10 YEARS:
10 STORIES OF IMPACT

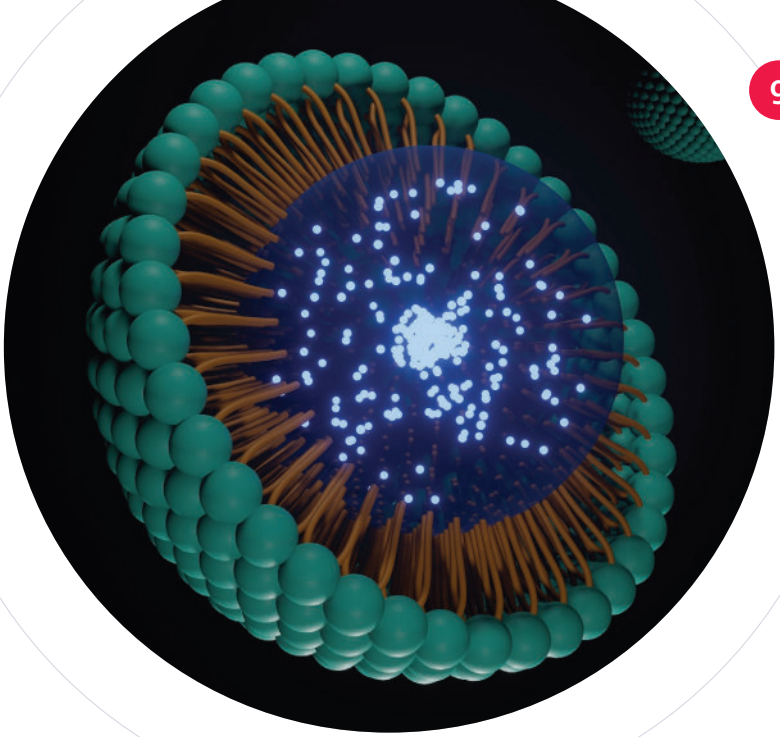
CASE STUDY 9

# Transforming T1D research into real-world solutions

There is an urgent need to take research discoveries from the bench to the bedside. JDRF and funding made possible through the Australian Type 1 Diabetes Clinical Research Network (T1DCRN) play a vital role in investing in early-stage discoveries to progress them to the point of commercial viability, at which point they invite support and funding from alternative sources that take them to clinical trials and into the hands of the T1D community.

Australian Type 1 Diabetes Clinical Research Network

JDRF IMPROVING LIVES. CURING TYPE 1 DIABETES.



## WHAT PROBLEM DO WE NEED TO SOLVE?

T1D research translation and commercialisation in Australia needs to be accelerated to progress discoveries from the bench to the bedside. This is vital to improve health outcomes as swiftly as possible and to maintain Australia's global leadership in T1D research.

Yet innovative and early-stage research that often offers the highest potential for novel breakthroughs is typically deemed too risky for investment by traditional funding bodies.

Organisations like JDRF play the vital role of supporting these projects in their early stages so they can overcome the initial barriers to progress to a point where they are commercially attractive and are likely to secure funding through alternative streams.

Without early support, countless discoveries would not have moved from the bench to the bedside, nor had any impact on people living with T1D.

## WHAT WAS FUNDED BY THE T1DCRN AND WHY?

Fostering research translation in T1D can create a thriving ecosystem that helps transform scientific breakthroughs into innovative therapies, diagnostic tools, and medical devices. Ultimately, these can unlock new means to intercept the progression of T1D, improve quality of life for those with T1D and reduce long-term complications associated with the condition.

For this to be possible, it is crucial to invest in innovative science ideas, robust infrastructure, and regulatory frameworks that support the translation of research findings into practical applications.

Strong partnerships between researchers, industry stakeholders, healthcare professionals, and advocacy groups can facilitate the rapid translation of discoveries into real-world solutions.

This investment in innovation and the translational pipeline is a fundamental component of the T1DCRN, with several researchers receiving support for novel scientific ideas that have since moved out of the lab, creating five commercial entities who are accelerating therapeutic developments to impact the lives of people with T1D.

