

# research appeal

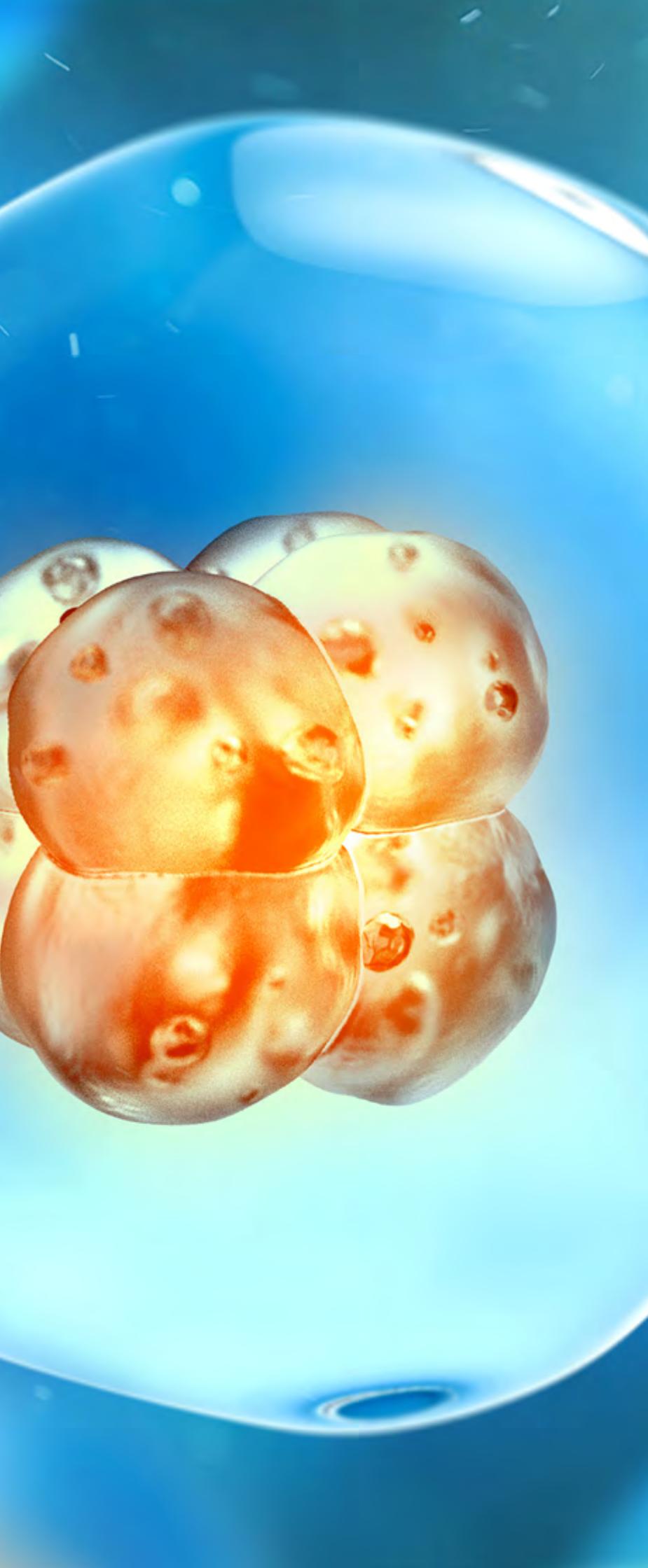
A PROSPECTUS FOR PHILANTHROPIC INVESTMENT



National  
Stem Cell  
Foundation  
of Australia

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# Stem cell research and its potential

Stem cell medicine is already transforming the lives of countless patients worldwide but there is so much more we need to learn if we are to harness its full potential.

We only have to look to the tens of thousands of people each year whose cancerous bone marrow has been returned to health by stem cell transplantation to realise the potential of stem cells for creating a better future for those suffering from disease. With the majority of these patients being children, it is fair to say that stem cell research is giving an entire generation opportunities they once didn't have, namely, the ability to grow up healthy and live full lives, free from disease.

In fact, when we look to the future of stem cell research, we know that the discoveries made today will make the biggest difference for the next generation. It's for their sake that we must continue to fund the future of scientific discoveries.



## A message from the Chairman

**DR GRAEME L BLACKMAN**  
AO FTSE FAICD

Today, you have the opportunity to make history.

The National Stem Cell Foundation of Australia (NSCFA) is launching an appeal to provide funding for two promising stem cell research programs.

Australia is leading the way in stem cell medicine and the projects we have selected for funding are at the cutting edge of medical research. Breakthroughs made in chronic lung disease in premature babies could not only save lives today, but shape the future of stem cell research around the world for years to come. It's an exciting time in stem cell research and one that I hope you'll play a key role in as a donor, supporter, and most importantly – visionary.

I'm asking you to consider investing in the future of this research. You can do this by helping to meet the demands of these projects over the next five years. Your donation – which can be a one-off gift, or an amount pledged over the course of that 2018-2022 period – will be used to ensure vital clinical trials can go ahead; giving researchers the answers they need to save lives.

