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Newsletter of Retina Australia (ACT) Inc

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February 2012

MESSAGE FROM THE PRESIDENT

A belated happy new year to everyone. We hope 2012 will be all you want it to be.

We at Retina Australia (ACT) certainly enter the new year with a spring in our step following a fabulous fundraiser at Thoroughbred Park in November that accrued a record total at a time when the funds we raise for scientific and medical research is more vital than ever. More details appear inside.

There is also the sense within the wider Retina Australia family, and indeed globally, that the pace of research into a cure for retinal dystrophies draws rapidly closer. We certainly hope so. Reports of research progress are also contained within this issue.

We would ask members to take particular note of reports herein about the Inherited Retinal Diseases Register administered by the Sir Charles Gairdner Hospital in Perth. They have implications for our ongoing fundraising.

Best wishes to all...

ROBIN POKE

MEMBERS' "MONTHLY LUNCH DATE"

Where: West Belconnen Leagues Club

Hardwick Crescent
Holt ACT 2615
0419 201 815

Date: 1st Monday of the Month

Location: West Belconnen Leagues Club, part of the Canberra Raiders Sports Club Group is located in the heart of West Belconnen, close to bus stops and Kippax Shopping Centre, and has ample on-site parking.

Lunchtime specials are very reasonably priced and the quality of the food is high.

The next monthly lunch is on **Monday**

5th March

Please let me know if you have a problem with transport and I will try to arrange for someone to pick you up.

Jan James
Secretary
Ph 6258 4823

GENERAL NEWS

FUNDRAISER A HUGE SUCCESS

We knew even before it started that Retina Australia (ACT)'s 2011 fundraiser at Thoroughbred Park was going to be a success, because a record number of 198 people had made bookings and we had to move the venue from the Rich Rewards Room to the much larger Silks Room! Those very generous and certainly fun-loving folk then proceeded to help us raise a record-breaking total of more than \$7000 for research into inherited retinal diseases. It was indeed a memorable day.

Special thanks must go to our special guest – sorry, guests! – Paralympic equestrienne Sue-Ellen Lovett and her beloved horse Ko-Olina, who came all the way from Dubbo to be with us and put on a dressage performance to remember.



Sue-Ellen then regaled us with her stories about her long-distance fund-raising rides and how she hoped very much that she and Ko-Olina would be selected for the London 2012 Paralympic Games. We learned subsequently, sadly, that this was not to be, but we nevertheless owe horse and rider a very big debt of gratitude.



Rachel Stokes
and John Barlow



Melisa Cantral,
Sue-Ellen Lovett
and Jan James



Elaine Grimley and June
Ashmore



Di and Jim Moir with David and
Jenny Kilby

We also owe particular thanks to a number of very generous sponsors and supporters, notably Myer Tuggeranong, Dream Design Furniture at Fyshwick, Women in Racing and the Canberra Racing Club. Each in their own way – Myer and Dream Design with their offerings of products for auctioning or raffling, Women in Racing for drumming up the numbers so brilliantly, and the Racing Club for its project management expertise. So Susan Morcombe, Andrew Hurst, Catherine Chapman and Peter Stubbs ... take a bow!



Lyn Tan, Susan Morcombe and Rosemary Harrison



Genevieve Hetherington, Sue King
Aldyth Mackay Gabriella Hawkins



Bob and Lyn Barlow, Lee Pope

ANNUAL GENERAL MEETINGS

Two weeks earlier, on 12 November, Retina Australia (ACT) held its 2011 Annual General Meeting, at which all executive members were elected unopposed. The only positional change was that Barbara Burton stepped down as Vice President and John Barlow took her place. Guest speaker Lindy Hou, a cycling Paralympian in Athens and Beijing, took us through the ups and downs of high performance sport and showed the audience her medals.

A month further back RA (ACT) was represented at the national AGM, held in Adelaide on 14 October, by John Barlow and Jan James. The major item on the agenda was the future of the Inherited Retinal Diseases Register (IRDR) being administered on behalf of Retina Australia by the Sir Charles Gairdner Hospital in Perth, and the means by which the Register continued to be funded. There follows a summary of the current situation where the IRDR is concerned.

GENOTYPING PROJECT

The initial three-year term of the national Inherited Retinal Disease Register/DNA Bank ceases on 31 March 2012. The cost of extending the project for a further three-year term has been estimated to be almost \$1 million, with specific attention to be given to sequencing/analysing the DNA material obtained during the first three years of the scheme's existence. However, efforts made by both Retina Australia and the Sir Charles Gairdner Hospital to obtain funding from the Federal Government and other sources was unsuccessful.

