands leading pipeline targeting lysosomal disorders with 5th investigational gene therapy

Program expected to enter Phase 1/2 clinical trial in the second half of 2021

CAMBRIDGE, Mass., Oct. 5, 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 announced an exclusive, worldwide license agreement and a collaborative research funding agreement with The University of Manchester for an investigational lentiviral gene therapy for mucopolysaccharidosis type II (MPS II), or Hunter syndrome, a rare and deadly lysosomal disorder that primarily affects young boys.

Hunter syndrome, which affects an estimated one in 100,000 to one in 170,000 males worldwide, causes devastating complications throughout the body and brain, including severe cardiac and respiratory dysfunction, skeletal malformations and hearing impairment. Children with severe cases of Hunter syndrome typically show early symptoms in their toddler years and begin to regress developmentally around age six, losing basic motor skills and cognitive function. The current standard of care is weekly enzyme replacement therapy (ERT), which can delay some complications but does not halt overall progression of the disease and has not been demonstrated to address cognitive issues. Even with ERT, people with Hunter syndrome face life-limiting symptoms and a significantly reduced life span.

“We believe a lentiviral gene therapy approach is well suited to treat a progressive and pervasive disease such as Hunter syndrome, which affects organs throughout the body and severely impairs cognitive function. If we treat children early, before their symptoms arise, we hope to prevent the tragic complications that rob these young children of their futures,” said Geoff MacKay, AVROBIO’s president and CEO. “We believe our deep experience with investigational gene therapies for lysosomal disorders will enable us to efficiently move the program through clinical development in collaboration with Prof. Brian Bigger, who has done tremendous work to develop and optimize this investigational gene therapy. We’re proud to add this program to our leading lysosomal disorder pipeline and excited about its potential to change the lives of patients and families living with Hunter syndrome.”

The investigator-sponsored Phase 1/2 clinical trial for Hunter syndrome is expected to enter the clinic in the second half of 2021. The program was developed by Brian Bigger, Ph.D., a professor of cell and gene therapy at the University of Manchester, U.K. Prof. Bigger has published preclinical data demonstrating that the introduction of the transgene with an optimized, proprietary tag has the potential to correct peripheral disease and normalize brain pathology. Primary investigators for the Phase 1/2 clinical trial will be Prof. Robert Wynn, M.D., consultant pediatric hematologist at the Royal Manchester Children's Hospital, and Dr. Simon Jones, MBChB, consultant pediatric physician for inherited metabolic diseases at the Willink Unit, Saint Mary's Hospital and the Manchester Centre for Genomic Medicine.

"We feel an enormous urgency to bring forward a treatment that may halt this deadly disease in its tracks, before symptoms emerge and before children lose their physical and cognitive skills," said Prof. Bigger. "We are delighted to be working with AVROBIO on this program. Both of our teams have deep experience running international clinical trials in other lysosomal disorders. AVROBIO also has a leading gene therapy platform, plato®, which is designed to optimize the consistency, predictability and efficacy of its gene therapies and to enable efficient scaling for worldwide commercialization. Both these factors make AVROBIO an excellent partner for our investigational Hunter syndrome gene therapy. By working together, we believe we can greatly accelerate development of this important program."

The investigational gene therapy, which will be called AVR-RD-05, involves ex vivo transduction of the patient's own hematopoietic stem cells with a therapeutic transgene designed to express functional enzyme the patient needs to maintain cellular health, coupled to a proprietary protein tag that is designed to improve stability of the enzyme in the bloodstream and facilitate uptake by tissues from head to toe. When reinfused into the patient, the gene-modified stem cells are expected to engraft in the bone marrow and produce generations of daughter cells, each carrying the transgene. Those daughter cells are then expected to differentiate into macrophages, microglia and other components of the immune system and circulate throughout the body and central nervous system, potentially enabling widespread distribution of functional enzyme.

AVROBIO's other investigational gene therapies for lysosomal disorders are being evaluated in a Phase 1 and Phase 2 clinical trial for Fabry disease, a Phase 1/2 trial for cystinosis and a Phase 1/2 trial for Gaucher disease. In addition, the company is advancing a preclinical program in Pompe disease. Further details on the new Hunter syndrome program will be discussed at AVROBIO's upcoming virtual R&D Day on Tuesday, Nov. 17, 2020.

Financial terms of the agreement

The University of Manchester's technology transfer office, the University of Manchester Innovation Factory and AVROBIO have negotiated an exclusive, worldwide license agreement to the technology and a collaborative research funding agreement. Under the license agreement, AVROBIO will pay The University of Manchester an upfront cash payment and additional payments based on development and regulatory milestones. The company will also pay The University of Manchester a mid-single digit percentage royalty on annual net sales of licensed products. Additionally, under the collaborative research funding agreement, AVROBIO will cover budgeted Phase 1/2 clinical trial costs.

About Hunter syndrome

Hunter syndrome, also known as mucopolysaccharidosis type II (MPS II), is a lysosomal disorder caused by a mutation in the *IDS* gene that leads to a deficiency of the lysosomal enzyme iduronate-2-sulfatase (IDS), which is essential for breaking down large sugar molecules called glycosaminoglycans (GAGs, also known as mucopolysaccharides). Without functional IDS, toxic levels of GAGs build up throughout the body and central nervous system, causing a wide range of symptoms including cognitive decline and cardiac and respiratory dysfunction. The current standard of care is weekly enzyme replacement therapy, which may delay some symptoms but does not halt the overall progression of disease and does not cross the blood-brain barrier, an intricate web of protective tissue that selectively prevents macromolecules from entering the brain. Even with treatment, people with Hunter syndrome face life-limiting symptoms and a significantly reduced life span. The disorder affects an estimated one in 100,000 to one in 170,000 males worldwide; about two-thirds of cases have an early, severe progressive form.

About AVROBIO's personalized gene therapy approach

Our investigational lentiviral gene therapies start with the patient's own hematopoietic stem cells. We use a lentiviral vector to transduce those cells in order to insert a therapeutic gene designed to enable the patient to produce a supply of the functional protein they lack. These cells are then infused back into the patient, where they are expected to engraft in the bone marrow and produce generations of daughter cells, each containing a copy or copies of the therapeutic gene. To optimize engraftment, we use a personalized conditioning regimen with precision dosing of busulfan to make space and enable durable engraftment in the patient's bone marrow and central nervous system (CNS). Busulfan is an established conditioning agent that has been administered to hundreds of patients treated with lentiviral gene therapies. Our approach is designed to drive durable production of the functional protein throughout the patient's body, thereby potentially addressing symptoms from "head to toe," including those originating in the CNS.

About lentiviral gene therapy

Lentiviral vectors are differentiated from other delivery mechanisms because of their large cargo capacity and their ability to integrate the therapeutic gene directly into the patient's chromosomes. This integration is designed to maintain the therapeutic gene's presence as the patient's cells divide, which potentially enables dosing of pediatric patients, whose cells divide rapidly as they grow. Because the therapeutic gene is integrated using the vector into patients' own stem cells, patients are not excluded from receiving the investigational therapy due to pre-existing antibodies to the viral vector.

About AVROBIO

Our vision is to bring personalized gene therapy to the world. We aim to halt, reverse or prevent disease throughout the body with a single dose of gene therapy designed to drive 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 preclinical programs in Hunter syndrome and 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 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 prospective product candidates, including our new program for Hunter syndrome, the design, commencement, enrollment and timing of ongoing or planned clinical trials, including the planned investigator-sponsored Phase 1/2 clinical trial for Hunter syndrome, 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, and 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. 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, including AVR-RD-05 for Hunter syndrome, 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 development timeline 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 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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