Any gifts made as part of this appeal will be matched dollar-for-dollar by the NSCFA up to a total value of \$2 million over the entire appeal; doubling the impact of your generous gift.

The two projects – which you can read more about in the following pages – are being conducted in conjunction with the Hudson Institute of Medical Research, and led by some of Australia's most experienced stem cell researchers. Both projects are at clinical trial stage, with patients, researchers, and staff ready to begin work.

All they need now is you. I hope you'll join us.

# About the foundation

Australia excels in stem cell research. The National Stem Cell Foundation of Australia (NSCFA) is dedicated to progressing cutting edge stem cell science through supporting promising researchers and projects, and educating the public about responsible stem cell treatments.

The NSCFA is a philanthropic foundation established in 2011 to continue aspects of the stem cell mission previously undertaken by the government-funded Australian Stem Cell Centre. After that organisation's closure, the NSCFA worked to establish new governance, staff, and administrative systems, and develop a unique brand, marketing, and fundraising strategy.

The NSCFA board is drawn from diverse backgrounds in stem cell science, medicine, and finance. With their voluntary assistance, the Foundation has developed an investment strategy for residual funds transferred from the Australian Stem Cell Centre.

Each year the NSCFA supports early-career stem cell researchers by funding their attendance at national and international scientific meetings. The Foundation has also provided funding to a team at the Royal Eye and Ear Hospital working on stem cell treatment for an incurable form of blindness called macular degeneration.

In 2014 the NSCFA launched the Metcalf Prize, named in honour of the late Professor Donald Metcalf AO whose research enabled the development of bone-marrow transplants. This stem cell treatment has saved the lives of countless people around the world suffering from blood cancers. The Metcalf Prize is awarded each year to two stem cell researchers (one male and one female) who are working in Australia. It is judged by an independent scientific panel and valued at \$50,000 to each researcher. Our eight Metcalf Prize winners are already showing the difference that this funding makes, with each having made breakthroughs in their respective areas of stem cell research.



Kaylene Young  
2014 Metcalf Prize  
female awardee

## OUR GUIDING PRINCIPLES ARE TO

- be the major non-government funder of stem cell research, providing seed funding for early trials so they may reach final clinical stages;
- support Australian stem cell treatment and regenerative medicine aimed at curing currently untreatable diseases;
- identify and support research projects that urgently translate to improvements in patient care;
- commit to educating the Australian public about the importance of stem cell research;
- support early career stem cell researchers to speak at conferences overseas, and in Australia; and
- partner with our other stakeholders to progress our mutual goals, values, and philosophies.

## OUR GREATEST OPPORTUNITY

Through the careful management of our financial resources we have been able to build a capital fund that now allows us to make significant grants to promising research in stem cell treatments. In fact, the Foundation Board has agreed to release up to \$2 million from our capital fund over the next five years in matching grants, meaning the potential exists for up to \$4 million to be directed towards vital stem cell research, with the support of partners like you.

We believe this is a unique opportunity; not only to fund two potentially game-changing stem cell projects, but to more broadly raise awareness for the cause of stem cell research in Australia.

## THE NATIONAL STEM CELL FOUNDATION LEADERSHIP

An expert team leads the NSCFA. Board members come from diverse backgrounds in stem cell science, medicine, and finance.

### NON-EXECUTIVE DIRECTOR, CHAIRMAN

**Dr Graeme Blackman** AO, PhD, BSc (Hons), BD, MTheol, FTSE, FRACI, FAICD, FloD.

Dr Blackman is a former Professor of Pharmaceutical Chemistry at the Victorian College of Pharmacy and former Chairman of IDT Australia Ltd.

### NON-EXECUTIVE DEPUTY CHAIRMAN

**Emeritus Professor Graham Macdonald** AM, MD, BS, BSc (Med), FRACP, FRCP, FANZCC.

Deputy Chairman of the Foundation, Professor Macdonald, was formerly chairman of the Australian Stem Cell Centre.

### NON-EXECUTIVE DIRECTOR

**Emeritus Professor Richard Smallwood** AO, MB, BS, MD, FRACP, FRCP, FACP (Hon).

Professor Smallwood is Emeritus Professor of Medicine, University of Melbourne, former Chair of the National Health and Medical Research Council, and Professor Smallwood was Chief Medical Officer of Australia from 1999 to 2003.

### NON-EXECUTIVE DIRECTOR

**Mr David Collins** MEng, MBA, CA, GAICD, MIET.

Mr Collins was Chief Executive Officer of the Australian Stem Cell Centre from September 2009 until November 2011.

### NON-EXECUTIVE DIRECTOR

**Mr Stuart Gooley** BComm (Melb) FCA, FCPA.

Chairman of the Finance, Audit, and Risk Management Sub-committee, Mr Gooley had a 42 year period in the accounting profession with Arthur Andersen and Ernst & Young.

### NON-EXECUTIVE DIRECTOR

**Professor Caroline Gargett** BAppl Sci, M Appl Sci, PhD.

Professor Gargett is currently Deputy Director (Women's Health) at the Ritchie Centre, Research Group Head Endometrial Cell Biology, and a National Health & Medical Research Council (NHMRC) Senior Research Fellow.

