AVROBIO Inc. Launches to Develop Novel, Clinical-Stage Cell and Gene Therapies Targeting Immuno-Oncology and Rare Diseases

- *Disruptive Therapies with Potential to Displace Standard of Care Treatments for Acute Myeloid Leukemia (AML) and Fabry Disease*
- *Announces Management Team of Industry Leaders with Unmatched Scientific, Business and Manufacturing Expertise in Cell and Gene Therapies and Rare Diseases*
- *Licenses Innovative Cell and Gene Therapy Technologies from University Health Network (UHN) of Toronto, ON*
- *Secures Seed Financing from Atlas Venture of Cambridge, MA*

February 18, 2016 07:00 AM Eastern Standard Time

CAMBRIDGE, Mass.--(BUSINESS WIRE)--AVROBIO, a clinical-stage biotechnology company developing transformative cell and gene therapies targeting cancer and rare diseases, today announced its launch plans. The company's priority is to accelerate development of two novel cell and gene therapies pioneered within the labs of Dr. Christopher Paige and Dr. Jeffrey Medin (now at the Medical College of Wisconsin) at the University Health Network (UHN) in Toronto, ON. Phase 1 programs will be in the clinic by early to mid-2016 in both acute myeloid leukemia (AML) and Fabry disease. The company will simultaneously work to expand its proprietary cell and gene therapy platform to treat additional indications.

"AVROBIO's highly innovative therapies offer potentially life-altering impact for patients following a single infusion of genetically-modified cells," said Geoff MacKay, AVROBIO's President and Chief Executive Officer. "We are very proud to carry forward the groundbreaking work of our founding scientists with investment from Atlas Venture and partnership with the Center for Commercialization of Regenerative Medicine (CCRM). We have built a team capable of accelerating the development of truly important therapies for patients in immense need."

Joining Mr. MacKay on the leadership team are Dr. Kim Warren, Head of Operations, Dr. Chris Mason, Chief Science Officer, and Deanna Petersen, Chief Business Officer. All are recognized veterans of the cell and gene therapy field and bring proven leadership in rare diseases.

“Following extensive research and pre-clinical development, we are happy to have our technology reach clinical development and are excited to work with the AVROBIO team to continue translating our science into proven medicine. Lentiviral gene therapy approaches have been demonstrated in clinical trials to be a safe and effective vehicle for the delivery of therapeutic genes into targeted cells. A broader goal is to leverage our proprietary ex-vivo gene therapy backbone across a number of serious diseases thereby accelerating the development of potential breakthrough therapies,” said Dr. Jeffrey Medin.

Cell and gene therapies represent a new paradigm in human health, with the potential to deliver dramatic disease-modifying effects with long-lasting, durable impact. Underlying these advances are a deeper understanding of cell biology, immunology and a newer generation of vector designs enabling safe and effective delivery of therapeutic genes targeted to specific cells. AVROBIO’s initial two programs are leveraging the established safety and effectiveness of ex-vivo gene therapy to provide AML and Fabry patients with new therapies that will potentially significantly improve both their quality of life and lifespan.

“This deal validates our vision that Toronto will be a world-class center for the commercialization of cell-based technology developed at UHN,” said John Reid, Director of the team at UHN’s office of Technology Development and Commercialization, which negotiated the deal on behalf of UHN.

AVR-01 is designed to be a potent anti-cancer immunotherapy which triggers the immune system to first detect, and then eradicate, tumor cells. The patient’s cancer cells are genetically modified to express one of the most powerful immune signaling agents, the cytokine IL-12. The modified cells are then infused back into the patient via a one-time procedure, where they quickly activate cytotoxic CD4+ T cells which specifically target tumor cells and thus eliminate the cancer. A long-lasting anti-cancer immune response is maintained via both CD4+ and CD8+ cytotoxic T cells.

AVR-02 is designed to deliver lasting benefits for Fabry disease patients. The company’s approach is to genetically modify a patient’s own cells by adding a functional copy of the faulty gene. CD34+ hematopoetic stem cells are genetically modified to express the enzyme alpha-galactosidase A. The modified cells are then infused back into the patient via a one-time procedure. The objective is to deliver long-lasting or permanent, continuous elevation of endogenous enzyme thereby significantly improving patient outcomes and eliminating onerous lifetime biweekly intra-venous infusions of enzyme replacement therapy.

“Atlas is very pleased to invest in the AVROBIO team and their novel therapies. We look forward to very near-term generation of clinical data in 2016 from both phase 1 trials,” said Bruce Booth, Partner at Atlas Venture and member of AVROBIO’s Board of Directors.

About AVROBIO

AVROBIO is a clinical stage company developing transformative ex-vivo gene therapies targeting cancer and rare diseases. The catalyst for creating AVROBIO is to accelerate scientific breakthroughs related to the convergence of cell and gene therapies. The patient’s own cells can be effectively modified to deliver novel genes to treat serious debilitating disease resulting in durable patient outcomes. A unifying theme across our portfolio is the expert design and manufacturing of optimal cell and gene delivery systems. AVROBIO has offices in both Toronto, ON and Cambridge, MA. More information is available at www.avrobio.com.

About Atlas Venture

Atlas Venture is a biotech-focused, early-stage venture capital firm that creates and invests in life sciences startup companies in the U.S. Atlas is based in Cambridge, Massachusetts. Since 1993, Atlas has invested in over 150 early stage life sciences companies. For more information, visit www.atlasventure.com.

About the University Health Network (UHN)

University Health Network consists of Toronto General and Toronto Western Hospitals, the Princess Margaret Cancer Centre, Toronto Rehabilitation Institute, and The Michener Institute for Education at UHN. The scope of research and complexity of cases at University Health Network has made it a national and international source for discovery, education and patient care. It has the largest hospital-based research program in Canada, with major research in cardiology, transplantation, neurosciences, oncology, surgical innovation, infectious diseases, genomic medicine and rehabilitation medicine. University Health Network is a research hospital affiliated with the University of Toronto. Visit us at www.uhn.ca. Technology Development and Commercialization (TDC) is the team at the University Health Network (UHN) that facilitates the transformation of hospital research innovations into medical products that improve healthcare outcomes. Learn more at http://tdc.uhnresearch.ca.

About the Centre for Commercialization of Regenerative Medicine (CCRM)

CCRM, a Canadian not-for-profit organization funded by the Government of Canada's Networks of Centres of Excellence program, the Province of Ontario, and leading academic and industry partners, supports the development of regenerative medicines and associated enabling technologies, with a specific focus on cell and gene therapy. A network of academic researchers, leading companies, strategic investors and entrepreneurs, CCRM aims to accelerate the translation of scientific discovery into marketable products for patients with specialized teams funding and infrastructure. CCRM sources and evaluates intellectual property from around the globe, offers various consulting services, conducts development projects with partners, and establishes new companies built around strategic bundles of intellectual property. CCRM has a fully resourced, 6,000 square foot development facility used to both evaluate and advance technologies, and 40,000 square feet in development for advanced cell manufacturing. CCRM is the commercialization partner of the Ontario Institute for Regenerative Medicine and the University of Toronto's Medicine by Design. CCRM is hosted by the University of Toronto and was launched in Toronto's Discovery District on June 14, 2011. Visit us at www.ccrm.ca.

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