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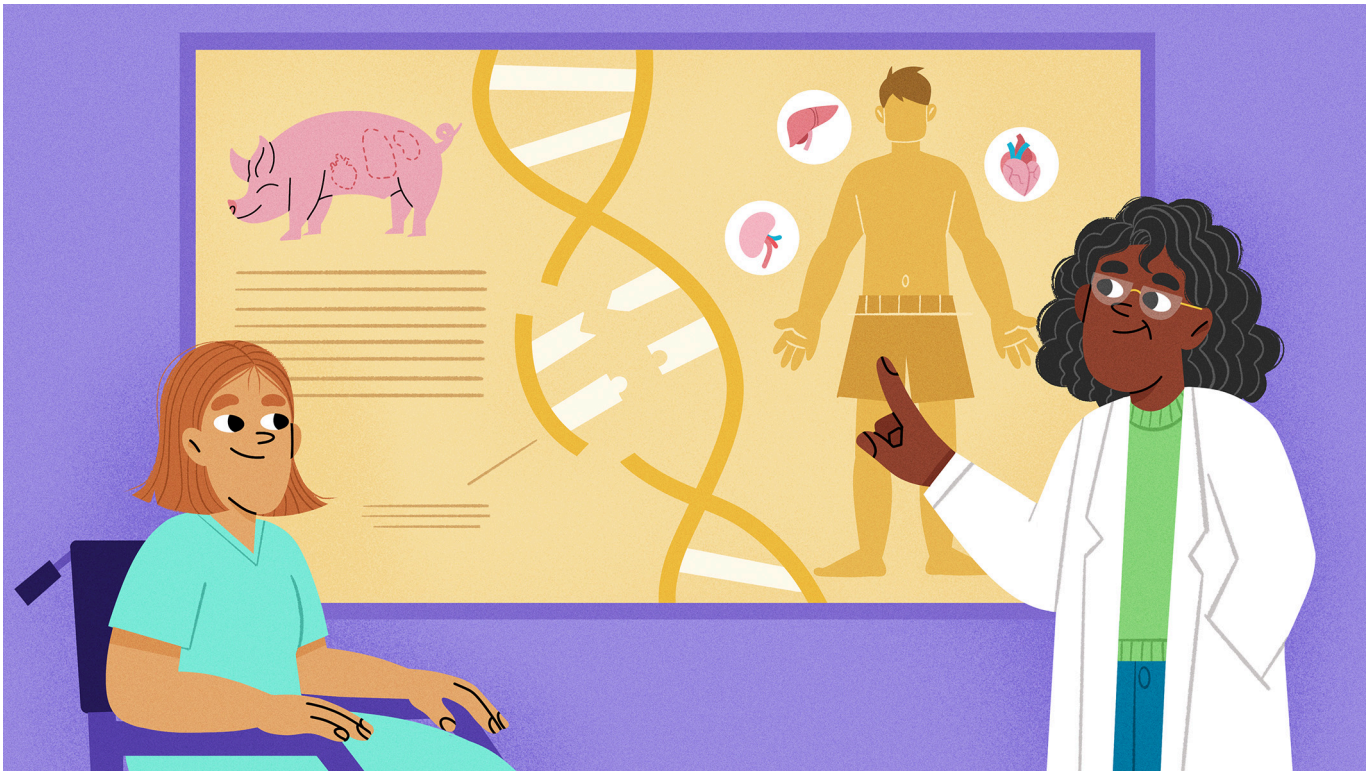

SAVING LIVES WITH GENE-EDITED ORGANS

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YOUNG REVIEWERS:


ESHAAN
AGE: 13


STEPHANIE
AGE: 14


VIHAAN
AGE: 10

Thousands of people around the world are waiting for life-saving organ transplants, but there are not enough organs to meet the demand. Gene-edited organs from animals, especially pigs, could help solve the organ shortage. Using a technology called gene editing, scientists can change the DNA of pig organs, making them safer and more compatible with human bodies by turning off genes that cause the body to attack the foreign organ and removing dangerous virus genes. This technology is already being tested—pig kidneys and hearts have been transplanted into patients, providing important information that will help improve future procedures. Scientists are also studying how transplanted pig cells could treat diseases like diabetes and Parkinson's. While challenges and ethical questions remain, this technology has the potential to save thousands

ORGAN TRANSPLANTS

A medical procedure where a healthy organ from one person or animal is placed into another person to replace a damaged or failing organ.

