Tomorrow’s medicine starts today
Since its creation in 2011, the Stem Cells Australia initiative has increased our understanding of how to control and use stem cells in research. Our members have placed Australia at the forefront of stem cell medicine, and now we are developing new diagnostic, therapeutic and biological applications that will transform healthcare in the years and decades ahead.

Our researchers are learning about how the heart forms so they can identify drugs to stimulate heart repair and improve function; they are analysing big data to predict how cells behave and create custom immune cells; they are helping patients with damaged corneas see again using grafts made from their own stem cells; and much more. Many of these achievements rely on large interdisciplinary teams from across Australia.

Our emerging clinical network has been crucial to support translation of new discoveries and fostering the development of an Australian stem cell biotech industry. Our work rests on three pillars of research and translational growth: Regenerative Medicine, Disease Modelling, and Designer Cells. We are building on our past research and expanding our network, which now involves more than 350 researchers across 14 leading universities and medical research institutes. Our members have wide-ranging expertise that spans developmental biology, bioethics, mathematics, computational science, bioengineering, pharmacology, clinical practice and law.

Another key element of SCA’s mission is training the next generation of Australian research leaders. With the support of a highly engaged committee of early career researchers we have provided unique learning opportunities for more than 200 trainees since our inception.

SCA recognises that the fast pace of stem cell research means informing and engaging the Australian community is also vital. We aim to provide accessible information about scientific progress, as well as address important ethical and regulatory implications of discoveries in regenerative medicine and stem cell research.

Stem Cells Australia has been supported since 2011 through the Australian Research Council Special Research Initiative Scheme.

We are proud of what we have achieved with support of the Australian Research Council to prepare the field of stem cells for a future in Regenerative Medicine, Disease Modelling and Designer Cells, both in Australia and internationally. As such, Stem Cells Australia will have a lasting impact on the future of medicine.

SCA Achievements (2011-2018)

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Stem cell medicine

Research capacity building
Ethics, engagement & policy
Clinical translation
Infrastructure
Entering clinical phase
Public outreach events

2 | Stem Cells Australia
Stem Cells Australia’s three pillars of research and translational growth address many important diseases and chronic conditions, including heart and kidney failure, blindness, stroke, leukaemia, Parkinson’s disease, MS, dementia and muscular dystrophy. They are also driving further fundamental research that is discovering completely novel ways to control stem cells and their properties, opening up new applications.

**THEME 1: Regenerative Medicine**

Seeks to develop new therapies by either recruiting stem cells within organs to promote repair, or administering new cells and tissues made from stem cells to restore normal function after disease, illness or injury.

**THEME 2: Disease Modelling**

Delivers greater understanding of development and disease, providing a platform for testing potential new drugs. Tissues created from patient-derived stem cells will drive tailored approaches to diagnosis, disease monitoring and treatments.

**THEME 3: Designer Cells**

Uses a combination of molecular tools to design and construct completely novel types of cells, built to deliver a specific function. For example, a universal cell that is engineered to be compatible for all transplant patients, or that produces therapeutic compounds only when given a specific command to do so.
Modelling brain circuitry

With the help of a revolutionary robot, Professor David Adams and Associate Professor Mirella Dottori are studying neurons, testing drug candidates for chronic pain, and working towards precise, personalised neurological treatment.

David has been studying the neurology of chronic pain, while Mirella is a neural stem cell expert. Based at the University of Wollongong, their collaboration focusses on cells called dorsal root ganglia sensory neurons. These cells sense pressure, temperature, position, touch and pain, and the duo believe they could hold the key to many neurological disorders including chronic pain.

“Many diseases and disorders are caused by altered firing of signals along sensory nerves. Growing human sensory neurons [from stem cells] means we can study their development and function in both health and disease,” says Mirella.

Measuring electrical activity in neurons can help to understand how ion channels function, which in turn is expected to open up new avenues of treatment for neurological diseases and disorders.

David and Mirella are using a SynchroPatch 384PE robot which can measure the electrical activity of as many as 384 cells at a time.

