Spring 2026

visionary

Challenging the inevitably of blindness

Changing Poppy's future

Double your donation with our Matched Giving campaign

CENTRE FOR Eye Research Australia | 30 years

Celebrating 30 years of CERA and accelerating research

This year, CERA celebrates 30 years of creating the future in sight. By challenging the status quo, we continue to drive breakthroughs that prevent vision loss and restore sight.

To mark this milestone, we invite you to support our annual **Matched Giving campaign starting on Thursday 8 October from 9am. Every donation will be doubled by a group of generous anonymous donors – up to our goal of $200,000 – with all funds supporting research into treatments and cures for inherited retinal diseases (IRDs).**

In this edition of Visionary, we explore IRDs and the research bringing treatments closer. Affecting around 19,000 Australians, these conditions are a leading cause of blindness in children and working-age adults. Once considered untreatable, IRDs are now the focus of promising gene therapy research aimed at slowing vision loss and restoring sight.

We also share the story of five-year-old Poppy and her family's journey with Usher syndrome type 2A, and spotlight Adara Bio's work to bring CERA-developed gene and RNA therapies closer to patients.

We also celebrate supporters like the Ruth Marie Sampson Foundation, whose investment in our work is helping launch a new research program for families affected by Usher syndrome.

Every donation made for our annual Matched Giving campaign will be matched up to our goal of $200,000, for us to continue to create a future where vision loss is no longer inevitable and sight can be restored for people with IRD's.

Together, let's challenge the inevitably of blindness.

With best wishes,

Professor Keith Martin
Managing Director
Centre for Eye Research Australia

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CERA gratefully acknowledges Australian Vision Research's support of our work and of Professor Keith Martin as Director of Research (Victoria).

Changing Poppy's future

Poppy's diagnosis of Usher syndrome 2A led her family to connection, strength and hope.

After their daughter Poppy was born, Ali and her husband Ben were happily focused on the joys of welcoming a new baby into their family as well as caring for their older daughter Evie. Then, when Poppy underwent a routine hearing screening and it revealed that something wasn't right, everything changed.

"The doctors kept saying it was probably fluid in her ear," says Ali.

"Then after more testing, suddenly we were being told she had mild to moderate permanent hearing loss and would need hearing aids for the rest of her life.

"As she still reacted to noises – like a door would slam and she'd startle – we thought, 'Well, she can hear, so everything must be fine.'"

While the family was adapting to Poppy's hearing loss, Ali and Ben began learning more about the challenges Poppy would likely face as she grew up, which led to a determination to understand what had caused it.

Genetic testing provided Poppy with a clear diagnosis: Usher syndrome type 2A. This rare inherited condition causes mild to severe hearing loss from birth and progressive vision loss from the teenage years.

The vision loss in people with Usher syndrome is caused by retinitis pigmentosa – an eye disease that begins as night blindness and tunnel vision in adolescence or early adulthood and progresses to blindness later in life.

(Continued Page 4)

While there is currently no cure for Usher syndrome, hearing aids and cochlear implants can significantly improve people's hearing. Meanwhile, ongoing research and clinical trials at CERA are actively investigating potential treatments to prevent or cure vision loss and blindness.

Facing the unknown

For Ali and Ben, the diagnosis brought a second wave of grief.

"We'd only just started coming to terms with the hearing loss when we found out she would also experience progressive vision loss," says Ali.

"Suddenly I'm thinking that my child is going to go blind. What does that mean for her future? What does that mean for our family? There were so many unknowns."

The symptoms of Usher syndrome type 2A vary considerably from person to person. While Poppy's hearing loss is expected to remain relatively stable, her vision is likely to steadily deteriorate over time. The timing and rate of vision loss vary widely and cannot be predicted accurately for any one individual.

In those early days, uncertainty fuelled fear for Ali and Ben.

"When you don't have information, your anxiety fills in the gaps," says Ali.

"Ben and I found ourselves imagining the worst-case scenarios and worrying about everything from education and independence to career opportunities and quality of life for Poppy."