As a result of this inability to fund the extension for a full *three* years to 31 March 2015, Retina Australia received from the hospital a supplementary application for funding based on continuation of the project for a further *one* year, to 31 March 2013, with strong efforts nevertheless being made during 2012 to obtain funding through to 31 March 2015. The cost of the *one*-year extension, around \$360,000, was the major item considered by Retina Australia at its 14 October 2011 AGM and subsequent Directors' Meeting. The result of the deliberations was considered a positive outcome, with all of the States/Territories to contribute to ensuring the continuation of the project at least until April 2013.

Extract from a message from Dr John De Roach, Chief Investigator, the Australian Inherited Retinal Disease Register and DNA Bank

We wish to thank Retina Australia for agreeing to extend our funding for the Australian Inherited Retinal Disease Register and DNA bank for a further 12 months.

Historically, Retina Australia (WA) began funding what was then known as the Western Australian Retinitis Pigmentosa Register in 1984. In 2001 it was renamed the Western Australian Inherited Retinal Diseases Register and DNA Bank. In 2009 Retina Australia provided additional funding to translate this resource into a national register and DNA bank, it became a national IRDR, and Retina Australia recently announced an extension of its funding: \$358,622 for the period 1st April 2012 to 31st March 2013.

This national resource is now well-established. As a result, we are increasingly receiving requests for the provision of (de-identified) information or DNA from diverse research groups, both within Australia and from overseas. Recent examples include requests for DNA from participants with particular indicators from a laboratory in the USA that is uncovering a new gene implicated in Congenital Stationary Night Blindness; requests from a prestigious Western Australian institution for access to possible participants and genetic information, for inclusion in a Stargardt research trial, and requests from a Victorian group for possible participants for inclusion in an Australian Bionic Eye clinical trial.

It is the existence of a comprehensive Australian register of IRD-affected families, the DNA collected from those families, the clinical and family history that we have collected and the genetic analysis results obtained when we analyse this DNA that enable many diverse Australian and international groups to pursue their research with the use of this resource, thus obviating the need to duplicate expensive and time-consuming work. This is the only existing resource detailing the spectrum of genetic mutations causing IRD in Australia, thus allowing Australian research groups to focus on genetic areas of research that will initially benefit the most people. Also, as gene-specific research trials become more common over the next few years, this resource will play a pivotal role in identifying possible suitable participants, as a result of the genetic information we have pro-actively determined for so many participants.

An important spin-off of this research is our developing clinical service. We are becoming increasingly active in reporting a participant's genetic results to them via their ophthalmologist, as an aid to treatment, counselling, and consideration for possible inclusion in trials of the most recently developed therapies. So far we have [indirectly] reported genetic results to about fifty participants, and are currently seeking the creation of a hospital position to continue this valued service. This service, which allows the definitive cause of a patient's inherited retinal disease to be determined (thereby enabling informed decisions regarding treatment, counselling and gene-specific clinical trial participation), would not have evolved without RAWA's and RA's continued support for our research.

MORE FAMILIES NEEDED TO PARTICIPATE IN THE INHERITED RETINAL DISEASES REGISTER

Since 1 April, 2009 the IRDR/DNA Bank has been in operation as a necessary progression of research involving retinal gene therapies. The very significant success with animal trials gave the impetus for human trials to be approved and to commence.

The development and application of a gene therapy for an individual requires knowledge of the nature of that individual's genetic defect. This is done by provision of a blood (or in some instances saliva) sample from which the DNA is then extracted, with such being 'sequenced' to attempt to determine the specific gene responsible for the inherited retinal disease.

Over 2500 DNA samples have now been obtained. Retina Australia has funded the exercise through until 31 March 2012 with the expectation being that around 3000 DNA samples will have been collected during the three years involved. *However, the Sir Charles Gairdner Hospital has indicated to Retina Australia that, after the initial strong response from members across Australia, there has been a slowing in the receipt of expression of interest forms.*

Involvement in the program is voluntary, and families as well as individuals are strongly urged to participate. An Expression of Interest form, available from RA (ACT), needs to be completed and sent to the nominated department at the Sir Charles Gairdner Hospital. In due course the department will contact the member, seeking a DNA sample and family history information (family tree etc covering blood relatives). The department will provide everything needed for the sample to be collected. Other than the cost of getting to and from a pathologist there will be no cost to program participants, such being covered by the Retina Australia funding.

For privacy and possible family sensitivity reasons, the results of the DNA extraction, and subsequent analysis, will not be made known to the participants, however if specific results show that they could possibly be eligible for gene therapies as they become available, or involvement with gene therapy trials, they may be advised to make contact with an ophthalmologist or a genetic counsellor, to whom the DNA information will be made available.

