

Research Matters

Your global research round-up



Dear Friend,

Welcome to *Research Matters*, your summary of medical research breakthroughs from home and around the world. Every two months, the team at the Perkins will hand-pick fascinating science stories for you!

This is your first edition.

Below are some of 2025's biggest breakthroughs, as well as more recent discoveries. You'll find a promising early-step breakthrough in pancreatic cancer treatment, a new potential way to treat autoimmune diseases, a revolutionary cancer reversal discovery, a world-first treatment that saved a baby's life and a new drug invented right here at the Perkins to melt plaque and treat cardiovascular disease forever.

You're receiving this email because you signed up to receive it via our website. Thank you for choosing to stay up to date with the latest research. We hope you find this information interesting and useful.

Happy reading!



Professor Peter Leedman AO
CEO, Researcher, Doctor, Donor
Harry Perkins Institute of Medical Research

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PANCREATIC CANCER

A promising early step
in new treatment



You could be witnessing a game-changer for one of the world's deadliest cancers!

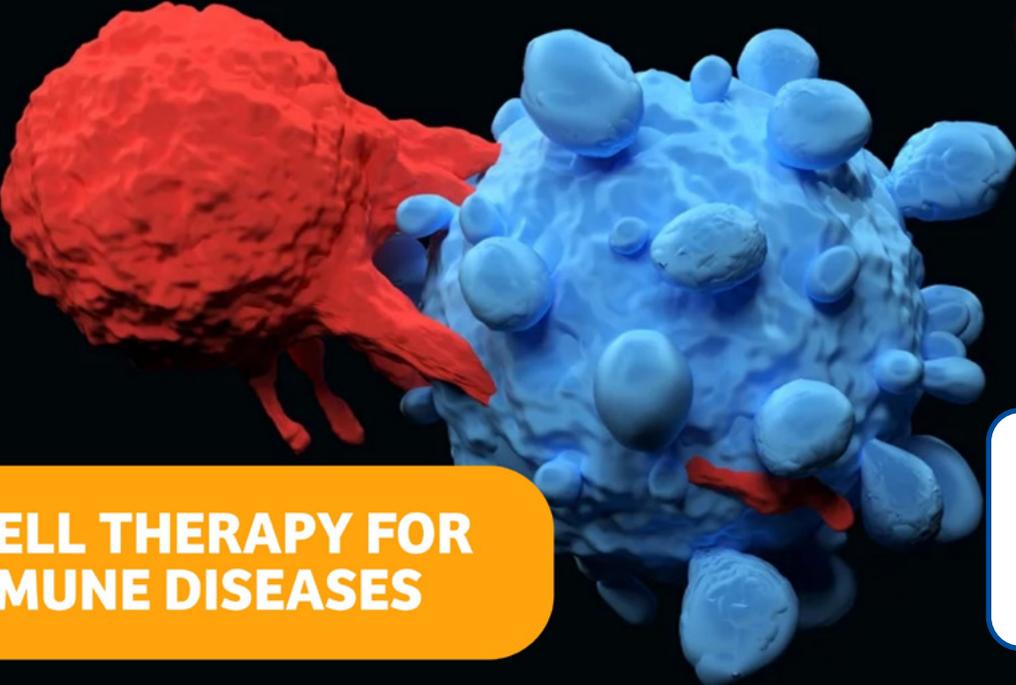
A Spanish research team lead by biochemist Mariano Barbacid has designed a three-pronged therapy (combining a drug, an experimental mutated gene inhibitor and a protein degrader) that successfully eliminated pancreatic tumours in mice. These tumours were eradicated completely and durably, with no significant side effects.

These studies open the pathway to design novel combination therapies that may improve the survival of pancreatic cancer patients. In Australia, the five-year survival rate for pancreatic cancer is amongst the lowest of all cancers at 14%. The increasing incidence and low survival rate made pancreatic cancer the third-most deadly cancer in 2025.

However, regarding next steps, Barbacid said that “it is important to understand that although experimental results like those described here have never been obtained before, we are still not in a position to carry out clinical trials with the triple therapy.”

The authors of the publication emphasise this further. “Despite the current limitations, these results could open the door to new therapeutic options to improve the clinical outcome of patients with pancreatic ductal adenocarcinoma in the not too distant future.”

This study was published in the journal *Proceedings of the National Academy of Sciences* with Carmen Guerra, PhD as co-lead author and Vasiliki Liaki, PhD and Sara Barrambana as first authors. Scan the QR code to read more!