### EXECUTIVE OFFICER

**Ms Julia Mason** BA & BComm (Melb), Grad. Dip. Applied Finance and Investment, MBA (Chicago), GAICD, F Fin.

Julia has a more than 20 year career in the private and not for profit sectors in both Australia and Asia and has held several directorships.

### COMPANY SECRETARY

**Mr Graeme Mehegan** BBus(Acc), FCPA, FCIS.

Mr Mehegan was Chief Financial Officer for the Australian Stem Cell Centre from August 2009 until November 2011, and prior to that held senior finance roles in book publishing and consumer electronics organisations.

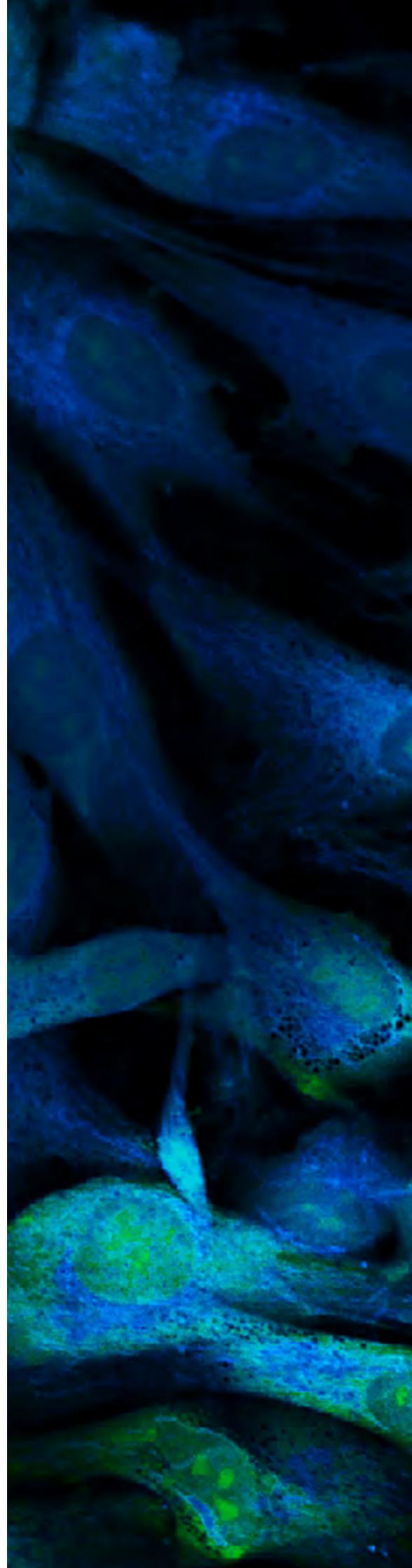
# What are stem cells?

Stem cells have been part of us since birth. They are in our hair, skin, intestine, bone marrow, brain, joints, and muscles. As we grow through childhood and adolescence, stem cells generate new tissue and when we have stopped growing, they replace damaged and aged tissue. It is this ability to repair damaged tissue that makes stem cells so unique, and their applicability in modern medicine so far-reaching.

It is no stretch to say over the next few decades, as we are able to learn more about stem cells, we will see them become part of many modern medical treatments; giving hope to many facing currently untreatable and often life-threatening illnesses.

Key areas of research for future therapies include:

- stroke and traumatic brain injury
- blindness
- deafness
- baldness
- spinal cord injury
- lung and breast cancers
- chronic lung disease in premature babies
- acute heart damage
- motor neurone disease
- liver cirrhosis
- diabetes
- uterine disorders
- prostate disease
- muscular dystrophy
- osteoarthritis and rheumatoid arthritis
- genetic bone disease
- multiple sclerosis and other neurological disorders