At the Retina International Congress in June 2010 it was indicated that gene therapy trials are in the pipeline for several specific IRD genes/groups including LCA (currently under way), Stargardt's and Usher's with it being generally accepted that gene therapy activities are no longer regarded as solely research related activities – they are now close to being part of the clinical diagnosis and treatment process.

This further emphasises the vital need for all Australians with an inherited retinal disease, and nominated blood relatives, to be part of the Australian IRDR Register and DNA Bank. The second three year phase will see the continued collection of DNA material (i.e. from newly diagnosed persons etc) but most importantly the sequencing of the DNA collected during the first three year phase.

HELP WITH NUMBERS, PLEASE

Meanwhile Retina Australia (ACT) needs to learn, if possible, within the bounds of personal privacy, precisely how many of its members, and members of their families, have contributed their DNA to the IRDR. This is because official figures appear to conflict with what we know from personal enquiries. There are financial implications inherent in this request. The contribution we in the ACT make to the national 'pot' is contingent on how many members are involved in the program. If this request applies to you (and yours), we would be very grateful if, in the strictest confidence, you would provide our Secretary, Jan James, with this important information.

UPCOMING CONGRESSES

The Retina International General Meeting and Congress are to be held in Hamburg in July 2012, when it is expected that there will be extensive reporting on significant developments in the areas of gene therapy trials and stem cell treatments for inherited retinal diseases. There are indications that major advances are to be reported at the Congress.

Three months later, from 19 to 21 October, the 2012 Retina Australia Congress will be hosted by RA (NSW). Professor Robin Ali from London's Moorfields Hospital is to be the keynote speaker. Robin is the leader of the group of ophthalmic surgeons who performed the first of the human gene therapy trials at Moorfields, and is an acknowledged expert in his field. He is also doing a lot of work with stem cells.

The venue is The Sebel at Parramatta. There is to be a host of interesting speakers on developments in research plus a range of interesting workshops from which to choose. The proceedings will commence on Friday evening with a welcoming Cocktail Party, then on Saturday night there will be a Gala Dinner with professional entertainment and guest speaker Don Burke.

For those not familiar with Sydney, there is a fast and regular train service from within the Sydney Airport Terminal to Central Sydney and thence to Parramatta. In particular cases, the hotel will pick people up from the airport.

Details of the Congress, including a range of accommodation and booking information, will follow.

AUSTRALIAN BLINDNESS FORUM

Retina Australia is exploring the possibility of joining the Australian Blindness Forum (ABF) as an associate member, to speak as a strong voice to Government, particularly in light of the recent Productivity Commission Reports on the National Disability Support Scheme and Caring for the Older Australians. The ABF has as its major concern that blindness is kept as a priority within the Health and Disability Systems.

RESEARCH UPDATE

Why Gene Therapy Works for Retinal Disease

Oxford, UK - 27 October 2011

Professor Robert MacLaren, honorary consultant at the Oxford Eye Hospital and Moorfields Eye Hospital explains that gene therapy is ideally suited to the eye because:

- 1) Most of the incurable causes of blindness are genetic and correcting the genetic defect is the most logical treatment;
- 2) Only a small dose of the gene therapy is needed in the eye, compared to the liver or lung, for instance, where up to ten thousand times as many viral particles would be needed to get an effect;
- 3) The eye is relatively separate from the immune system, which reduces the likelihood of an immune reaction to the viral gene therapy;

4) The cells in the eye do not divide, which means we can use a viral vector which does not interfere with the DNA in the host cells. This also makes it much safer. Effectively we are creating an extra mini-chromosome that carries the missing gene.

Source: The University of Oxford

Gene Therapy Drug to Treat Usher Syndrome Receives FDA Approval for Clinical Development

Oxford, UK – 18 October 2011

Oxford BioMedica, the leading gene-based biopharmaceutical company, announced that the US Food and Drug Administration (FDA) has approved its Investigational New Drug (IND) application for the Phase I/IIa clinical development of UshStat®, a novel gene-based treatment for Usher syndrome type 1B. UshStat® was designed and developed by Oxford BioMedica using the Company's proprietary LentiVector® platform technology and is the third program to enter clinical development under the Phase I/II ocular collaboration agreement signed with Sanofi in April 2009.