CAR T-CELL THERAPY FOR AUTOIMMUNE DISEASES



Imagine reprogramming your immune system to protect itself! That's what scientists in Barcelona have just tested, using a cancer treatment concept to treat autoimmune diseases.

Research conducted by a team of scientists based in Barcelona revealed that CAR T-cell therapy (a revolutionary technology originally developed to help treat blood-related cancers using the body's own immune cells) may also have applications in restoring the immune system and treating autoimmune diseases like systemic lupus erythematosus (SLE).

SLE is a chronic, unpredictable autoimmune disease that attacks healthy tissue, causing widespread inflammation and damage to joints and organs like the skin, kidneys and the brain.

CAR T-cell therapy is a form of immunotherapy that uses the patient's own T lymphocytes (immune cells) that are genetically modified in the lab to express a Chimeric Antigen Receptor (CAR). These receptors enable the T-cells to identify with high precision a target on the surface of other cells.

CAR T-cell therapy represents a conceptually new approach to treating severe autoimmune diseases. Rather than continuously suppressing the immune system, it may allow for deep immune reprogramming by targeting B-cells and the T-cells with which they interact.

Read the review published in the journal *Clinical Reviews in Allergy & Immunology*, lead by researcher Dr Ahmad Matarneh, in the QR code above.



CANCER CELL REVERSION

Original technology that changes a cancer cell's characteristics



What if you could reverse the process of a cell becoming cancerous so it becomes more like a healthy cell again?

A Korean-based group lead by Professor Kwang-Hyun Cho has begun development of an original technology that centres on cancer reversal treatment.

This treatment differs from more common treatments in that it does not kill cancer cells, but changes their characteristics to reverse them to a state similar to normal functioning cells.

The team has found a molecular switch inside a cell's genetic network that induces cancer reversal at the precise moment a normal cell changes into a cancer cell, called the 'critical transition' moment.

The discovery of a biological 'undo' button is groundbreaking and continues to be studied by Prof Kwang-Hyun and his team. Read more about their discovery published in *Advanced Science* journal by scanning the QR code above.





FIRST EVER BESPOKE CRISPR THERAPY FOR BABY KJ



Now this is taking your personalised medicine to the limits!

In a world first, a baby boy in Philadelphia named KJ who had a devastating genetic disease is now thriving after becoming the first known person to receive a bespoke, CRISPR therapy-for-one to mend a disease-causing gene mutation.

CRISPR (Clustered Regularly Interspaced Short Palindromic Repeats) is a powerful, precise gene-editing technology that scientists use to cut and modify specific DNA sequences in living cells, offering potential cures for incredibly rare genetic diseases and cancer.

Researchers tailored KJ's CRISPR-based therapy to correct a specific genetic sequence in his genome.

Little KJ received three doses of the gene-editing treatment that mended a mutation that impaired his body's ability to process protein. For the first time in his life his body was able to metabolise the protein, stopping dangerous ammonia from building up in his body and damaging his brain.

Read the full *New England Journal of Medicine* publication, the research lead by Cardiologist Kiran Musunuru of the University of Pennsylvania Perelman School of Medicine and Rebecca Ahrens-Nicklas, a physician-scientist at Penn Medicine and the Children's Hospital of Philadelphia by scanning the QR code above.





ATHERID - A DRUG CANDIDATE FOR CARDIOVASCULAR DISEASE



A home-grown world-first treatment that can dissolve the fat built up in the walls of your arteries. Now *that's* a life-saver!

Professor Shirley Jansen AM, Director of the Heart & Vascular Research Centre at the Harry Perkins Institute of Medical Research, and Associate Professor Juliana Hamzah, Group Leader of the Perkins Targeted Drug Delivery, Imaging and Therapy Lab, have been on a 10-year journey, culminating in developing a world-first drug candidate that dissolves plaque build-up to treat atherosclerosis. This is a blood vessel disease that causes a build-up of cholesterol and other fats within artery walls.

This build-up causes veins to narrow and become more prone to blockage, increasing the risk of stroke, heart attack, kidney failure and other life-threatening conditions.

The drug candidate, ATH01, could offer the cure.

Juliana and Shirley have used the research discovery to establish Atherid Therapeutics Pty Ltd. They have received \$750,000 in Medical Research Future Fund funding through the *Targeted Translation Research Accelerator Drugs and Devices* program and are currently producing the drug in preparation for a future clinical trial.

You can read more about this innovative treatment and its development by scanning the QR code above.