## AUSTRALIAN RESEARCHERS ARE LEADING THE WAY

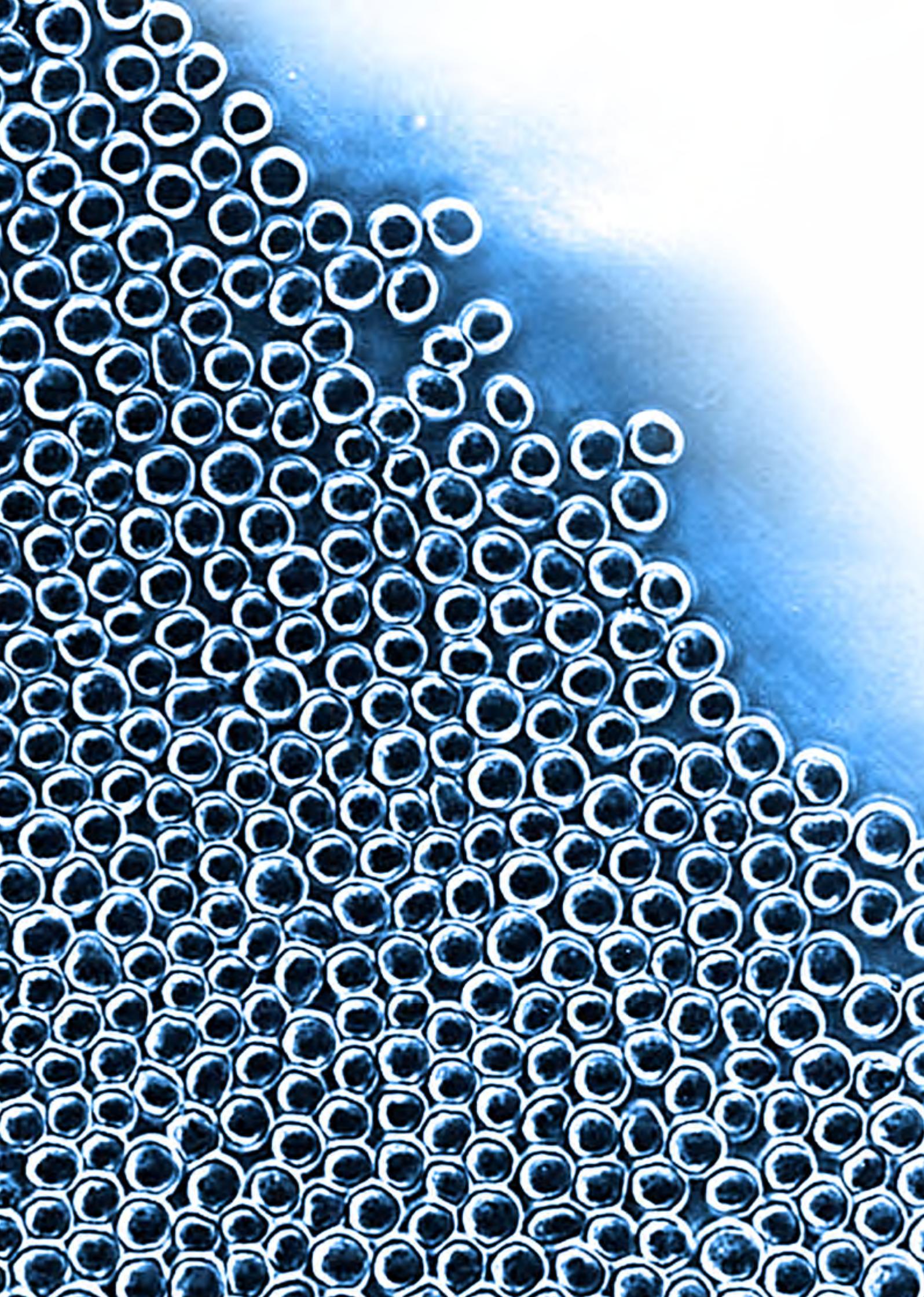
Australia is one of the global leaders in stem cell science and it is our job to keep it that way. However, our top scientists find it increasingly difficult to secure funding for their projects whether this be through Commonwealth Government funding and the National Health and Medical Research Council (NHMRC), State Government sources, or their own institutions.

Together we must work to grow the potential of stem cell research in this country and protect the future success of Australian stem cell research. We can do this by ensuring the best and brightest researchers and the most promising projects have the funds they need to thrive.

All too often, Australian scientists are lured away to other countries because they can't get the financial support needed to continue their research. Moreover, many potentially ground breaking research projects fail to ever achieve the results researchers know could be possible – due to lack of funding.

We must not let this continue to happen. Join us in funding the next phase of stem cell research in this country and help Australian researchers create the type of future we all know is possible.





# Promising research projects

Over the past few years the Foundation's Science and Ethics Committee has reviewed promising stem cell research projects from all over Australia to identify those worthy of further support.

The two projects chosen as the focus of this appeal have been selected because they are at stages where the first endeavours have been successful, and because they have the potential to change lives. In each case, the initial theories underpinning the project have already been proven; either in experimental studies or preliminary clinical trials, and the research is now ready to move onwards to the next stage where subsequent trials need to be funded.

Attracting funding for these next stages is crucial if we want to see this research progress. While funding for more advanced testing is easier to come by, with pharmaceutical companies and research centres likely to offer full support once all the groundwork has been done, these early stage trials in medical research are notoriously difficult for researchers to fund. With the funding we seek during this appeal we can help see these projects through to the next phase of clinical testing.

In fact, without the support of people like you, it is more than likely that each project would either fail to progress to the next stage or experience significant delays; leaving many crucial ideas untested and countless patients without hope of a cure and a better future, free from disease.

Together, we must ensure each of these projects goes ahead.