## WHAT HAS THIS DELIVERED AND WHY IS THIS IMPORTANT?

One prominent example of taking a novel innovation from the lab to care is the journey of **Professor Ranjeny Thomas.**

As an immunologist at the University of Queensland and a consultant rheumatologist at the Princess Alexandra Hospital, Professor Thomas has been working towards developing a therapy to 're-train' the immune system and intercept the destructive autoimmune process in T1D.

Having studied rheumatoid arthritis for nearly 20 years, Professor Thomas and her team discovered a way to increase the body's tolerance of its own cells, thereby preventing the immune system from attacking the body. This approach was adapted from the discovery of a novel immunotherapy for T1D which combines small fragments of insulin antigens with nanoparticles containing an immunomodulatory drug.

Collaborating with **Associate Professor Emma Hamilton-Williams**, Professor Thomas demonstrated in pre-clinical models that this antigen-specific immunotherapy restored tolerance to autoimmune attack without compromising normal immunity against infections, suggesting its potential to prevent the destruction of insulin-producing beta cells.

With support through the T1DCRN and additional funding from The Leona M. and Harry B. Helmsley Charitable Trust, Professor Thomas completed the necessary pre-clinical studies for a first-in-human clinical trial. Engaging with contract manufacturing as well as regulatory and commercialisation experts ensured the suitability and safety of the therapy for human trials.

Professor Thomas is now at the stage of taking this therapy from the bench to the bedside, and is closer than ever to launching the immunotherapy into its first-in-human clinical trial.

This was supported through the establishment of a commercial entity, Liperate Therapeutics Pty Ltd, a startup company of which Professor Thomas is the scientific founder. In 2024, Liperate Therapeutics will initiate a first-in-human clinical trial testing the immunotherapy.

> Thanks to the long-term support of my funders, I have been able to develop my discoveries from initial conceptualisation all the way through to clinical trials, and my hope is that this will bring great benefits for people living with T1D. Our entire team is committed to unlocking new ways to re-educate the immune system to protect its insulin-producing cells. If we are successful we could change the outcome of a diagnosis of T1D, and this would be a dream come true.
>
> **Professor Ranjeny Thomas,** immunologist, University of Queensland, and consultant rheumatologist, Princess Alexandra Hospital

The trial will include adults and children with recently-diagnosed T1D, and Liperate Therapeutics is now raising capital investments to expand clinical trials of this technology. This offers great hope for transforming the trajectory of T1D and could provide a game-changing therapy for the T1D community. Without early support from the T1DCRN, it may never have reached this stage.

Other achievements to bridge the gap between basic science and s include the work of **Professor Josephine Forbes**, who is leading research into disrupting RAGE signalling to delay T1D progression and reduce kidney complications. Professor Forbes collaborates with another JDRF-supported researcher, **Professor Merlin Thomas**, who through the T1DCRN's support and translational stewardship formed a spin-off company called RAGE Biotech.

Additionally, **Professor Helen Thomas** (pictured above) at the St Vincent's Institute of Medical Research established a fee-for-service facility, Effica, for testing new therapeutics in T1D. Within just over two years, they have engaged with nine biotech and pharmaceutical companies, generating $1.6 million in revenue.

Effica serves as the pre-clinical domain for ATIC, supporting testing and facilitating progress in new immunotherapy development in Australia and New Zealand.

Leveraging JDRF's initial support, commercially focused research endeavours have attracted nearly $9 million in investments from venture capital funds, research funders, and industry collaborations. These efforts have a significant impact on building the capabilities of Australian researchers to bring therapies and technologies closer to the people who need them.

The rapid translation of research into clinical care lies at the heart of the T1DCRN's strategy and is key to treating, preventing, and ultimately curing T1D.

The T1DCRN recognises the need to create a strong community of researchers dedicated to improving the lives of people with T1D through translation, while also supporting other researchers in adopting best practices, connecting with industry, accessing funding, and influencing policy decisions.