But they soon realised that what helped most wasn't simply time to understand the condition and how it would affect Poppy's life. It was being a part of the Usher syndrome community and finding hope in the research underway at CERA to find a cure.

↑ *Bright future: Poppy and her big sister Evie.*

← *Strength in connection: Poppy with her mum Ali and her dad Ben.*

Why research matters

"We believe that every story told, every dollar raised and every piece of research becomes part of the chain that eventually leads to a cure for Usher syndrome. That's what community is about – supporting each other," says Ali.

Research breakthroughs don't happen overnight. They are built step by step, discovery by discovery, donation by donation. And as CERA's scientists work towards new treatments for Usher syndrome, Ali remains hopeful.

"You never know what will be the final piece of the puzzle that leads to a cure – whether it's funding research, participating in studies, sharing a personal story or raising awareness. Every contribution matters," she says.

For Ali and Ben, research offers something incredibly important: possibility and hope for future treatments to prevent vision loss, slow its progression and, ultimately, restore sight that has already been lost – for not just Poppy, but for everyone living with Usher syndrome.

"We need solutions for people at every stage of their journey," says Ali.

Research into treatments for inherited retinal diseases like Usher syndrome is currently at a turning point.

Where these conditions were once considered untreatable, gene therapies are beginning to emerge that could protect a person's vision for life.

However, inherited retinal diseases can be caused by hundreds of different genes. Further research is essential to unlock the full potential of gene therapy and expand treatment options for more of these conditions, including Usher syndrome type 2A.

Finding hope in community

Meeting other people and families living with Usher syndrome who were navigating similar experiences gave Ali and Ben a new perspective – helping to ease much of the grief and uncertainty they had been experiencing alone.

"Even something as simple as your child having a playdate with another child who wears hearing aids can make a huge difference," Ali says.

"It helps normalise their experience and shows them they're not alone."