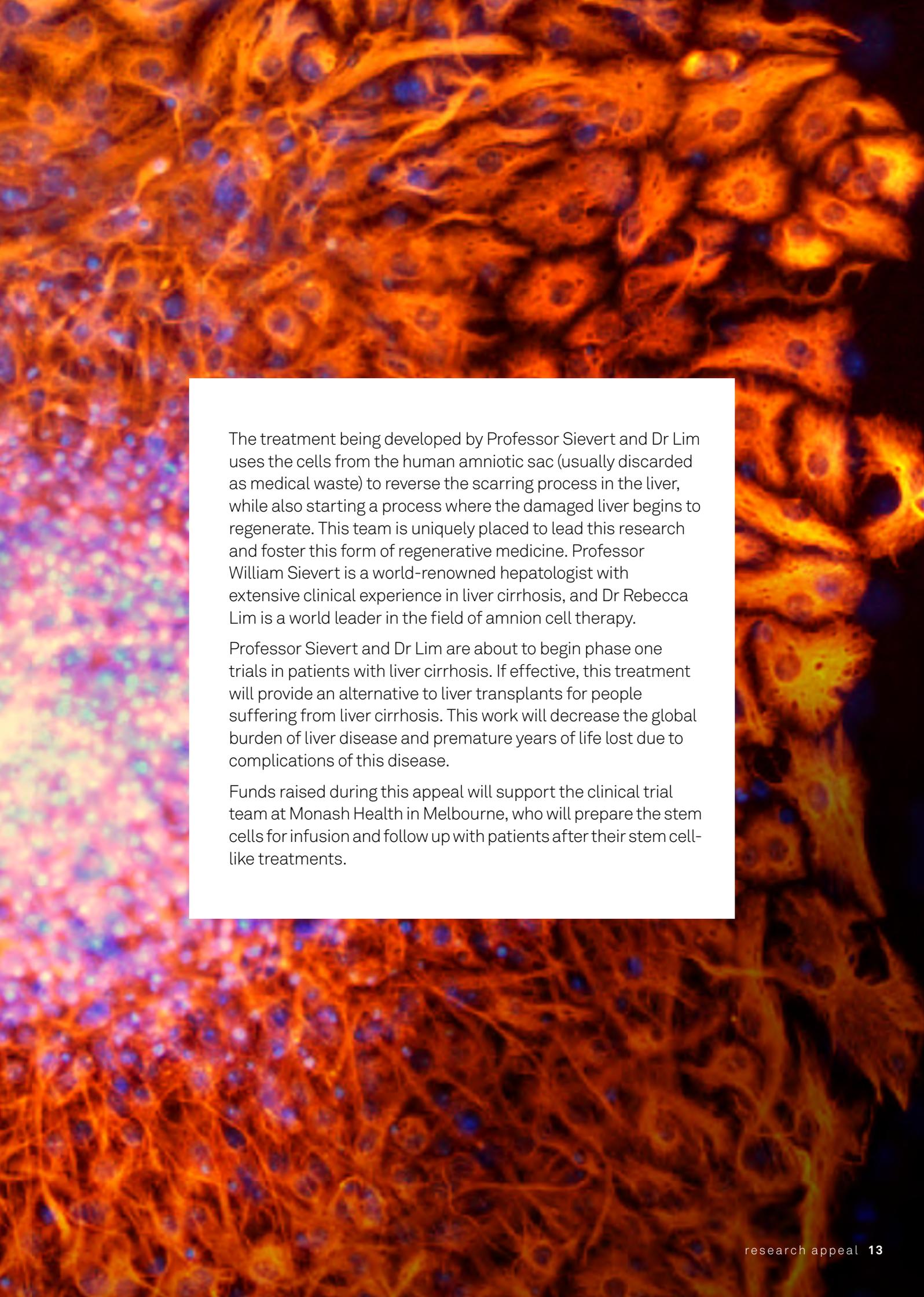
## LIVER CIRRHOSIS

### – PROFESSOR WILLIAM SIEVERT AND DR REBECCA LIM

Liver cirrhosis occurs when scar tissue replaces healthy cells in the liver, impacting a person's metabolism, their body's ability to clot blood effectively, and how their body filters drugs and toxins. This can be the end result of many disease processes, including hepatitis.

There is no cure for liver cirrhosis. Those in the advanced stages of cirrhosis face liver transplantation as their only option. The surgery is risky and the fortunate recipients require a lifetime of anti-rejection drugs. However, many patients die on the transplant list, waiting for their new livers, and there are patients who simply do not make it onto the transplant list due to other medical complications.



A microscopic image of liver tissue, showing a dense network of cells. The cells are stained with orange and blue dyes, highlighting their structure and nuclei. The background is dark, making the stained cells stand out.

The treatment being developed by Professor Sievert and Dr Lim uses the cells from the human amniotic sac (usually discarded as medical waste) to reverse the scarring process in the liver, while also starting a process where the damaged liver begins to regenerate. This team is uniquely placed to lead this research and foster this form of regenerative medicine. Professor William Sievert is a world-renowned hepatologist with extensive clinical experience in liver cirrhosis, and Dr Rebecca Lim is a world leader in the field of amnion cell therapy.

Professor Sievert and Dr Lim are about to begin phase one trials in patients with liver cirrhosis. If effective, this treatment will provide an alternative to liver transplants for people suffering from liver cirrhosis. This work will decrease the global burden of liver disease and premature years of life lost due to complications of this disease.

Funds raised during this appeal will support the clinical trial team at Monash Health in Melbourne, who will prepare the stem cells for infusion and follow up with patients after their stem cell-like treatments.



## BRONCHOPULMONARY DYSPLASIA (BPD)

### – PROFESSOR EUAN WALLACE AND DR REBECCA LIM, IN PARTNERSHIP WITH THE HUDSON INSTITUTE OF MEDICAL RESEARCH

Almost 1 in 10 babies in Australia are born premature and are often placed on essential and lifesaving respiratory support immediately after birth. An unfortunate side effect of this lifesaving care is that up to 60 per cent of these premature babies will develop a chronic lung disease called bronchopulmonary dysplasia (BPD).