“There are only a few of these robots worldwide and it’s currently the most powerful way to study the activity of cells in real time. It not only measures responses to drugs, but also monitors and maps the activity of growing cells,” David says.

“We see this as a two-way street,” Mirella says. “David was moving into using human-derived cells, and I wanted to further test the functionality of the sensory neurons we generate from stem cells."

While Mirella and David are focussing on chronic pain for now, their larger goal is develop a platform to rapidly screen large numbers of drugs to deliver personalised treatments for people with neurological conditions.

“This technology is versatile,” Mirella says. “It could be applied to most neurological conditions.”

“We reached the limit of what we can discover in rat and mouse cells, so we needed to move into human cells.”

“I have been investigating chronic pain pathways in rodents for more than 20 years, but we have reached the limit of what we can discover in rat and mouse sensory neurons,” David says. “The research needed to move into human cells, and that is where Mirella’s expertise was vital.”
Fixing hearts by finding out what makes them tick

You can learn a lot about hearts by trying to build one from scratch. A pair of scientists have grown ‘beating’ human heart muscle tissue from stem cells and are exploring cardiac regeneration.

Developmental biologist Associate Professor Enzo Porrello became interested in how newborn mammal hearts can regenerate while working in Dallas, Texas at one of the leading labs researching heart development.

They’ve brought their skills together to create beating human heart tissue for cardiac research in the lab and developed patented technology to do it at scale.

They’re investigating the development of the human heart in detail, including the short-lived self-healing ability of newborn babies’ hearts.

“There are a small number of case reports of human newborns recovering from heart attacks, which is very intriguing,” says Enzo.

Their previous research with newborn mice shows that heart muscle cells lose their regenerative potential at the same time as they massively increase their force production. They identified the thousands of genetic changes that happen during this process. Then they identified the equivalent pathways in humans.

“We want to understand what happens in this window in time, firstly to engineer better tissues and secondly to come up with better therapeutic drugs for heart failure and regeneration,” explains James.

Their work building functional heart tissue from stem cells is also revealing how the cells work together. Enzo and James are making heart tissue with the right ratio of muscle and supporting cells, and that are integrated so they can contract in synchrony.

“Other cell types have a huge impact on the heart muscle cells, which was previously under-appreciated,” says James. “This is increasingly important as we get closer to creating tissues for drug discovery or transplantation into patients.”

Enzo now leads the Cardiac Regeneration group at Murdoch Children’s Research Institute and the University of Melbourne. James has moved to QIMR Berghofer. However, their research collaboration continues, with projects making tissues for drug screening, making ‘artificial pumps’ for children with congenital heart conditions, developing drugs to simulate heart repair or improve function, and broadly improving our understanding of the heart and how it works.

Heart disease is Australia’s leading cause of death. Each year, about 54,000 Australians suffer a heart attack, with an average of 23 deaths per day. “We’re trying to understand how the heart works in order to fix it, the same way a car mechanic needs to understand how a car engine works in order to fix it.” – James Hudson

Associate Professor James Hudson has a background in chemical and biological engineering. In Germany, he developed bioengineering techniques to make force-generating human heart tissue at the University Medical Center in Göttingen.

Enzo and James met when they both returned to Australia to work at the University of Queensland. It was a match made in scientific heaven.
Studying heart development one cell at a time

Examining how individual heart cells develop is revealing how they make decisions to form a working heart.

Once an adult heart is damaged, it has no ability to heal itself. Dr Nathan Palpant at the Institute for Molecular Bioscience at the University of Queensland and Associate Professor Joseph Powell at the Garvan Institute of Medical Research and the University of New South Wales are trying to understand how that might be changed by tracking individual stem cells along their journey to becoming heart cells.

"Heart development is a difficult and complicated process, but we think the answers to heart repair are likely to lie in understanding heart development," Nathan says. "So we are using stem cells to model development as it occurs in our bodies."