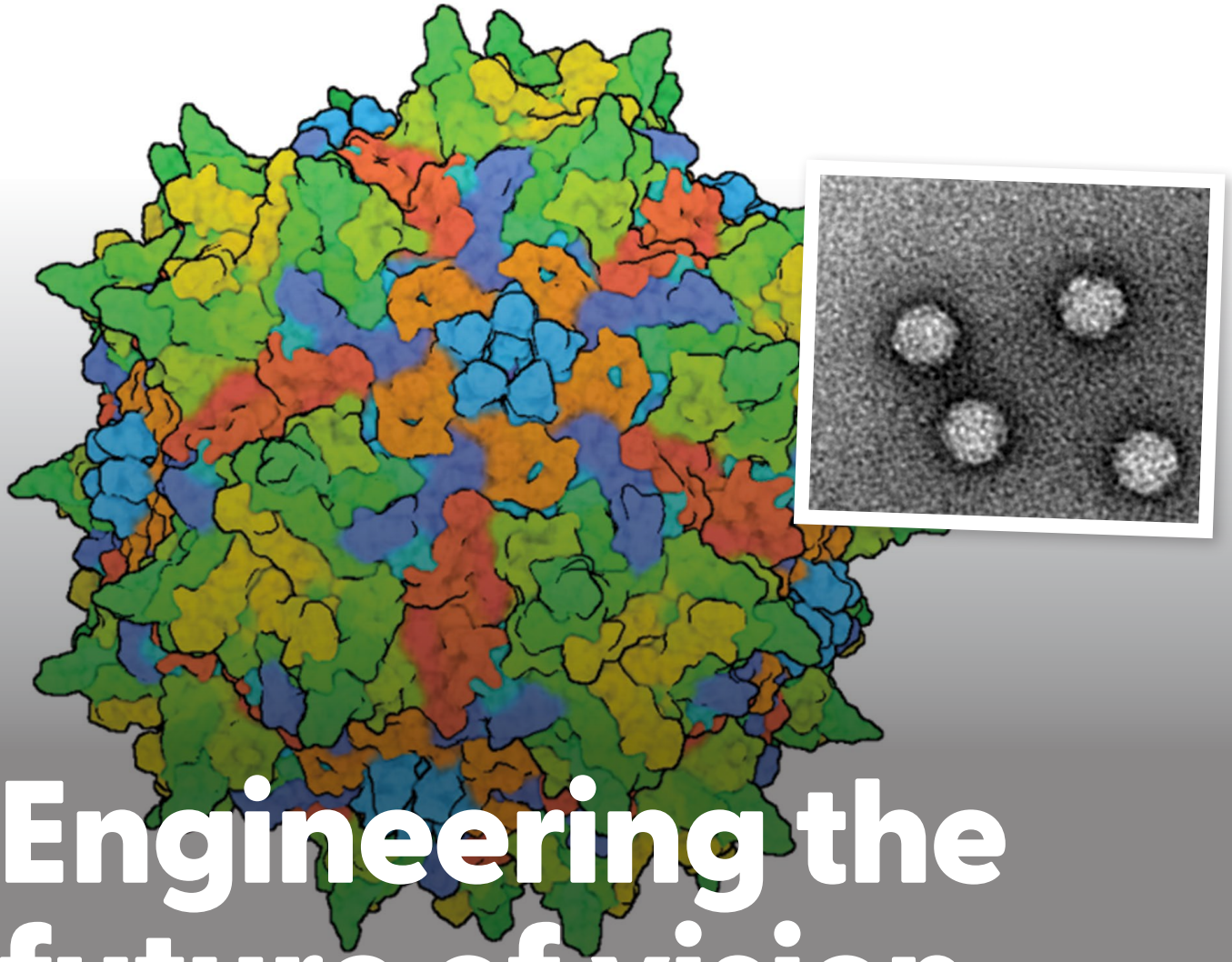
Expanding the possibilities

Today, Poppy is a happy and energetic five-year-old with no signs of vision loss yet. The family travels and loves new experiences, but the reality of Usher syndrome 2A remains quietly present.

"A cure honestly wouldn't change much for us today," Ali says.

"But it would change everything about Poppy's future.

"A cure would get rid of the ticking clock that is constantly there. It would expand the possibilities for her."



Engineering the future of vision

Vectors – the essential carriers for gene therapies targeting inherited retinal diseases.

IRDs were once considered untreatable, but thanks to years of research new gene therapies are now emerging that could help slow, stop or even reverse vision loss.

In IRDs, genetic changes mean the cells responsible for vision do not receive the 'instructions' they need to work properly. In many cases, this causes progressive vision loss over a person's lifetime.

Gene therapy is transforming how IRDs can be treated, offering new hope for people living with conditions such as retinitis pigmentosa, Stargardt's disease and Usher syndrome.

For researchers developing these gene therapies, the effectiveness of vectors is crucial. How well a vector works determines how effectively treatments penetrate damaged cells, how safely they can be delivered, and which genes they can carry.

In his lab at CERA, Head of Ocular Genetic Therapeutics Research Dr Jiang-Hui (Sloan) Wang is establishing a Vision Vector Core to engineer the next generation of viral vectors to target IRDs and other genetic eye conditions.

← *Advanced vectors: computer-generated image of an AAV particle and (inset) an electron micrograph of AAV particles.*

What is a vector?

A vector is a delivery system designed to transport therapeutic genes directly into the specific cells in the eye. Vectors are usually viruses with the disease-causing genes removed and helpful genes put in their place.

Think of a vector as a tiny truck that carries a therapeutic gene or gene-editing tool, protects it during transport, and delivers it to the retina – the light-sensitive tissue at the back of the eye that powers our vision.

These therapeutic genes give retinal cells the correct instructions they need to function properly, helping to prevent – and in some cases potentially reverse – vision loss.

For some IRDs, this could one day mean a single treatment that tackles the cause of the disease before vision is lost.

Targeting deep retinal cells

Dr Wang is focused on engineering advanced adeno-associated virus (AAV) vectors – tiny, harmless viruses that deliver healthy genetic instructions directly to the cells affected by disease.

"These types of vectors do not cause disease, are safe and are capable of entering directly where it's needed in the retina," says Dr Wang.

