

Research Matters

Your global research round-up



Dear Friend,

Welcome to the third edition of *Research Matters*, your update of medical research breakthroughs and information delivered straight to your inbox.

In this edition, you'll find:

- information about ATH01, a new drug candidate that can permanently cure lipids and fats in arterial walls
- a spinal cord repair and function breakthrough
- a landmark pancreatic cancer trial
- a world-first treatment for epilepsy
- information about 'bispecific antibodies' and how they are used to treat cancer
- how you can become a Research Buddy and help guide research into protecting kidneys after heart surgery (and earn an honorarium of \$50/hour)

I hope you find this information interesting and useful!

You're receiving this email because you signed up via our website. Thank you for choosing to stay up to date with the latest global medical research.

Happy reading.



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



ATH01 COMMUNITY Q&A RECORDING



ATH01 – A world-first plaque-busting drug candidate.

What if there was a drug that could cure atherosclerosis, one of Australia's leading cardiovascular diseases? Here's your early access to the video recording of our recent Community Q&A, where we discussed drug-candidate 'ATH01' and spoke to the pioneers behind this ground-breaking research.

Eagle-eye readers would remember we talked about this drug candidate in the very first issue of Research Matters. We're happy to share this progress update!

On 29 September Perkins researchers A/Prof Juliana Hamza and Prof Shirley Jansen AM spoke at what was an inspiring panel discussion exploring the journey of ATH01, a world-first drug candidate designed to dissolve cholesterol and other harmful lipids from inside artery walls, potentially preventing heart attacks, strokes and amputations caused by atherosclerosis.

After more than a decade of research and extensive pre-clinical testing, ATH01 is on the path towards first-in-human clinical trials.

As an important subscriber to Research Matters, you get early-access viewing of the recording of this Q&A session. Discover how this remarkable innovation was developed, what makes it different from existing treatments and the potential it holds to transform care for patients at risk of cardiovascular disease by scanning the QR code on this page.





SPINAL CORD DAMAGE – IS NERVE REPAIR POSSIBLE?

Reversing spinal cord damage.

Can you believe that a modified protein could potentially help an injured spinal cord repair itself?

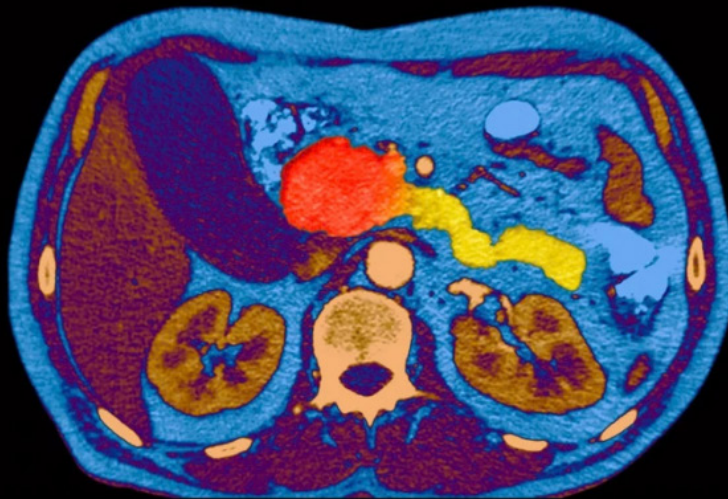
Professor Tatiana Lobo Coelho de Sempaio, a Brazilian biologist and scientist, is known for her research in cell biology, regenerative biology and the extracellular matrix, with much of her work focused on helping damaged nervous-system tissue recover.

A big part of her work is her research around laminins, a protein taken from the human placenta. These proteins help cells attach, move, develop and survive and play an important role in the nervous system.

Her research led to the development of polylaminin, a modified form of laminin. When injected into a damaged spinal cord, it fills the wounded space and forms a 'bridge' that allows neurons to reconnect above and below the injury, potentially restoring movement and sensation.

Read about this first-in-human pilot study to verify the safety and feasibility of polylaminin in people with spinal cord injury, published in Nature by scanning the QR code above.





PANCREATIC CANCER BREAKTHROUGH

A landmark pancreatic cancer trial.

You won't believe these unprecedented successful results against targeting pancreatic cancer.

A new experimental drug has shown to double the life-expectancy in people with a particular form of advanced pancreatic cancer.