The development of a stem cell into a heart cell requires a series of complex changes, and changes in one cell affect the activity of others. By looking at individual cells along the path of development, researchers can learn how different types of cells are made as they work together to build the heart. This, in turn, can reveal what goes wrong in conditions such as heart disease.

The standard sequencing experiments for studying genetic material use tissue samples from multiple cells for analysis. This means rare cells can’t be studied and variations between cells might go unnoticed.

Single-cell sequencing, however, analyses how genes function in an individual cell. It can reveal rare cell populations, uncover how genes might switch each other on and off, and track the development of distinct "families" of cells.

"When we began this project in 2016 we were the only lab in Australia, and the third in the world, to have a high-throughput single-cell sequencing machine," says Joseph.

Nathan and Joseph believe that research collaborations like theirs are vital and valuable.

"We think the answers to heart repair are likely to lie in understanding heart development."

"My lab focuses on stem cell biology and cardiovascular disease development; Joseph works on statistical genetics and single-cell sequencing. Neither of us could do this research alone," says Nathan.

Joseph agrees. "We can now address questions that were inaccessible before."

In 2018 the pair found that a gene called HOPX plays a crucial role in controlling heart growth. Their next step is to zoom in even further on cellular development, to study the genetic activity that happens in the nuclei of individual cells.

"This is a resolution of information we’ve never seen before," says Nathan. "The questions we could answer are almost limitless."

"Stem Cells Australia"
Big data points the way to custom stem cells

Genomic biologist Professor Christine Wells and biostatistician Dr Kim-Anh Le Cao are analysing big data to discover what makes stem cells tick. Already the pair have found new ways to classify stem cells, and they’re working to predict the cells’ behaviour and even create custom immune cells.

Christine directs Stemformatics, an online encyclopaedia of hundreds of high-quality stem cell studies from other researchers vetted and archived by Christine’s team. They’ve amassed an enormous amount of data about genetic activity in certain stem cell types at many stages of development.

To find trends across the studies, Christine called on Kim-Anh’s statistical expertise.

“The partnership with Kim-Anh became very exciting because she brought a very creative and rigorous approach,” Christine says.

Kim-Anh’s solution was to develop a new, purely statistical way to compare the studies in the Stemformatics catalogue.

“We asked: what is the common information between all these different data sets?” says Kim-Anh.

They found that a kind of adult stem and progenitor cells called mesenchymal stromal cells (MSCs) share a unique set of genetic molecules regardless of which tissue they have come from. This allowed them to be distinguished from other, similar cells in the tissue they live in.

“The path to stem cells that can reliably regenerate tissues is to first understand the design principles that make any cell what it is.”

“It was the first time that anyone attempted to bring all these disparate data sets together and we could find a very reproducible signature of these MSCs,” says Christine.

The next step, predicting the behaviour of the cells, requires looking at their protein signatures as well as their genetics. Protein data is often scarce, but Kim-Anh has found ways to identify correlations between genomic and protein signatures that allow her to “fill in” missing protein information.

Christine and Kim-Anh’s collaboration is slowly revealing the design principles that make specific cells what they are, which raises the possibility of making hybrid stem cells that combine features from different kinds of existing cells.

For example, Christine’s laboratory is currently working on lab-made macrophages, the immune cells that eat up cellular garbage, invading germs and cancer cells. The designer macrophages could perform new functions such as making and delivering medicines.

“The next generation of cells for regenerative medicine will be made for specific purposes. We’re moving away from using existing cells to actually designing what we need,” says Christine.
Clearing corneas and restoring vision

The eye’s cornea depends on stem cells to help maintain transparency. If disease or trauma depletes stem cell reservoirs, a rapid and painful loss of vision soon follows.

Professor Stephanie Watson and Professor Nick Di Girolamo have used stem cells to repair their patients’ vision. It’s the culmination of a 15-year collaboration to restore sight in Australians with corneal disease.

Stephanie is an international leader in research and innovation with the University of Sydney and a practising corneal surgeon. She met Nick as an early career scientist through a research group at the University of New South Wales and they discovered their shared interest. Nick is now a Director with the School of Medical Sciences at UNSW.