Many IRDs take hold in cells deep within the retina. Getting a therapy to these cells, safely and effectively, remains one of the biggest hurdles in developing treatments for IRDs.

To address this challenge, Dr Wang is developing a new vector engineered to reach these deep retinal cells, delivered through a less invasive injection, improving both how effectively and safely gene therapies target the cells that need them.

By carrying the corrective therapy to the right cells safely and effectively, his work aims to open the door to treatments IRDs that have so far been out of reach.

"This kind gene therapy delivery can change the lives of so many people," says Dr Wang.

Smarter vectors, future treatments

Dozens of AAV and other vector-based trials are now underway around the world, investigating new treatments for retinitis pigmentosa, Leber congenital amaurosis, choroideremia, achromatopsia, and age-related macular degeneration.

Researchers are also starting to use artificial intelligence to design smarter vectors – with the potential to reach deeper layers of the retina, trigger fewer immune reactions, and be delivered without surgery.

"The impact of vectors being used in eye health for early intervention and routine care treatments in the coming years cannot be underestimated," says Dr Wang.

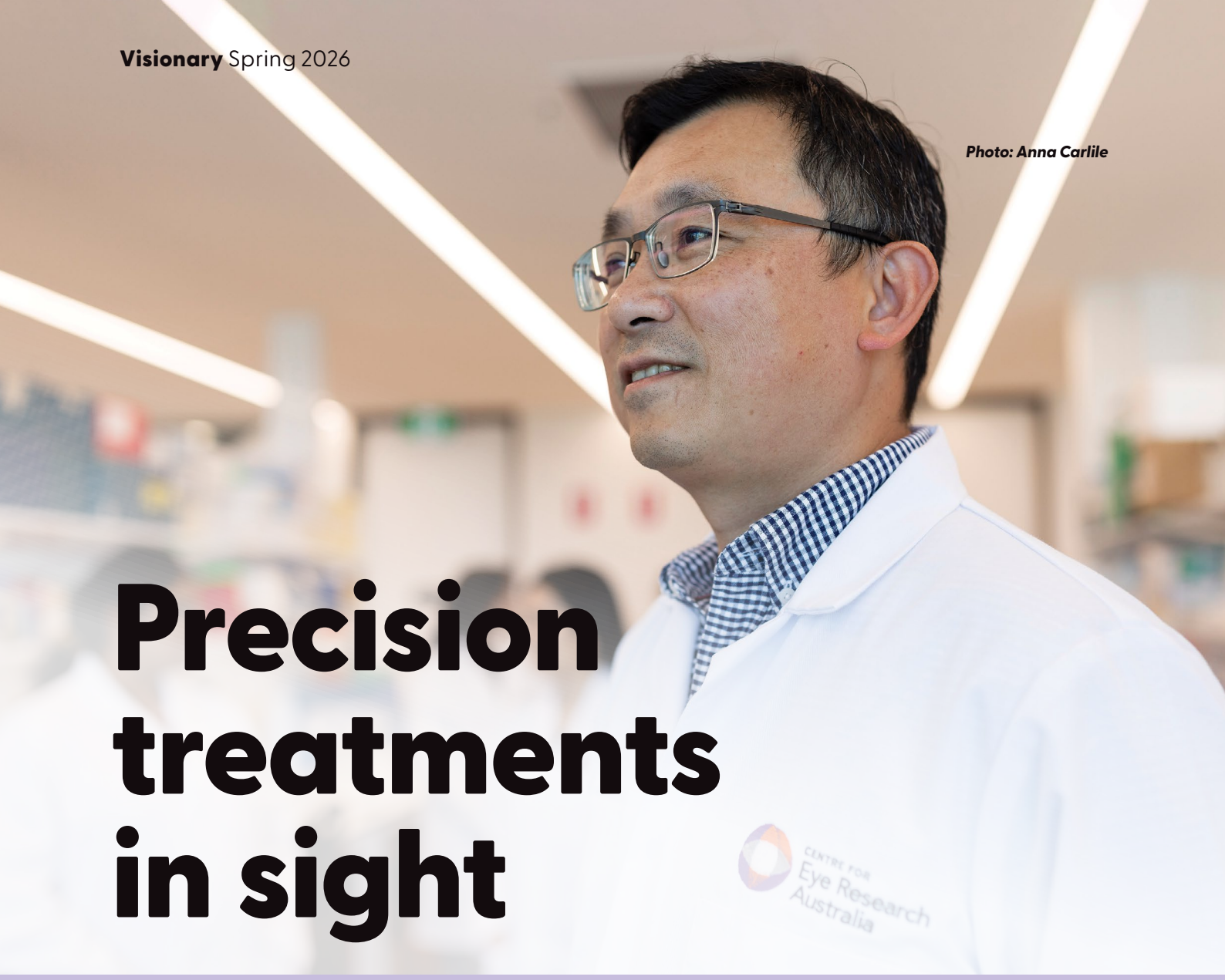


Photo: Anna Carlile

Precision treatments in sight

CERA's new spin-out company, Adaria Bio Pty Ltd, is developing precision treatments for genetic blindness.

CERA's Head of Genetic Engineering Research Professor Guei-Sheung (Rick) Liu is taking a new approach to help make sight-saving treatments available to more people.

He has founded CERA's newest spin-out company, Adara Bio, to translate his RNA editing research from the lab towards the clinic and develop precision treatments for a broad range of inherited retinal diseases.

"Rather than developing every treatment from scratch, which is slow and costly, Adara Bio aims to build an adaptable platform to treat many different IRDs," Professor Liu says.

The technology has the potential to speed up treatment development and help reach patients faster, including people living with some of the rarest inherited retinal diseases.

Changing the message

Most of the gene therapies currently available work by replacing a faulty gene with a healthy copy. The healthy gene is delivered to the eye using a viral vector - a modified virus that safely carries the treatment to the target cells.

