

eGenesis Study Addressing Cross-Species Viral Transmission Concern in Xenotransplantation Published in Science

First Pigs without Active Porcine Endogenous Retrovirus (PERV) Produced Using CRISPR-Cas9 Genome Editing Technology – a Landmark Advance for Xenotransplantation

Eliminating PERV Establishes a Foundation for Safe Xenotransplantation

CAMBRIDGE, Mass. – August 10, 2017 – eGenesis, a biotechnology company focused on transforming xenotransplantation into a lifesaving medical procedure, announced the publication of a study in the journal [Science](#) by eGenesis scientists and their collaborators demonstrating the inactivation of PERV to prevent cross-species viral transmission and a breakthrough in producing the first PERV-free pigs, an important milestone for xenotransplantation.

Xenotransplantation, the use of animal organs for human transplant, is a promising approach to alleviate the severe shortage of organs for human transplantation but the risk of cross-species transmission of PERVs, among other issues, has to date impeded its use in humans. eGenesis is committed to harnessing CRISPR technology to deliver safe and effective human transplantable cells, tissues and organs grown in pigs, thus addressing a dire need for hundreds of thousands of patients worldwide.

“This is the first publication to report on PERV-free pig production,” said Luhan Yang, Ph.D., co-founder and chief scientific officer at eGenesis. “We generated a protocol to enable multiplex genome editing, eradicated all PERV activity using CRISPR technology in cloneable primary porcine fibroblasts and successfully produced PERV-free piglets. This research represents an important advance in addressing safety concerns about cross-species viral transmission. Our team will further engineer the PERV-free pig strain to deliver safe and effective xenotransplantation.”

This study examined the risk of PERV infectivity and demonstrated *in vitro* that PERVs infected human cells and were transmitted to human cells that had no history of contact with porcine cells in the co-culture condition, substantiating the need to address this issue in order to ensure safe xenotransplantation practice.

Researchers developed a strategy to enable efficient and precise genome editing in primary cells using CRISPR-Cas9 technology. In conjunction with a method to inhibit primary cell death during multiplex genome editing, researchers successfully produced viable PERV-free porcine embryos via somatic cell nuclear transfer, using engineered primary cells. They then implanted the PERV-free embryos into surrogate sows and demonstrated the absence of PERV re-infection, initially in fetuses and finally in recently born piglets. These



piglets are the first animals born free of endogenous virus and will be monitored for any long-term effects and impact by the eGenesis team.

The eGenesis team, having produced the first piglets free of active PERVs, is working toward combining the safety benefits of PERV-free pigs with additional gene editing addressing immunological response to increase organ immune and functional compatibilities.

About CRISPR Gene Editing Technology

CRISPR is a genome-editing tool that can selectively delete, modify or correct a disease causing abnormality in a specific DNA segment. CRISPR refers to Clustered Regularly Interspaced Short Palindromic Repeats occurring in the genome of certain bacteria. CRISPR technology uses a protein-RNA complex composed of either the protein Cas-9 or Cpf1, each of which binds to a guide RNA (gRNA) molecule that has been designed to recognize a particular DNA sequence.

About Xenotransplantation

Currently in the United States, there is a tremendous unmet demand for transplant organs with more than 118,000 people in need of a lifesaving organ transplant. Of those, more than 75,000 people are active waiting list candidates. The concept of cross-species transplantation, known as xenotransplantation, is the transfer of living cells, tissues or organs from one species to another. Due to the shortage of human organs, xenotransplantation emerged as an alternative potential option and its clinical potential is being explored with new technologies such as CRISPR Cas-9. Xenotransplantation is not new – the first serious attempts (then called heterotransplantation) first appeared in the scientific literature in 1905 and it has been explored with limited success over the last century.

About eGenesis

eGenesis is a biotechnology company focused on leveraging the advancements of gene editing technologies to deliver safe and effective human transplantable cells, tissues and organs to the hundreds of thousands of patients worldwide who are in dire need.

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