Vironexis Biotherapeutics Launches with FDA Clearance of IND Application for First-Ever Clinical Trial of an AAV-delivered Cancer Immunotherapy

- Vironexis's off-the-shelf, single-dose gene therapies aim to deliver cancer treatments that are safer, more effective and long-lasting, and more easily manufactured and administered
- Leveraging its TransJoin™ platform, Vironexis has built a pipeline of more than 10 product candidates for blood-based cancers, solid tumor metastasis prevention and a cancer vaccine
- Vironexis's foundational technology is based on pioneering, published research led by Timothy Cripe, M.D., Ph.D., at Nationwide Children's Hospital
- Vironexis's $26 million seed financing was led by Drive Capital and Future Ventures, with participation from Moonshots Capital and Capital Factory
- Vironexis plans to advance its first two oncology programs into the clinic in Q4 2024 and 2025, respectively

SAN DIEGO, Calif., September 12, 2024 – Vironexis Biotherapeutics, focused on transforming the future of cancer treatment by pioneering AAV-delivered T-cell immunotherapy, launched from stealth today, unveiling its TransJoin™ AAV Gene Therapy Platform and a pipeline of more than ten product candidates for blood-based cancers, solid tumor metastasis prevention, and a cancer vaccine. Vironexis's $26 million seed financing was led by Drive Capital and Future Ventures, with participation from Moonshots Capital and Capital Factory. The company has received clearance of its Investigational New Drug (IND) application from the U.S. Food and Drug Administration (FDA) for VNX-101, its first gene therapy product candidate, for the treatment of CD19+ acute lymphoblastic leukemia. Vironexis anticipates initiating patient enrollment of a Phase 1/2 trial of VNX-101 in the fourth quarter of 2024, which will mark the first-ever clinical trial of an AAV-delivered cancer immunotherapy. VNX-101 has received both Fast Track Designation and Rare Pediatric Disease Designation from the FDA.

"We're excited to launch Vironexis from stealth and reveal our noteworthy progress advancing AAV-delivered T-cell immunotherapy," said Samit Varma, co-founder and CEO of Vironexis. "Our novel technology builds on the power of T-cell immunotherapy while overcoming key shortcomings and challenges of existing approaches such as CAR-T and bispecific antibodies. We believe we have the opportunity to dramatically improve upon the safety, efficacy and durability of these drug classes, while streamlining manufacturing and significantly lessening the burden of treatment for patients. Our focus on execution has yielded an expansive pipeline and a clinic-ready lead program in just three years. We're working as quickly as possible to transform the future of cancer treatments for patients."

TransJoin enables a patient's body to express an engineered transgene that redirects T cells throughout the body to tumor cells. The proprietary technology requires only a single dose and provides a bridge that joins together T cells and tumor cells to promote long-term, continuous T cell-mediated tumor killing. The foundational technology for TransJoin was licensed from Nationwide Children's Hospital. In

2022, research led by Timothy Cripe, M.D., Ph.D., Chief of the Division of Pediatric Hematology/Oncology/Bone and Marrow Transplant, describing the TransJoin technology was published in *Science Advances* [link here to paper]. Dr. Cripe is one of Vironexis's co-founders, along with Mr. Varma, a seasoned biotechnology and gene therapy company entrepreneur, and Brian Kaspar, Ph.D., a notable gene therapy scientist-entrepreneur who founded AveXis, Inc. (acquired by Novartis) and pioneered the AAV gene therapy ZOLGENSMA®.

"AAV is a proven delivery technology with multiple approvals since 2017. Recognizing the pivotal impact of AAV delivery for the treatment of rare diseases, we believed its unique ability to enable long-term, continuous expression of a therapeutic protein could be the missing link to overcome the myriad challenges associated with first-generation T-cell immunotherapies like CAR-T. We subsequently demonstrated the potential of this approach in the preclinical setting," said Dr. Cripe. "It's thrilling to now be on the cusp of seeing how this technology translates in the clinical setting. Our ultimate goal is to help a vastly broader population of patients realize the tremendous benefits of T-cell immunotherapy."

Vironexis is in the process of obtaining pre-IND input from the FDA for its second program, VNX-202, as a treatment for the prevention of metastatic HER2+ cancer (including breast cancer and other tumor types), and plans to start dosing patients in a Phase 1/2 clinical trial in 2025. The company's other current product candidates include treatments for BCMA/GPRC5D+ multiple myeloma, CD19/20+ B-cell lymphoma; treatments to prevent metastases in GD2+ neuroblastoma, HER2+ gastric cancer, PSMA+ and MSLN+ pancreatic cancer, B7H3+ ostersarcoma and GP350+ nasopharyngeal cancer; a cancer vaccine for GP350+ nasopharyngeal cancer; and a treatment for CD19+ systemic lupus erythematosus, which Vironexis plans to partner for further development.

Molly Bonakdarpour, Partner at Drive Capital commented, "As a portfolio investment, Vironexis offers an ideal blend of groundbreaking technology, impressive preclinical data, the potential for vast patient impact, and a founding team with deep, relevant expertise and a proven track record of company formation, strategic thinking, and successful execution. Vironexis's notable productivity in a very short timeframe has been remarkable. We look forward to its upcoming transition to a clinical-stage company."

Steve Jurvetson, Co-Founder of Future Ventures, added, "We were drawn not only to the novelty of the TransJoin technology but also to its broad applicability, spanning treatments for blood-based cancers, solid tumor metastasis prevention, and cancer vaccines, as well as immune disorders. The versatility of this platform is truly standout, and the Vironexis team's progress in rapidly building a pipeline that explores the expanse of these opportunities is extraordinary."

How TransJoin Works

Vironexis's TransJoin technology is designed to enable the expression of a secreted T cell engager that binds the tumor cell on one side (changeable depending on the product candidate indication target) and T cells via CD3 on the other side. CD3 is a protein that helps promote T-cell recognition of and activation against cancer cells. Following a single, one-time intravenous infusion, the TransJoin technology instructs the liver to continuously secrete the bispecific protein into the bloodstream to redirect T cells to tumor cells. TransJoin provides a bridge that joins T cells and tumor cells, resulting in long-term, consistent

serum levels of the therapy and, thus, long-term, consistent T-cell-mediated tumor cell killing. TransJoin's extremely low-dose AAV delivery minimizes toxicity and adverse events.

About Vironexis Biotherapeutics

Vironexis is focused on transforming the future of cancer treatment by pioneering AAV-delivered T-cell immunotherapy. Our TransJoin™ AAV Gene Therapy Platform enables the creation of off-the-shelf, single-dose gene therapies designed to overcome the key challenges and shortcomings of current immunotherapies, including CAR-T and bispecific antibodies. Our current pipeline includes more than ten product candidates for blood-based cancers, solid tumor metastasis prevention, and a cancer vaccine. Our lead program, VNX-101, for the treatment of CD19+ acute lymphoblastic leukemia, is anticipated to begin clinical trials in the fourth quarter of 2024. Visit us at vironexis.com and follow us on LinkedIn and X.

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