One of the biggest challenges is that some disease-causing genes are too large to fit inside the viral vectors used to develop

Editing genes: Professor Guei-Sheung (Rick) Liu is accelerating his research through a new spin-out company.

gene therapies. Conditions caused by these larger genes, such as Usher syndrome type 2A, require a different approach, and Professor Liu and his team are addressing this challenge through the development of an RNA editing platform.

While DNA acts as the body's instruction manual, RNA carries those instructions to cells. With a single dose, the treatment is designed to correct faulty RNA messages so cells can function correctly, helping to prevent vision loss.

Because the treatment works at the RNA level, it does not alter a person's DNA. The platform is also compact enough to fit within the viral vectors already used clinically in eye therapies.

"The reason we're starting with Usher syndrome type 2A is that it is caused by a very large gene, and there is currently no way to treat it using a traditional gene therapy approach," Professor Liu says.

The EDITRA™ platform has also been designed to be adaptable, making it possible to develop treatments for many other inherited retinal diseases caused by large genes.

"If we can use the platform to treat Usher syndrome type 2A, we can modify it to treat many more of these conditions," Professor Liu says.

"This approach is faster and more efficient than developing treatments one disease at a time, and we hope it will help bring therapies to a much wider range of people living with inherited retinal diseases."

The technology has already been tested in eye models, and the team is now working towards an eventual clinical trial.

To help accelerate its path to patients, Professor Liu founded Adara Bio in collaboration with ophthalmology company Medic Vision AI. Medigen, a publicly listed Taiwanese pharmaceutical company, is a major shareholder of Medic Vision AI.

"We hope this means that we can accelerate the development of our work towards a treatment for Usher syndrome 2A, as well as eventually many more conditions," says Professor Liu.

Ruth's legacy of hope

The Ruth Marie Sampson Foundation has made a transformational gift to research that will help families living with Usher syndrome.

Thanks to the Ruth Marie Sampson Foundation's donation of $1.7 million CERA will establish a dedicated Usher syndrome clinic within the VENTURE inherited retinal disease (IRD) registry.

Usher syndrome is a rare genetic condition that causes hearing loss from birth and progressive vision loss from the teenage years.