The approval of the IND follows the decision by the US Recombinant DNA Advisory Committee (RAC) to approve the UshStat® Phase I/IIa protocol in May 2011. The open label, dose escalation Phase I/IIa study will enrol up to 18 patients with Usher syndrome type 1B at the Oregon Health and Science University's Casey Eye Institute, Portland, Oregon. The study, led by Professor Richard Weleber, will evaluate three dose levels for safety, tolerability and aspects of biological activity.

Usher syndrome is the most common form of deaf-blindness which affects approximately 30,000-50,000 patients in the US and Europe. One of the most common subtypes is Usher syndrome type 1B. The disease is caused by a mutation of the gene encoding myosin VIIA (*MYO7A*), which leads to progressive retinitis pigmentosa combined with a congenital hearing defect. UshStat® uses the Company's LentiVector® platform technology to deliver a corrected version of the *MYO7A* gene to address the vision loss associated with the disease. On the basis of pre-clinical data, it is anticipated that a single application of UshStat® to the retina could provide long-term or potentially permanent stabilisation of vision. There are currently no approved treatments available for Usher syndrome type 1B. UshStat® has received European and US Orphan Drug Designation which brings development, regulatory and commercial benefits.

Dr Stephen Rose, Chief Research Officer of the Foundation Fighting Blindness, an early funding collaborator of Oxford BioMedica's pre-clinical ocular program, said: "The IND approval for UshStat® is great news for people affected by a particularly devastating condition. UshStat® will be the first vision treatment for any type of Usher syndrome to move into a human study and, as a corrective gene therapy, it holds potential to halt the disease in its tracks."

Source: Oxford BioMedica

Gene Therapy Trial for Choroideraemia

Oxford, UK - 27 October 2011

The first patient to receive gene therapy for an incurable type of blindness has been treated at the John Radcliffe Hospital in Oxford as part of a trial led by the University of Oxford. If successful, the advance could lead to the first-ever treatment for choroideraemia, a progressive form of genetic blindness that first arises in childhood and is estimated to

affect over 100,000 people worldwide.

"This disease has been recognised as an incurable form of blindness since it was first described over a hundred years ago. I cannot describe the excitement in thinking that we have designed a genetic treatment that could potentially stop it in its tracks with one single injection," says Professor Robert MacLaren of the University of Oxford, who is leading the trial.

Jonathan Wyatt, 63, from Bristol had the surgery at the Oxford Eye Hospital and is the first of 12 people in this initial human trial who will receive the novel gene therapy.

Mr Wyatt was diagnosed with choroideraemia in his late teens and has suffered progressive sight loss ever since. He now sees only blackness except for a small area of a few degrees in diameter in the centre of his vision.

Choroideraemia is a genetic disease that leads to progressive degeneration of the retina in the eye. It generally affects males only and there is no treatment. The diagnosis is usually made in childhood and leads to blindness in men by their forties. It occurs due to deficiency of the REP1 gene located on the X chromosome.

The novel gene treatment was developed by Professor MacLaren at Oxford University in collaboration with Professor Miguel Seabra at Imperial College. It is designed to provide the gene missing in people with choroideraemia to stop the deterioration that gradually leads to blindness.

It uses a virus essentially as a delivery vehicle that ferries DNA, including the missing gene, into the right part of the eye. The virus has been engineered to infect the light-sensitive cells in the retina known as photoreceptors. There the gene is switched on and becomes active. With this particular gene therapy the treatment could provide a one-off permanent correction of the disease because the gene is thought to remain in the retinal cells indefinitely.

"This trial represents the world's first ever attempt to treat this disease and the first time that gene therapy has been directed towards the light-sensitive photoreceptor cells of the human retina," says Professor MacLaren. "This represents a major breakthrough and is highly significant for patients who are losing sight from other photoreceptor diseases, such as retinitis pigmentosa."

The trial will see twelve patients undergo surgery in which the gene therapy is injected into one eye. The other eye would then act as a control against which to assess any treatment effect. The researchers would, however, aim to go on to treat the second eye, should the treatment be proven to be effective.

The aim of the trial is primarily to assess safety, but it will also gain initial data on how effective the treatment is. The researchers estimate that it will take two years to know whether or not the degeneration has been stopped completely by the gene therapy.

"While safety appears so far to be fine, the efficacy of the gene therapy will only be evident after 24 months. We need this time to measure any effect as the degeneration caused by choroideraemia is slow."

Professor Seabra, who played a key role at Imperial College in identifying the gene causing

choroideraemia and in eliciting the mechanism of cell death in the retina, comments: "The ability to offer a gene replacement treatment for these patients was the final objective of 20 years of intense research in my laboratory. This is a moment of fulfilment for us and a dream come true for all choroideraemia patients."