Babies with this condition are highly susceptible to airway infections that may lead to death, as well as increased risk of cerebral palsy, and life-threatening diseases such as necrotising enterocolitis. The survivors of moderate-to-severe BPD remain at increased risk of cardiovascular disease throughout adolescence and adulthood.

Professor Euan Wallace and Dr Rebecca Lim are leading a research group that is using stem cell-like therapy to treat lung damage in premature babies. They have recently completed a first-in-human clinical trial, using amnion cells in six extremely premature babies. Thus far, the team has shown that the stem-like cells from the human amnion are safe. The team now plans to move into phase 2 dose-escalation trials with over 200 babies, across two of Melbourne's largest hospitals.

When the amnion cells are administered, they are attracted to the injured lungs and begin a repair process by reducing inflammation and activating the stem cells in the lung. This triggers a repair process and overcomes the stunting effect that oxygen supplementation and steroid treatments have on the premature lung.

If proven effective, amnion cell therapy can be used to improve the survival rates and quality of life of many premature babies.

Dr Lim has been the world's top-ranked researcher in her field of amnion cell research since 2010. (Her story is told later in this brochure). Professor Euan Wallace is one of Australia's most respected obstetricians having personally cared for 7,000-8,000 women during their pregnancies, as well as authoring more than 230 published research papers.

Funds raised through this appeal will be used to cover costs associated with running the next stage of clinical trials, for example, to hire research staff and research assistants.

## CAIDEN AND RACHEL'S STORY

It's been almost three years since Rachel Walter's son Caiden was born but she still remembers the exact moment when she found out, at 27 weeks and six days gestation, that he was going to make his entry into the world.

"[The doctors] weren't 100% sure if they were going to deliver him yet, perhaps just keep an eye on him. But all of a sudden, they decided it was time and that's when I got all emotional and started crying. It was too early," she recalls.

For this first-time mum, Caiden's birth was not the special moment she imagined; in fact, it was a full 24 hours before she even got to meet him. "He was just covered in wires and tubes," she says. "He wasn't allowed out of his incubator. It was 17 days before I got to have my first cuddle with him."

Like many premature babies, the combination of Caiden's under-developed lungs and the life-saving respiratory support he received at birth led to Caiden developing BPD, or chronic lung disease. As a result, he was in hospital for twelve and a half months, fighting a daily battle to survive.

At one point, as he was approaching ten months of age, he became so sick that his parents and doctors feared he wouldn't survive. It was at this moment that he became the first premature baby in history to receive stem cell-like treatment for his lungs.

"He was on death's door," Rachel recalls. "When we decided to go ahead with [the treatment], we really had no other choice; there was nothing else the doctors could do to help him so we thought we'd give it a go."

While in the next stage of BPD trials, babies will receive the treatment in their first few weeks of life – where it will have the most impact in preventing BPD from further developing – Dr Lim says Caiden was chosen to be the first recipient because all caring for him agreed that he was so sick, there was nothing to lose.

Although impossible to know if the treatment helped – due to Caiden's age and the fact that the ideal dosage was yet to be determined – his mum believed it did.

Caiden still continues to fight the effects of BPD today – as well as the other medical issues related to his premature birth – but continues to get stronger every month and is slowly developing into a healthy, happy, toddler. For Rachel – who could have lost her son so many times – that's reason to celebrate.





## REBECCA LIM: A RESEARCHER'S STORY

As the lead researcher on the focus projects of The National Stem Cell Foundation of Australia's appeal – both which utilise amnion cell therapy – Rebecca Lim has a keen interest in seeing funding get to where it is needed most – where it will save lives.

“If all it takes to make a difference is to validate some of these laboratory findings in the clinic, if this is going to take us closer to something that we can offer patients, then it would be a travesty not to.”

In the case of her BPD research, Rebecca says she's confident that the amnion cells will minimise the lung disease to the point where it no longer has lifelong implications for premature babies who contract the disease. And for those suffering liver cirrhosis, she sees the amnion cells as a viable alternative for the many who cannot access a liver transplant.

But she says outcomes like these are only possible if funding for clinical trials exists.

“I think the shame around the world, not just Australia ... is you get academics who have these great ideas with huge potential but due to lack of funding, these projects die an academic death. The discoveries never make it to the clinic where the real work begins. On the flip side, there are so-called stem cell clinics that charge patients a small fortune for unproven, and sometimes, unsafe treatments without appropriate clinical testing. It is crucial that we translate research findings into cures through clinical trials that are run with scientific rigour.”

Rebecca is confident that with support, these projects will not only help save lives but will “help open the floodgates” for further stem cell research around the world.

This is your opportunity to be a part of this important advancement in Australian stem cell research.

# From our partners



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**Director:** Professor Elizabeth Hartland

1 February 2018

Attention: Mr. Graeme Blackman

Dear Graeme,

Stem cell research has long been a priority for the Hudson Institute of Medical Research and sits at the heart of some of our most important discoveries. In the near future, regenerative science will play an even greater role in Australian research, within which, Hudson scientists will play a critical role.