Vision loss in Usher syndrome is caused by retinitis pigmentosa – a degenerative eye disease that typically begins with night blindness and narrowing peripheral vision during adolescence or early adulthood and can eventually lead to blindness.

There is currently no cure for Usher syndrome, but cochlear implants and hearing aids can help with hearing loss.

However, advances in gene therapy research are bringing new hope for treatments that could protect the sight of people living with this condition.

Connecting research and families

The new Usher syndrome clinic will create a direct link between scientists developing new treatments and families living with the condition, helping to build the participant registries needed to support future first-in-human trials.

← *A lasting gift: Ruth Sampson's legacy has the potential to change lives.*

The clinic's research aims to identify new genes and treatment targets, measure the natural history of the disease, validate novel clinical trial outcomes, and explore the psychosocial aspects of Usher syndrome.

It will connect families with genetic testing and help build VENTURE's advanced genomic testing capability, supporting families with inconclusive diagnoses.

The donation will support VENTURE co-lead Dr Tom Edwards, IRD Research Fellow Dr Sena Gocuk, one new laboratory-based genomics role and new Ruth Marie Sampson Clinical Research Coordinator, who will work directly with families.

The team will collaborate closely with Dr Ceecee Britten-Jones at the University of Melbourne, who is conducting major research into the genomics of IRDs.

About VENTURE

The VENTURE Study is a collaboration between CERA and the University of Melbourne.

It brings together clinical, genetic and real-world patient data to accelerate the development of sight-saving treatments for IRDs.

The program seeks to understand how IRDs progress over time, improve support and care for affected individuals and families, and identify people who may be eligible for future clinical trials.

With almost 800 participants enrolled so far – including around 30 people living with Usher syndrome – VENTURE is generating the critical evidence needed to advance breakthroughs in gene therapy and bring new treatments and potential cures closer to reality.

"Very few things are more important than eyes."

– Ruth Marie Sampson

Ruth's legacy

"Ruth once told us there are 'very few things more important than eyes', and the impact of those words lives on," says Diana Gibson, CERA's Head of Philanthropy.

"For families living with Usher syndrome, the impact of this gift extends beyond research outcomes. It also offers something equally important: hope that future generations of children with Usher syndrome may not have to face the prospect of losing their sight."

A tremendous boost

Dr Tom Edwards says the Ruth Marie Sampson Foundation's support is a tremendous boost for CERA's Usher syndrome research.

"We are deeply grateful for the Foundation's belief in our work," he says.

"We are proud to honour Ruth's legacy through research that has the potential to transform lives.

"The Foundation's commitment to eye research will leave a lasting impact on the lives of children and families across Australia."

Trial results put treatment in reach

Research conducted at CERA and Cerulea Clinical Trials points toward a potential first treatment for Stargardt disease – a sight-threatening genetic condition.

The international Phase 3 DRAGON trial – which included local participants from Cerulea Clinical Trials – has investigated whether the drug tinlarebant can treat the sight-threating condition Stargardt disease type 1, and has indicated it's capable of slowing the condition's progress.

The findings - which included local participants from Cerulea Clinical Trials - are a step towards a potential first treatment for the condition, which affects approximately 1 in 10,000 people.

Stargardt disease affects the retinal cells in the macula – the region of the eye responsible for central vision. It's a disorder caused by a genetic fault that leads to the degeneration of the retinal cells, leading to progressive central vision loss.

Stargardt disease can occur at any age between teenage years through to early and mid-adulthood. Currently, there is no approved treatment.

Moving closer to treatment

The findings, published by the drug's sponsor Belite Bio, showed that over two years, a daily tablet slowed the growth of these lesions by 36 per cent. Adverse events, when reported, were generally mild and consistent with tinlarebant's mechanism of action.

← *Trial success: Cerulea Clinical Trials was part of the DRAGON trial.*

The findings move the drug one step closer towards being approved by regulators as a treatment for the condition.

"Stargardt disease is one of the most common inherited retinal diseases and the most common macular dystrophy," says Dr Tom Edwards, Head of Retinal Gene Therapy Research at CERA.