The drug, daraxonrasib, disarms all three of the RAS family of proteins that are linked to some of the deadliest cancers. RAS proteins are molecular 'on-off' switches that help to control cell growth and division. Some mutations leave RAS proteins stuck in the 'on' position, which is what makes tumours grow uncontrolled.

Ideally, a cancer drug would switch these proteins off. Drugs typically work by nestling into deep pockets on the surface of proteins, but RAS proteins are unhelpfully smooth, leaving nowhere for the drugs to anchor to, making them ineffective.

A large clinical trial has found that daraxonrasib nearly doubled life-expectancy in people living with a form of advanced pancreatic cancer from 6.7 months to 13.2 months. This success is raising hopes that other seemingly untouchable tumour targets could soon be treated.

Read further about this breakthrough and clinical trial by scanning the QR code above.





LIFE-SAVING EPILEPSY TREATMENT

Stephanie Higginson with her son, Bohdi. Credit: © ABC News Timothy Ailwood.

A world-first treatment for rare genetic epilepsy disorder.

When Stephanie Higginson's son was three months old, treatment for his epileptic-induced seizures seemed impossible. On Bohdi Higginson's worst day, he had 74 seizures and there was nothing Stephanie could do to help.

At eight months old, Bohdi was diagnosed with KCNT1-related catastrophic epilepsy, an often-fatal genetic disorder with no known treatment at the time. Only 18 cases had ever been recorded in Australia.

Conventional medications were not helping, and as time progressed, Bohdi was missing developmental milestones. Without intervention, Bodhi's future looked bleak, with the chance of permanent disability or death increasing.

Bodhi has made medical history by becoming the first person in the world to receive a precision-medicine treatment for his epilepsy. He received his first dose of the treatment on April 21 and had his final seizure just three days later.

A multidisciplinary team assessed the treatment through the Sydney Children's Hospitals Network's (SCHN) new innovative therapies pathway, designed to fast-track access to promising treatments. This saved Bodhi's life.

The innovative therapies pathway could ultimately benefit other children with conditions that are currently difficult to treat. Learn more about Bodhi, his condition and what his treatment means for other children with rare genetic diseases by scanning the QR code on this page.





WHAT IS A BISPECIFIC ANTIBODY?

Bispecific antibodies (orange and yellow, artist's illustration) grab two different proteins rather than the standard one. Credit: © Thom Leach/Science Photo Library/Getty Images.

Bispecific antibody drug in limelight.

What if antibodies could be designed to bind to more than one target, improving their effectiveness? Californian researchers say that these antibodies, called bispecific antibodies, could be the next step in fighting cancer and other diseases.

Bispecific antibodies work by linking to a protein expressed by cancer cells and to immune cells called T-cells. Also called T-cell engagers, these bispecific antibodies stimulate T-cells to attack the cancer cell.

At least 12 such T-cell engagers have been approved for treating cancers, including leukaemia and myeloma.

With the success of bispecific cells, scientists are working on creating multispecific antibodies, which can bind to three or more proteins, that stimulate a far better immune response to cancer. Other companies are also making bispecific cells that target other cells outside of T-cells, improving the innovation's scope for disease treatment.

Read more about this by scanning the QR above.





BECOME A RESEARCH BUDDY!

Research buddy: help shape kidney research.

Have you or someone you care for experienced acute kidney injury, chronic kidney disease, dialysis or kidney complications following heart surgery? You could help shape research to protect kidneys!

Researchers in the Perkins Translational Renal Research lab are looking for people with lived experience to join the team as a Research Buddy and help shape a project aimed at identifying people at risk of kidney injury before they undergo cardiac surgery.

Your insights will help ensure this important research remains patient-centred, improves communication about kidney health risks and focuses on outcomes that matter most to patients and families.

Experience required:

Your lived experience as a patient or carer.

No prior research experience required.

Ability to be inclusive and clear with your communication.

Comfortable contributing to discussions in a group setting.

Familiar with Zoom/Teams and/or confident or able to come to the Harry Perkins Institute of Medical Research.

A \$50 per hour honorarium is offered to successful applicants.

Applications close 8 October, 2026. To learn more and apply, scan the QR code below.

Interested in becoming a Research Buddy for other involvement opportunities? Join the Consumer and Community Involvement Program by scanning this QR code and receive these opportunities straight to your inbox!