It's promising to see such a significant step towards philanthropic funding in this area and I am excited about a future where the National Stem Cell Foundation of Australia plays a prominent role in championing stem cell research and regenerative medicine in this country.

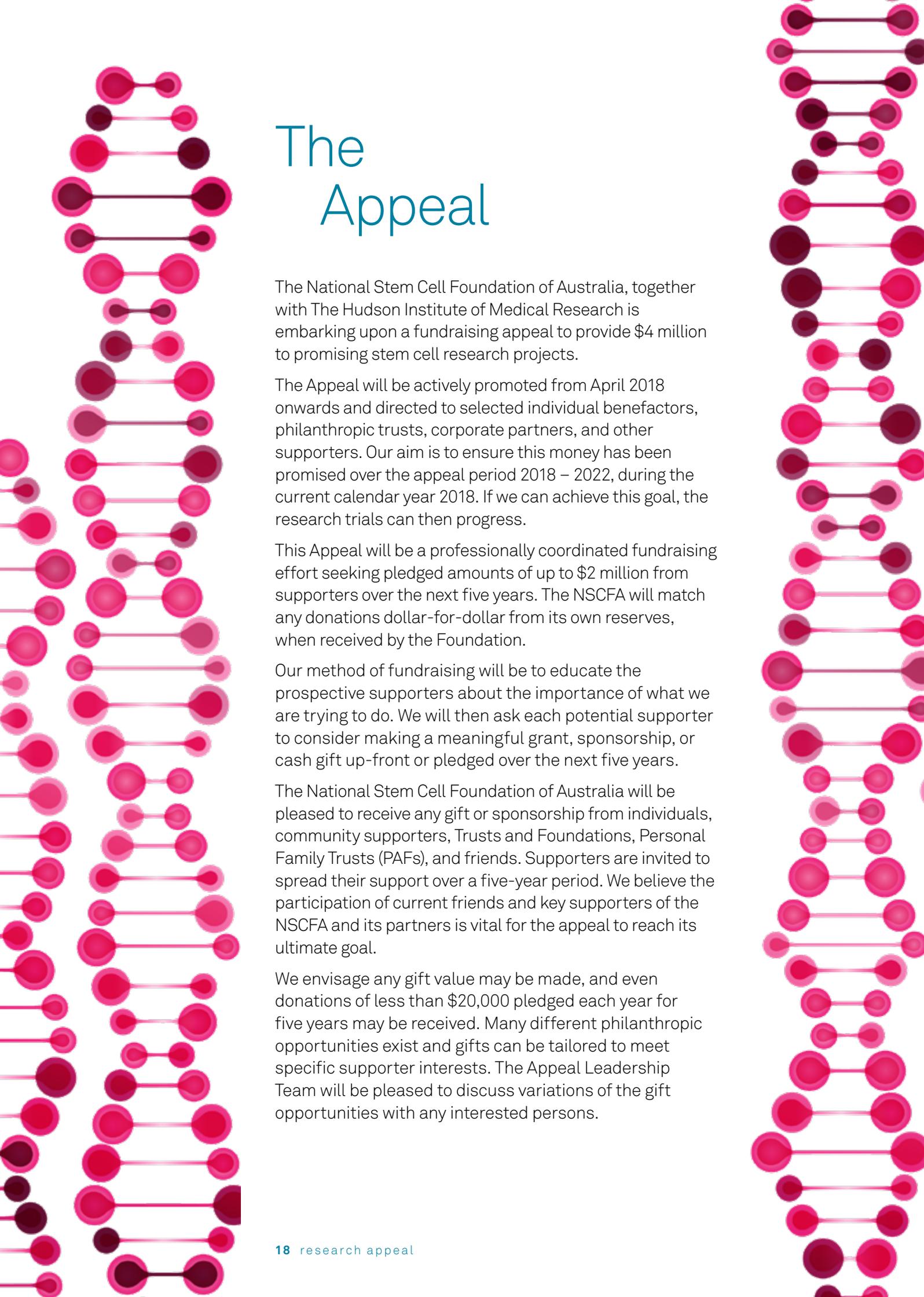
The Institute is proud to be associated with the NSCFA and to be included in this promising fundraising appeal. I commend the Foundation's directors for initiating the project and for their foresight and commitment to Australian researchers.

This appeal has the Hudson Institute's endorsement and my personal best wishes for its success.

Yours sincerely

A handwritten signature in black ink, appearing to be "E. Hartland", written over a light blue horizontal line.

Professor Elizabeth Hartland  
Director and CEO Hudson Institute of Medical Research  
Head, Department of Molecular and Translational Science  
Monash University



# The Appeal

The National Stem Cell Foundation of Australia, together with The Hudson Institute of Medical Research is embarking upon a fundraising appeal to provide \$4 million to promising stem cell research projects.