"It's a very welcome result and satisfying that CERA and Cerulea Clinical Trials had a part to play in it.

"I'm certain it's even more satisfying for the participants, without whom we couldn't complete trials like this."

Local participants in the study were drawn from CERA and the University of Melbourne's VENTURE registry – a database of close to 800 people, who have or are carriers of genetic conditions that affect sight, including Stargardt disease.

VENTURE provides a way to study how these diseases progress, and its registry is a crucial way of getting people into trials for new drugs.

The power of clinical research

Cerulea Clinical Trials Chief Executive Officer Dr Michelle Bradney says the organisation is proud to have played a critical role in the successful completion of the study.

"This is an accomplishment that reflects exceptional expertise and collaboration across our internal teams, but also as a trusted partner of Belite Bio," she says.

Dr Bradney explains that Stargardt disease presents a unique set of challenges for clinical research. The disease can progress differently from person to person, and it requires highly specialised assessments and multidisciplinary care. Identifying and supporting eligible patients through clinical trials can be complex, and takes a high level of precision, technical capability and compassion.

"Cerulea has a strong commitment to advancing potential new therapies that make a difference to people living with vision loss and blindness," she says.

"We are honoured to have contributed to this clinical trial and would like to acknowledge the bravery and altruism of our participants who make the decision to participate in novel research.

"Their commitment is what makes these results possible and gives hope for a potential therapy that could change the future of Stargardt disease care."

People interested in joining studies at Cerulea can register their interest at ceruleaclinicaltrials.org.au

On target for breakthroughs

After more than two decades of generous donations, elite shooter Kim Frazer is taking aim at eye disease by leaving a gift in her will to CERA.

Sharp focus: Kim is a three-time Commonwealth Games gold medallist.

For Kim Frazer, making a difference has always been about looking ahead.

An accomplished bridge player, author and former elite target shooter who won three gold medals for Australia at the Commonwealth Games, Kim has always been passionate about helping people reach their potential.

Following a successful corporate career, Kim has devoted much of her retirement to volunteering and community service. She currently chairs the Bridge Australia Foundation, which supports youth development in the card game. This passion for community and helping others drives her to support CERA's research.

"Supporting research is one way of helping people you'll never meet, but whose lives may be better because of discoveries made in the future," says Kim.

Kim has donated to CERA since 2002 and has also chosen to include a gift to us in her will – ensuring her contribution to vision research continues well into the future.

Supporting research with real-life impact

Kim has worn glasses for most of her life due to short-sightedness, so she understands first-hand how vision problems can affect everyday life.

She believes investment in eye research has the potential to improve quality of life for millions of people, while reducing the personal and financial burden associated with vision impairment.

"Glasses are expensive, particularly if you need specialised prescriptions or multifocal lenses," she says.

Kim also understands that breakthroughs require patience, expertise and long-term support.

"Australians are often very generous when there's an immediate need, but lasting change requires long-term investment, and it takes a lot of money and time to create cures and treatments."

CERA Managing Director Professor Keith Martin says CERA's research would not be possible without supporters like Kim.

"As CERA marks its 30th anniversary this year, we celebrate donors like Kim who have played a critical role in advancing vision research and continue to help us create the future in sight."

Give monthly

and help create the future in sight

At CERA we're celebrating 30 years of creating the future in sight.

When you give to CERA, you help challenge the inevitability of blindness. Your support accelerates breakthroughs that prevent vision loss and restore sight – ensuring blindness is never accepted as something people must live with.

Join our community of regular givers and help accelerate sight-saving research by providing the reliable monthly funding our researchers need to drive the next 30 years of breakthroughs.

With your support, we'll move faster every month to a future without vision loss.

Set up your monthly donation today

donate.cera.org.au

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CENTRE FOR Eye Research Australia | Creating the future in sight

CENTRE FOR Eye Research Australia | 30 years

Donate now to our annual Matched Giving campaign.

Double your donation up to $200,000

Donate at doublehope.cera.org.au or call us on 1300 737 757