Working together, Stephanie and Nick’s teams have transplanted healthy stem cells into patients’ damaged eyes. In initial trials the cornea was restored in six out of ten patients and 80 per cent also experienced better vision. They are now improving the treatment and developing protocols so that the procedure can be offered to more patients.

Stephanie sees collaboration as crucial to helping more patients than just the ones she treats as a surgeon.

“Fifteen years ago, I was seeing lots of patients with corneal scarring and stem cell deficiency and I didn’t have a way to help them. I was very lucky to receive an NHMRC Research Fellowship, which gave me the opportunity to work with Nick,” she says.

“I’ve always hated having to say to patients ‘Medicine can’t help you at the moment.’”

“I can identify what patients need and Nick brings the technical laboratory skills and deep understanding of stem cells. We have complementary skills but a shared vision.”

Stephanie has worked with colleagues at Stem Cells Australia, the Ophthalmic Research Institute of Australia, the Royal Australian and New Zealand College of Ophthalmologists, and patient groups to raise awareness of the potential of stem cells and to determine funding priorities relevant to patients.

“We want treatments that have real benefits to patients and to make sure that whatever we do is about achieving outcomes patients care about,” she says.

While they have achieved profound results, Stephanie says that the funding environment in Australia is challenging. There are also clinics offering stem cell treatments that are risky and not clinically proven.

“With stem cells there’s a lot of hope but there are also hoaxes. It’s important that people considering treatments can easily find credible information to make sure they’re part of legitimate trials and are not paying for unproven stem cell treatments,” she said.

“We have to collaborate at multiple levels—from doctors and scientists to governments who regulate, to patients and the community as a whole—to really take advantage of what stem cell medicine can offer in its best form,” she says.
Mini-kidneys tell two sides of a genetic story

Gene-editing technology combined with stem cells provides a powerful new way to study genetic kidney diseases and their treatments.

Melbourne researchers have used mini-kidney ‘organoids’ grown in the lab to unravel the mystery of why Mainzer-Saldino syndrome, a rare disease involving a single defective gene, causes life-threatening kidney damage. In doing so, they’ve proven an approach that can be used to study a whole range of other genetic kidney diseases.

The researchers took skin cells from a child with the disease, turned them into stem cells, and used CRISPR gene-editing technology to correct the mutation that causes the condition. This multidisciplinary effort was led by organoid pioneer Professor Melissa Little and her Kidney Regeneration group at Murdoch Children’s Research Institute, located on the grounds of the Royal Children’s Hospital in Melbourne.

“When you create reprogrammed stem cells from a patient’s own skin cells you carry across the patient’s whole genetic fingerprint,” explains paediatric nephrologist and PhD candidate Dr Tom Forbes. “A kidney organoid made from those stem cells behaves under the influence of that patient’s genes.”

They grew mini-kidneys from both diseased and gene-corrected stem cells, providing the perfect experimental control for studying the condition.

The defective gene, they found, caused malformation of cilia—finger-like cell projections that detect movement of liquid past the surface of the cell—which are vital for the normal function of kidney cells.

Group member Dr Sara Howden used a new method she invented to reprogram patients’ cells to stem cells and gene-edit to correct mutations at the same time.

Chronic kidney disease costs Australia’s economy $4.1 billion each year and is one of our biggest killers. In many cases the cause is genetic. It is estimated that one in ten Australians will show evidence of chronic kidney disease by 2020, but only one in four patients will receive a transplant.

Further information:

Stem Cells Australia

Project leader Melissa Little previously produced the world’s first mini-kidneys in a dish, grown from stem cells that were able to produce several different cell types and self-organise into the complex tissues of a functioning kidney.

Ultimately, Melissa’s group wants to make organs for transplantation from stem cells. Along the way, they expect their regenerated kidney tissues will improve the understanding, diagnosis and treatment of kidney diseases, and help identify life-extending drug treatments.