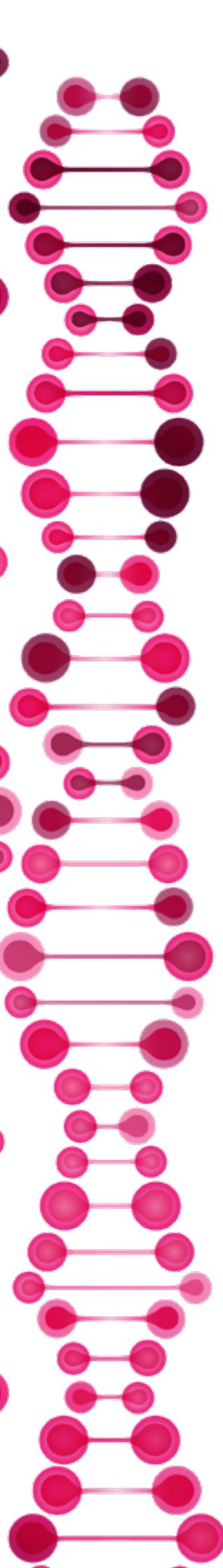
The Appeal will be actively promoted from April 2018 onwards and directed to selected individual benefactors, philanthropic trusts, corporate partners, and other supporters. Our aim is to ensure this money has been promised over the appeal period 2018 – 2022, during the current calendar year 2018. If we can achieve this goal, the research trials can then progress.

This Appeal will be a professionally coordinated fundraising effort seeking pledged amounts of up to \$2 million from supporters over the next five years. The NSCFA will match any donations dollar-for-dollar from its own reserves, when received by the Foundation.

Our method of fundraising will be to educate the prospective supporters about the importance of what we are trying to do. We will then ask each potential supporter to consider making a meaningful grant, sponsorship, or cash gift up-front or pledged over the next five years.

The National Stem Cell Foundation of Australia will be pleased to receive any gift or sponsorship from individuals, community supporters, Trusts and Foundations, Personal Family Trusts (PAFs), and friends. Supporters are invited to spread their support over a five-year period. We believe the participation of current friends and key supporters of the NSCFA and its partners is vital for the appeal to reach its ultimate goal.

We envisage any gift value may be made, and even donations of less than \$20,000 pledged each year for five years may be received. Many different philanthropic opportunities exist and gifts can be tailored to meet specific supporter interests. The Appeal Leadership Team will be pleased to discuss variations of the gift opportunities with any interested persons.



## WAYS YOU CAN SUPPORT OUR APPEAL

- make a five-year personal pledged donation;
- make a donation through your family trust; or
- include a gift in your Will towards the National Stem Cell Foundation of Australia.

The wishes of benefactors who do not want to be recognised publicly will be respected. As a benefactor in Australia, your gift is tax deductible and will contribute an amount that is nearly four times your “out of pocket” spend. The National Stem Cell Foundation of Australia will receipt and acknowledge all donations.

The NSCFA reserves the right to fully and finally approve all aspects relating to the acceptance, use, recognition, or other elements relating to any donations to this appeal.

## RECOGNITION OF SUPPORTERS

All families, individuals, and organisations making donations to the Appeal will be recognised. Donations will be acknowledged in Foundation and host research institute publications. We will name major supporters and sponsors (only with their consent) in any general media coverage. The Foundation will acknowledge the voluntary efforts and contributions of patrons and our Board, the research leaders, staff at the partner institutions, volunteers, and friends in helping with this important appeal.

Depending on the individual circumstances of the partner research institution, and with the permission of the supporters, the NSCFA may be able to recognise supporters with project naming rights, and on honour boards at the relevant institution.

The Foundation will ensure that benefactors receive regular reports and progress updates from the researchers they have supported. All contributors to this appeal will receive a special certificate of appreciation from the Foundation and will be recognised on the Foundation website and in other materials and reports produced.

Supporters will be invited to special recognition events hosted by the Foundation where we will acknowledge their generous contributions, and thank all those who have helped to achieve the goals of this vital appeal.

# Why it matters and how you can help

This appeal will not only ensure vital research goes ahead, it will help create opportunities for discoveries that will translate to better patient treatment and ultimately, save lives. That's not only important for today, but vital for the generations of our loved ones who will depend on us tomorrow.

Funding these projects will contribute to the advancement of Australian stem cell research; adding to the specialty skills and expert knowledge of our researchers and ensuring we are able to retain the best talent within Australia.

This is no longer an idea ... it is going to happen ... it is vital and your support is urgently needed.

Please contact us if you'd like to discuss your support or find out more about the vital research you'll be funding.

## **CONTACT:**

Julia Mason – Executive Officer  
National Stem Cell Foundation of Australia  
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*A gift to this appeal will fund research which can assist babies grow strong lungs and survive premature birth. This research may provide sufferers of liver disease with a better option for long-term health. Your support of this research appeal can help the families of patients to believe in a better future for their loved ones.*

**Dr Rebecca Lim**

*Within the next decade, we can expect that research into stem cells will lead to outstanding advances in relieving human and animal illnesses – untreatable in the present day. Perhaps more importantly, the understanding of cell function we gain from this research will open entirely new avenues of investigation in every field of medicine, biology, and even the way we think about the mind and its disorders.*

**Emeritus Professor Graham Macdonald AM**



National  
Stem Cell  
Foundation  
of Australia