DONOR

A person or animal who gives an organ for a transplant, either after they pass away or, in some cases, while they are still alive.

RECIPIENT

The person who receives an organ during a transplant.

REJECTION

When the recipient's immune system attacks and damages a transplanted organ, treating it as a threat.

IMMUNE SYSTEM

The body's defense system that fights off germs and infections. It can sometimes attack a transplanted organ, thinking it is a dangerous invader.

XENOTRANSPLANTATION

Transplanting organs or cells from one species (like pigs) into another species (like humans).

GENE EDITING

A process that allows scientists to change DNA—the instructions for how cells work—to make animals or plants better suited for specific purposes, like creating organs compatible with humans.

of lives every year. With more research, gene-edited organs could give people in need of transplants a second chance at life.

THE WORLD NEEDS MORE ORGANS FOR TRANSPLANTS

Imagine being really sick and needing a new heart or kidney to survive, but being told you might have to wait years—if an organ becomes available at all. This is the reality for thousands of people who need **organ transplants**. While these life-saving procedures have helped millions, the demand for organs far exceeds the supply. Behind every transplant is a person—often someone very sick, who may need machines to keep them alive while they wait. For many families, each day without an available organ is a race against time.

Organ transplants rely on organ **donors**—people who provide an organ, either after they pass away or, in some cases, while they are still alive, such as donating one kidney or part of the liver. The person receiving the transplant is called the **recipient**. Commonly transplanted organs include kidneys, hearts, livers, and lungs, but the waiting lists for these organs are long. In the United States, over 100,000 people are waiting for an organ transplant, but only about 30,000 transplants will happen this year. Some patients may wait years for an organ, and sadly, many do not survive that long.

Even when a matching organ becomes available, there is another big challenge: **rejection**. The recipient's **immune system**, which protects the body from dangerous invaders, may see the transplanted organ as something unfamiliar and attack it, like it would a virus or bacteria. To prevent this, patients take medications to weaken their immune systems, but these drugs have side effects and are not always effective at stopping rejection.

To address the shortage of organs, scientists are exploring a surprising source: animals—specifically pigs (Figure 1). A new field called **xenotransplantation** is studying how organs from animals could be used to save human lives. But if the human body can reject an organ from another person, how could it possibly accept one from a completely different species?

EMERGING TECHNOLOGY: GENE-EDITED ORGANS

To make animal organs suitable for human transplants, scientists are using a powerful tool called **gene editing**. Gene editing allows scientists to make precise changes to an animal's DNA—the instructions that tell cells how to grow and function. One of the most commonly used tools for gene editing is called CRISPR-Cas9.