This gives Tom great hope for his patients who are facing a daunting future of dialysis or a kidney transplant.

“If these diseases can be detected through genetic testing and addressed before symptoms appear, it can save patients a lot of suffering and reduce the costs to our healthcare system.”
People suffering from serious illnesses are turning to unproven and risky stem cell therapies in growing numbers. Researchers are trying to understand why—and how to provide better information and support.

Stem cells have been saving lives for decades, largely through bone marrow and cord blood transplants treating leukaemia and other blood diseases. Unproven treatments are booming, however, with clinics in Australia and around the world spruiking cures for conditions from osteoarthritis and MS to dementia and diabetes.

Associate Professor Megan Munsie and her colleagues in Stem Cells Australia's Engagement, Ethics and Policy Program have heard many tales of patients spending thousands of dollars on treatments that often have no benefit and may be harmful or even deadly. “While some providers sincerely believe they can help patients, what’s often advertised can have the hallmarks of the perfect con,” says University of Melbourne health sociologist Dr Claire Tanner, who has teamed up with Megan and sociologist Professor Alan Petersen at Monash University to come to grips with the problem. “If the treatment fails, patients blame themselves or think their body has failed them.”

The team set out to gather evidence by talking to people who had considered or tried stem cell treatments, as well as their families. The stories of patient harm, exploitation and confusion they collected formed a key part of Stem Cells Australia’s submission to the Therapeutic Goods Administration review of regulations for stem cell treatments.

“Our interviews with patients provided solid evidence for policymakers. We showed that there really is a problem,” Claire says. The research also uncovered less obvious problems, such as unscrupulous clinics coaching patients on crowdfunding to meet exorbitant prices and how some health professionals are wary of advising patients on stem cell treatments because they feel they are not well informed themselves.

The team are working with doctors and patient groups such as Musculoskeletal Australia, MS Australia, MND Victoria and the Chronic Illness Alliance to understand how best to support patients. Support might include resources for GPs and other healthcare professionals, as well as tailored patient information provided through community groups and via websites and phone services.

Navigating the space where science and medicine overlap with regulations and social forces has required a wide range of skills. “That’s the strength of our interdisciplinary collaboration. Megan has a strong scientific background, as well as experience in bioethics, science communication and public engagement. Alan’s expertise is in sociology and mine is in gender studies and health sociology.”

Along with their collaborators Chinese Studies scholar Dr Jane Brophy and anthropologist Dr Casimir MacGregor, this group recently received a prestigious prize for their book on stem cell tourism.

What’s the harm?

Alistair, a father with a teenage son with a spinal cord injury, had placed a $12,000 deposit with a clinic in Mexico before hearing of fraud allegations with the clinic’s doctor. He told researchers of his frustration with the lack of warning from medical professionals in Australia. “Our interviews with patients provided solid evidence for policymakers. We showed that there really is a problem,” Claire says.

The research also uncovered less obvious problems, such as unscrupulous clinics coaching patients on crowdfunding to meet exorbitant prices and how some health professionals are wary of advising patients on stem cell treatments because they feel they are not well informed themselves.
Building tools for brain repair

Professor James Bourne and his team are laying the groundwork for using stem cell transplants to treat brain trauma with the discovery of an anti-scarring agent and new biomaterials to support transplanted cells.

“What we’re doing is a prelude to direct stem cell research. We hope to give potential stem cell therapies for brain trauma the best chance of success,” James says.

He and his team at the Australian Regenerative Medicine Institute at Monash University are studying nonhuman primates to understand how to create the best environments for repair after brain injury.

Brain injury—particularly stroke—is a serious health issue. Some estimates suggest that an Australian has a stroke every ten minutes, and many thousands of people are left with some form of disability afterwards.

Stem cells are currently under investigation as a tool to repair brain damage from strokes, trauma and other diseases. James is interested in what enables repair in the first place, to help ensure that cells transferred into a damaged brain can thrive.

