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AVROBIO Receives Orphan Drug Designation from U.S. FDA for AVR-RD-04 for Cystinosis

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AVR-RD-04 is the third AVROBIO investigational gene therapy to receive orphan status

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- 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 that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to the company's investigational gene therapy, AVR-RD-04, for the treatment of cystinosis. The gene therapy consists of the patient's own hematopoietic stem cells, which are genetically modified to express cystinosin, the protein that is functionally deficient in people with cystinosis. A Phase 1/2 clinical trial to evaluate the safety and efficacy of the investigational gene therapy in patients with cystinosis is ongoing, sponsored by AVROBIO's academic collaborator at the University of California, San Diego (UCSD).

"People living with cystinosis need new treatment options to keep cystine from accumulating in the lysosomes of cells, which leads to corneal damage and kidney deterioration, among other complications. Although the current standard of care has improved the outlook for this community, it does not halt disease progression or a wide range of debilitating complications which can severely impact daily lives," said Geoff MacKay, CEO and president at AVROBIO. "We believe lentiviral gene therapy is potentially well suited to comprehensively address these symptoms, since it is designed to restore functional cystinosin throughout the body and brain."

Orphan drug designation is granted by FDA to drugs and biologics which are intended for the safe and effective treatment, diagnosis or prevention of rare diseases or conditions that affect fewer than 200,000 people in the U.S. Orphan drug designation provides certain incentives which may include tax credits towards the cost of clinical trials and prescription drug user fee waivers.

About Cystinosis

Cystinosis is a rare, progressive disease marked by the accumulation of cystine in cellular organelles known as lysosomes, causing debilitating symptoms including eye complications (such as severe photophobia), muscle wasting and kidney failure, which often lead to a shortened lifespan. More than 90 percent of untreated patients require a kidney transplant before age 20. The current standard of care for cystinosis, a burdensome treatment regimen that causes severe halitosis and can amount to dozens of pills a day, does not halt disease progression.

About AVR-RD-04

AVR-RD-04 is an investigational, lentiviral-based gene therapy designed to potentially halt or reverse the progression of cystinosis with a single dose of the patient's own hematopoietic stem cells. Before being transplanted into the patient, the stem cells are collected and genetically modified to express functional cystinosin, a transport protein which reduces the cystine build-up in the lysosomes of cells that cause the symptoms of cystinosis.

The open-label, single-arm Phase 1/2 clinical trial evaluating the safety and efficacy of AVR-RD-04 is being conducted under the name CTNS-RD-04 by the company's academic collaborators at UCSD, and is led by Stephanie Cherqui, Ph.D., associate professor of pediatrics at UCSD's School of Medicine and a consultant to the company. The trial is actively enrolling up to six participants, and is funded by grants to UCSD from the California Institute for Regenerative Medicine (CIRM) as well as the Cystinosis Research Foundation (CRF).

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 the development and continued progress of our programs, including the intended incentives conferred by orphan drug designation, the therapeutic potential of our product candidates, the design, commencement, enrollment and timing of ongoing or planned clinical trials, including the ongoing Phase 1/2 trial of the AVR-RD-04 investigational gene therapy, the anticipated benefits of our gene therapy platform, the expected safety profile of our product candidates, timing and likelihood of success of our current or future product candidates, and the market opportunity for our product candidates. 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, 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 on Form 10-Q, 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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