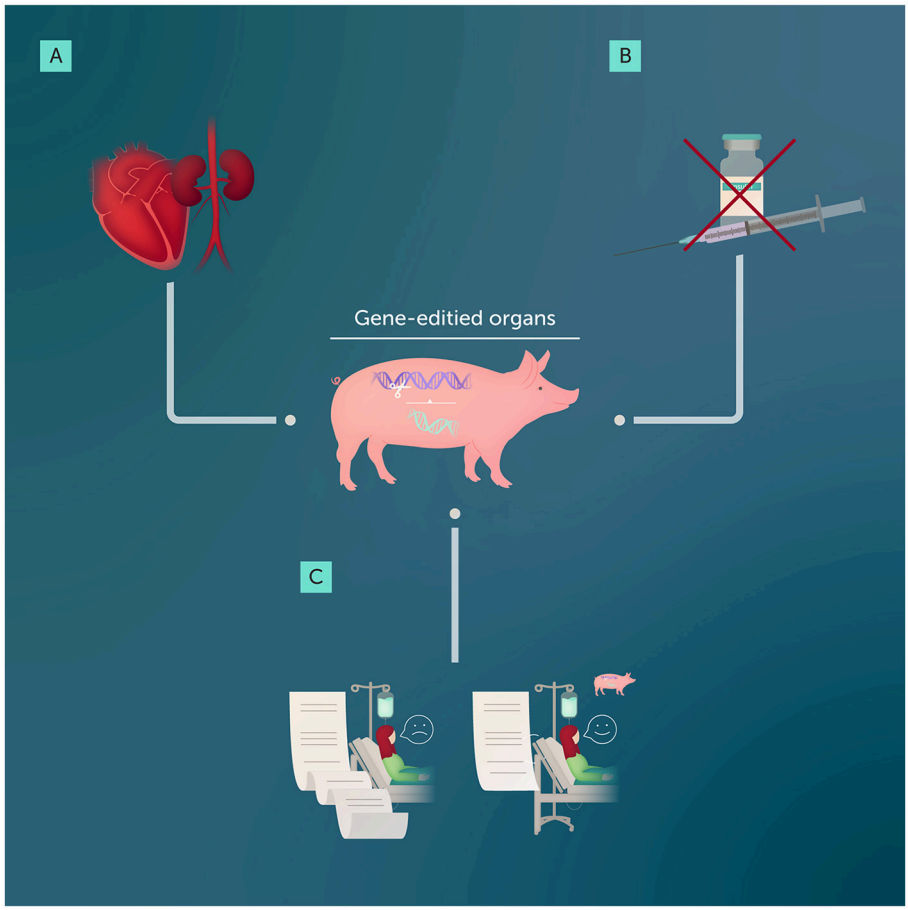

Figure 1

Gene editing can help to make organs from other animals suitable for transplanting into humans. **(A)** Early cases in which hearts and kidneys from pigs were transplanted into humans have provided scientists and doctors with the valuable information they need to make xenotransplantation safer and less likely to result in organ rejection. **(B)** Gene-edited pigs could provide pancreas cells that could reduce the need for insulin injections in people with diabetes. **(C)** Overall, successful xenotransplantation using gene-edited organs could shorten the wait list for donor organs, saving thousands of lives every year.

You can generally think of this technique like a pair of molecular scissors that can cut and paste specific bits of DNA exactly where they are needed.

In xenotransplantation, gene editing addresses two big problems. First, the human immune system naturally sees a pig organ as a threat and attacks it, causing rejection. To prevent this, scientists edit the DNA of pigs while the animals are still embryos, long before they are born. These changes include "turning off" certain pig genes that trigger the human immune system and adding human genes to the pig's DNA, to make the organ more compatible with the human body. The pig organs grow with these edits already in place, and when they are big enough, they are ready for transplant. The second problem gene editing addresses is safety. Like humans and many other animals, pigs have leftover virus DNA inside their own DNA—kind of like old "genetic scars" from infections their ancestors had long ago. These virus bits usually do not do anything, but there is a small chance they could become active and cause problems in humans after a transplant. Using gene editing, scientists can remove these virus genes to help make the organs safer for people who receive them.

With gene editing, scientists can engineer pig hearts or kidneys to work inside human bodies without causing harm (Figure 1). In some cases, scientists need to make multiple edits to the pig's DNA—usually around 10, but sometimes up to 69—to ensure the organs are safe and effective for transplantation [1].

TECH TO THE RESCUE

Gene-edited organs are already showing promise for saving lives. In 2024, doctors successfully transplanted a kidney from a pig into a human patient [2]. This groundbreaking procedure showed that xenotransplantation could work and paved the way for future advancements. The patient lived for 2 months after the surgery, and although he passed away, his death was not related to the transplant. Gene-edited pig hearts have also been transplanted into human patients [3, 4]. While these patients did not survive long-term, these early cases provide valuable information to improve the technology and make it safer.

Gene-edited pig organs could also help treat diseases like diabetes. Diabetes occurs when the pancreas, an organ that produces insulin, stops working properly and does not produce enough insulin. Insulin is a hormone that helps move sugar from the blood into the body's cells, where it is used for energy. People with type 1 diabetes often take daily insulin injections to manage their blood sugar. Scientists are exploring the idea of transplanting just the insulin-producing cells, called islet cells, from the pancreas of gene-edited pigs into patients with diabetes (Figure 1). These cells might restore the function of the pancreas, and reduce or even eliminate the need for daily injections.