“After an injury, and stroke in particular, the environment in the brain is really harsh. A stroke is caused when brain tissue doesn’t get enough blood and damage begins immediately. Injured brain cells release toxins and then inflammation, swelling and longer-term scarring cause further damage. Simply adding stem cells into this environment might not do any good, because they are likely not to survive,” says James.

“You wouldn’t plant flowers in a bed of sand and just hope for the best. They need water, fertiliser and support to grow, and we think the same will apply to cell implants. As well as trying to understand what happens in the brain once it is damaged, we are investigating materials to provide a scaffold for the cells being implanted.”

Scarring can stop repair processes before they even begin. James and his group have discovered a molecule that is present in infant’s brains that minimises scarring.

“We’re trying to improve recovery from brain injuries by using this molecule to make adult brains behave more like infant brains,” says Dr Leon Teo, a postdoctoral researcher in James’ group.

The team are currently working to demonstrate the effectiveness of their approach in patients undergoing neurosurgery. If this is successful, the next step is to connect with Associate Professor Clare Parish at the Florey Institute of Neuroscience and Mental Health, who is developing stem cell transplants to treat brain injuries.

“We believe our tools can help those cells survive and do their work,” says James.
Micro-lenses bring new cataract treatments in sight

Stem cells are being used to rapidly test and improve treatments for cataracts, thanks to an innovative solution developed by Dr Michael O’Connor and his team from Western Sydney University.

With novel stem cell technology, Michael has created hundreds of thousands of micro-lenses similar to the ones in the human eye. These micro-lenses offer a way to rapidly improve drug research and offer the potential for lens cell transplants in the future.

As well as identifying drugs that prevent or limit cataract formation, in future it may be possible to transplant lens cells to restore lens transparency in patients. This would be particularly beneficial for children, Michael says.

“Treating kids with cataracts is much more challenging than treating adults,” he says.

“Surgery is more difficult. It takes longer to complete the treatment—up to 10 years—and the vision outcomes are often much worse.”

“This is why I work with Megan Prictor from Cataract Kids Australia. We see lens cell transplantation as a huge potential opportunity for better treating childhood cataract to benefit children and their families,” he says.

Megan agrees, saying many families face great difficulties from when the baby is tiny.

“We see multiple surgeries and very challenging care regimes post-surgery and often the vision outcomes are poor. Treatment methods for congenital cataract haven’t changed in years. We urgently need new approaches,” she says.

Billions of dollars are spent each year around the world on cataract surgery, and hundreds of millions more treating resulting complications.

Finding drugs to treat cataracts or slow cataract development would be a significant improvement.

Michael’s human micro-lens technology does away with the need for animal lenses in research and drug screening, meaning more relevant results and fewer animals used.

Michael and Megan were brought together by Stem Cells Australia, which has also provided funding to test whether or not light-focusing lenses can be regrown in the eye after transplantation.

Collaboration with researchers and others around the world has been crucial for Michael’s work. He is currently working with a US-based company to test potential anti- cataract drugs. He also collaborates with cataract surgeons, including Professor Stephanie Watson who has had success in patients with corneal damage using a different type of stem cell.

“There is a lot of interest in furthering this technology. Industry and the community can clearly see the benefits,” says Michael.

“I’m excited about the potential to give children and adults with cataracts better vision outcomes. Being able to identify anti- cataract drugs could fix this problem for many patients and negate the common side effects of surgery, particularly poor vision,” he says.
Specialist cleaning cells in the brain play a key role in neurodegenerative diseases, and they may also hold the secret to new treatments for the likes of MS and Alzheimer’s.

Professor Colin Pouton and his team at the Monash Institute of Pharmaceutical Sciences found a way to isolate microglia, the immune cells of the brain, from stem cells. They also made the cells fluorescent so their activity can be tracked, opening up new avenues of research.

Professor Trevor Kilpatrick and his colleagues at the Florey Institute of Neuroscience and Mental Health think Colin’s engineered cells could be the key to creating a revolutionary treatment for multiple sclerosis.

