AVROBIO, Inc. Announces $60 Million Series B Financing to Advance Gene Therapy Pipeline for Lysosomal Storage Disorders and Apply Lentiviral Platform to Other Genetic Diseases

Company to initiate Phase 2 trial with lead gene therapy, AVR-RD-01, based on promising initial clinical data in Fabry disease

Strong investor syndicate supports multi-product portfolio with three additional gene therapies for Gaucher disease, cystinosis and Pompe disease

Cambridge, MA, February 1, 2018 – AVROBIO, Inc., **a clinical-stage biotechnology company developing transformative, life-changing gene therapies for rare diseases, announced today that it has completed a $60 million Series B financing. Proceeds from the financing will be used to advance multiple gene therapies from** AVROBIO's proprietary lentiviral platform**, including the company's lead gene therapy, AVR-RD-01, currently in Phase 1 for Fabry disease, as well as three additional gene therapies for other lysosomal storage disorders, Gaucher disease, cystinosis and Pompe disease. The Series B round was co-led by Cormorant Asset Management and Surveyor Capital (a Citadel company), and included participation by Aisling, Brace Pharma Capital, Eventide Asset Management and Morningside, along with existing investors Atlas Venture, SV Health Investors and Clarus Ventures.**

In advancing the company's gene therapy pipeline, AVROBIO plans to initiate a Phase 2 clinical trial this year with AVR-RD-01 in Fabry disease, based on promising initial six-month clinical results in the ongoing Phase 1 trial**. In the first patient with Fabry disease in the Phase 1 trial, treatment with a single dose of AVR-RD-01 resulted in normal plasma activity of α-galactosidase A enzyme, the lysosomal enzyme genetically deficient in patients with Fabry disease. In addition, the company plans to initiate clinical development of two other gene therapy candidates for cystinosis and Gaucher disease by mid-2019.**

"Gene therapy is a class of medicines that has made breakthroughs in the biotechnology landscape, and AVROBIO has carved out a strong position with its lentiviral platform focused on the significant therapeutic and market opportunities for gene therapies for lysosomal storage diseases," said Bruce Booth, D.Phil.**, Partner at Atlas Venture and Chairman of AVROBIO's Board of Directors. "AVROBIO has made remarkable progress with its gene therapy platform, including a very compelling initial clinical response in the first patient with Fabry disease to receive the company's lead gene therapy candidate. We have great confidence in the high caliber management team, and the rigorous science and clinical programs behind the gene therapies for Fabry disease and a range of other lysosomal storage disorders."**

With AVR-RD-01 and its pipeline of gene therapy candidates, AVROBIO is positioned to be the first company to advance into the clinic with gene therapies for lysosomal storage disorders, a class of rare genetic diseases in which patients have a defective gene that produces an enzyme or protein that serves a vital metabolic function. The company has initially applied its proprietary lentiviral gene therapy platform to lysosomal storage disorders, and will also apply the platform to a broad range of other diseases where systemic delivery of gene therapy may be therapeutically beneficial. The lentiviral vector technology is a gene transfer system for stable addition of genes into the patient's

own cells, designed to offer permanent genomic integration for a durable and potentially life-long curative benefit for patients.

"We are delighted by this exceptional group of investors supporting our plans to build AVROBIO as a leader in the field of lentiviral gene therapy," said Geoff MacKay, President and CEO of AVROBIO. "We are excited to continue to rapidly advance our clinical program for AVR-RD-01 in Fabry disease, while also moving two additional gene therapies from our pipeline into clinical trials. We are on the leading edge of applying gene therapy as a potential single-dose curative treatment for lysosomal storage disorders, and our vision is to use our lentiviral platform to have broader impact for patients by shifting the paradigm for a wide range of other diseases."

Leerink Partners acted as exclusive financial advisor for AVROBIO's Series B financing.

About AVR-RD-01 and Initial Clinical Results
AVR-RD-01 is a lentiviral gene therapy being investigated as a single-dose therapy with durable and potentially life-long curative benefit for patients with Fabry disease. AVR-RD-01 uses a state-of-the-art lentiviral vector system that is an efficient and proven gene transfer system for the stable addition of genes into the patient's stem cells. In patients with Fabry disease, their CD34+ stem cells are extracted and isolated, and then the cells are transduced with lentiviral vector carrying a normal GLA gene to create AVR-RD-01 gene therapy. AVR-RD-01 is then infused back into the patient on an out-patient basis, with the goal of restoring normal GLA gene expression so that α-galactosidase A enzyme is produced by the patient's own body.

In October 2017, AVROBIO presented initial six-month clinical data from the first patient with Fabry disease treated with AVR-RD-01. This administration of a single dose of AVR-RD-01 enabled the patient to achieve and, at six months, maintain normal plasma activity of α-galactosidase A, the lysosomal enzyme genetically deficient in patients with Fabry disease.

About AVROBIO's Lentiviral Gene Therapy Platform
AVROBIO has designed a state-of-the-art, 3rd generation, 4 plasmid lentiviral vector platform that is an efficient and proven gene transfer system for the stable addition of genes into the patient's own CD34+ stem cells. The lentiviral vector technology is designed to offer permanent genomic integration and can be applied to a range of diseases where systemic delivery may be therapeutically beneficial. AVROBIO's platform includes patented technology, unique manufacturing processes, gene therapy know-how and proprietary tools.

About AVROBIO, Inc.
AVROBIO, Inc., a leader in lentiviral-based gene therapies, is a clinical-stage company developing disruptive therapies that have the potential to transform patients' lives with a single dose. The Company is focused on the development of its gene therapy candidate, AVR-RD-01, in Fabry disease, as well as additional gene therapy programs in other lysosomal storage disorders including Gaucher disease, cystinosis and Pompe disease. AVROBIO's lentiviral platform has broad potential for other rare and non-rare genetic diseases. AVROBIO is headquartered in Cambridge, MA and has offices in Toronto, ON. For additional information, visit www.avrobio.com.

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