Beyond diabetes, gene-edited pigs could provide cells to treat other conditions. Parkinson's disease is a brain condition that affects movement and coordination. Parkinson's patients might be treated with transplanted pig brain cells, which could replace damaged cells in the human brain, improving a patient's symptoms and quality of life. Scientists are also studying how cells from gene-edited pigs might repair other damaged tissues or treat diseases like liver failure.

BIG CHALLENGES, BIGGER OPPORTUNITIES

Although much work remains, the use of gene-edited organs in xenotransplantation is a promising step toward solving the organ shortage crisis. One remaining hurdle is the risk of rejection. Even with gene editing, doctors must carefully monitor patients and use special medicines to help their bodies accept the transplants. Scientists are working to improve the gene-editing process to make pig organs even more compatible with human bodies.

Safety is another concern. As we mentioned, pigs naturally carry viruses in their DNA that might harm humans if transmitted during a transplant. Removing these virus genes with gene-editing tools lowers this risk, but more testing is needed to make sure the transplants are completely safe.

There are also ethical questions. For example, how do we balance the need to save human lives with ensuring the welfare of animals used for transplants? These important conversations will shape how xenotransplantation is developed and used in the future.

With more research and advancements in gene editing to overcome these challenges, xenotransplantation could eventually save thousands of lives each year. Imagine a world where no one has to wait years for a life-saving organ or worry about their body rejecting a transplant. Scientists, doctors, and policymakers are working together to make this vision a reality, offering hope to patients and families around the world.

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CONFLICT OF INTEREST: The authors declare that the research was conducted in the absence of any commercial or financial relationships that could be construed as a potential conflict of interest.

YOUNG REVIEWERS

ESHAAN, AGE: 13
Eshaan is a 13-year-old who loves math and science. He likes to read and play with his little brother. He also likes to tell jokes to people. His favorite subjects in math are counting and number theory. His least favorite thing is PE class.

STEPHANIE, AGE: 14
My name is Stephanie and I am 14 years old. I am an eighth grader in middle school and my hobbies include singing, playing tennis, and playing the clarinet. My favorite subject in school is ELA and I enjoy reading and writing mystery and dystopian stories. I have performed for the UniverSoul Circus before and I have been a reviewer for some scientific articles in this journal.

VIHAAN, AGE: 10
Vihaan is an awesome 10 year old who likes to play with brother, read books, and sculpt with clay. He likes creating funny jokes, rock climbing, and is very active. He likes to learn facts he may never need to use.

AUTHORS

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David K. C. Cooper is a senior investigator at the Center for Transplantation Sciences at Massachusetts General Hospital. He has spent decades working on ways to solve the shortage of human organs available for transplant. Originally trained as a heart surgeon, Dr. Cooper became one of the world's leading researchers in xenotransplantation—the idea of using animal organs to save human lives. His work focuses on making pig organs safer and more compatible with the human body through gene editing. He has published hundreds of scientific papers and has helped shape the field through both research and clinical collaboration around the world.
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Emanuele Cozzi is a professor of transplantation immunology at the University of Padua Hospital in Italy. He studies how the immune system responds to transplanted organs and how to prevent rejection, especially when using organs from animals. Dr. Cozzi has played a key role in advancing xenotransplantation research in Europe and has contributed to international efforts to make gene-edited pig organs safer for human patients. His work combines laboratory science with clinical insights to help improve transplant outcomes and save more lives.

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Geoffrey Ling is a professor of neurology at Johns Hopkins Hospital and a leading expert in brain injury and neurotechnology. He has served as a physician in the U.S. Army and worked on medical innovations at the U.S. Department of Defense, where he helped launch major programs to improve care for patients with traumatic brain injuries. Dr. Ling has also been involved in developing advanced treatments using living cells, including research on how cells from gene-edited pigs might be used to treat neurological diseases like Parkinson's. His work bridges neuroscience, engineering, and medicine to improve patient care.

BERNARD MEYERSON
Bernard Meyerson is Chief Innovation Officer Emeritus at IBM, where he led major advances in computing and technology. A physicist and inventor, he is known for developing new materials and processes that helped improve the speed and efficiency of computer chips. Dr. Meyerson has advised global organizations on science and technology policy, and he is a strong advocate for using innovation to solve big challenges in health, sustainability, and global equity. He brings this perspective to his work on emerging medical technologies, including the potential of gene-edited organs to save lives.