Trevor, a clinical neurologist who leads work on MS at the Florey and the Royal Melbourne Hospital, has been studying a molecule produced by microglia called MERTK. This molecule latches on to dead cells and debris in the brain and triggers microglial cells to clean them up.

“This clean-up is a necessary first step to let the body’s own repair mechanisms get to work.

“Even though the microglia doesn’t directly coordinate the repair—other cells regrow the myelin sheaths on nerve cells that are destroyed in MS—the repair won’t work properly unless the debris has been cleaned up,” Trevor says.

Trevor’s studies have shown that MERTK function may be corrupted in a significant proportion of people with MS. Injecting molecules that stimulate MERTK seems like a possible treatment, but the molecules are too large to cross the blood-brain barrier.

Instead, Trevor’s team are hoping to create MERTK-producing microglia and inject them into MS patients. The theory is that the injected cells will migrate to the brain and help the body fight off MS.

At present the research is studying the effects of microglial transplants in animals, but the next step will need human cells—and that’s where Colin’s cells come in.

“Colin’s group are already characterising the activity of their stem-cell derived microglia, detailing how well they take up debris. Once we’re confident that the cells are behaving the way we think they will, we can try to establish a proof of principle for tests in humans.”

And it’s not only in MS where microglia are significant—Colin is studying their role in other neurodegenerative diseases such as Alzheimer’s and Parkinson’s.

“The first thing that disappears in degenerative diseases is functional synapses, the connections between neurons. When cells make fewer contacts with other neurons, they go into programmed cell death and once that starts there’s no going back. We’re interested in that initial process of loss of synapses, and microglia are right in amongst it,” Colin says.

“Microglia seem to be involved in most of the degenerative diseases, but it’s only recently we’ve been able to study them.”

Enlisting the brain’s immune cells to fight MS
How reprogramming cells turns back time

For the past decade scientists have been able to reprogram skin cells, nasal cells and other mature cells to become pluripotent stem cells that can turn into any cell type in the human body. How it works is only starting to become clear.

Teams led by Professors Ryan Lister at The University of Western Australia, Jose Polo at Monash University and Ernst Wolvetang at The University of Queensland are working together to understand how this process occurs, whether all cell types follow the same path to becoming pluripotent cells, and if this impacts their ability to mimic disease in the laboratory.

Through a series of collaborations over the last ten years the scientists have uncovered a number of stem cell secrets, opening the door for more targeted research and, ultimately, treatments for diseases.

“Undertaking these complex research projects has only been possible by collaborating,” says Jose.

“Working together we have answered questions that otherwise would have been impossible or taken far longer to answer.”

Ryan agrees. “By combining the distinct scientific techniques that our different laboratories specialise in—which range from manipulating and interrogating cell identity and function, to mapping features of the genome and sophisticated computational analyses—the whole can be bigger than the parts.”

The laboratories are spread across the country but they share information, ideas and reagents and connect online and face-to-face thanks to support from Stem Cells Australia.

Progressing stem cell science as quickly as possible is of particular interest to Ernst who works on human diseases and is involved with patient advocacy groups.

“I want to understand how mutations in genes or alterations in gene copy number lead to diseases, especially those that affect the brain, and how a decline in stem cell function contributes to ageing,” said Ernst.

So far, the teams have made several discoveries related to neurodegenerative diseases including leukodystrophies, Rubinstein-Taybi syndrome and Down syndrome, identifying new potential treatments and providing insights into the cell types and processes that underlie Alzheimer’s disease.

While they have taken big steps, particularly over the last ten years, towards understanding the paths that cells take towards pluripotency and disease, all three researchers agree there is still a lot of work for them to do together.

“Human pluripotent stem cells offer a powerful model system for understanding the molecular processes that cause disease.”

Ryan and Jose have deciphered how specialised cells such as skin cells are reprogrammed and found that the ‘roadmap’ for change is different in different cells. This means that scientists are one step closer to formulating consistent reprogramming processes in different cell types.