Source: The Nuffield Laboratory of Ophthalmology

Gene therapy has the potential to restore sight to the blind

California, US – 20 April 2011

Researchers at the Keck School of Medicine of USC have developed a potential therapy for blindness that involves delivering a gene encoding a light-sensitive protein to inner retinal cells, enabling photosensitivity in these cells and restoring visual function in mouse models.

The research, led by senior author Alan Horsager PhD, a neuroscientist at the Keck School, focuses on blindness caused by retinitis pigmentosa and age-related macular degeneration, conditions that lead to gradual loss of photoreceptors in the retina and eventual blindness. Horsager's research targets other cells in the retina, called bipolar cells, which are part of the retina's intricate signal processing system.

"It's a very targeted approach that maintains the natural processing of the retina" said Horsager. "There is a lot more to understand, but initial indications suggest we have developed something that can have enormous benefit to people. Preclinical studies are the next step to determine the potential therapeutic benefit for humans."

After the gene encoding the light-sensitive protein is delivered via an adeno-associated virus, the bipolar cells become light sensitive and take over the light-capturing function of the lost photoreceptors.

"This is a massive collaborative effort between USC, MIT, the University of Florida, and Eos Neuroscience, building on a lot of great science," said Horsager. "We are simply aggregating this science and establishing proof-of-concept for a blindness therapy."

The research establishes that this therapy works independent of the underlying cause of photoreceptor degeneration, suggesting that people suffering from retinitis pigmentosa or age-related macular degeneration would benefit.

"We conducted multiple studies to establish that this technology is safe and does not appear to generate any immune response or inflammation in the eye," said Mehdi Doroudchi PhD, the first author and head of cell biology at Eos. The delivery system, an adeno-associated virus, is currently being used in multiple clinical trials of gene therapy throughout the U.S. and abroad.

The technology used to make the bipolar cells light-sensitive, known as opto-genetics, had its origins in a collaboration spearheaded by Boyden in 2004, which revealed that a light-activated protein from algae known as channelrhodopsin-2, when expressed in neurons, made them activatable by light. Boyden's group has since revealed an entire family of light-sensitive proteins that enable neurons to be switched on and off by different colours of light, which are now in widespread use throughout the field of neuroscience for analysing how neurons work in brain circuits.

"It's really exciting to think of the clinical applications opened up by the ability to control neurons by light," said Boyden. "The eye, which can access light from the outside world, is a perfect test bed for the use of optogenetic tools for treating intractable disorders."

Source: University of California

First Patients Undergo Stem Cell Transplantation Treatment

Marlborough, Mass. US – 14 July 2011

Advanced Cell Technology (ACT) has announced the dosing of the first patients in each of its two Phase 1/2 clinical trials for Stargardt's macular dystrophy and dry age-related macular degeneration (dry AMD) using retinal pigment epithelial (RPE) cells derived from human embryonic stem cells (hESCs). Both patients successfully underwent the outpatient transplantation surgeries on July 12 and are recovering uneventfully.

Both the Stargardt's trial and the dry AMD trial will enrol 12 patients each, with cohorts of three patients each in an ascending dosage format. Both trials are prospective, open-label studies designed to determine the safety and tolerability of hESC-derived RPE cells following sub-retinal transplantation into patients with Stargardt's and dry AMD at 12 months, the studies' primary end point.

"This first treatment milestone is welcomed by scientists, stem cell advocates and patients hoping for cures," said Gary Rabin, interim chairman and chief executive officer of ACT. "The two trials could not have started any smoother, and we are very pleased to announce that the procedures went well. The dosing of the first patients represents an important milestone for ACT and opens the doors to a potentially significant new therapeutic approach to treating the many forms of macular degeneration. We believe that these procedures represent a key step forward in therapeutic stem cell research, and the capacity to treat a variety of devastating diseases."

Dr. Schwartz, the studies' principal investigator, explained: "One patient in each clinical trial, the Stargardt's trial and the dry AMD trial, has undergone surgical transplantation of a relatively small dose (50,000 cells) of fully-differentiated retinal pigment epithelial (RPE) cells derived from human embryonic stem cells. Early indications are that the patients tolerated the surgical procedures well. The primary objective of these Phase 1/2 studies is to assess the safety and tolerability of these stem cell-derived transplants. We will be carefully monitoring our patients over the course of the trials. We are privileged to be collaborating with ACT and honored to be working with these pioneering patients."

Dry AMD, the most common form of macular degeneration, Stargardt's and other forms of atrophy-related macular degeneration are usually untreatable. Safe and effective therapies are greatly needed for the treatment of these common forms of blindness. Disease progression of both Stargardt's and dry AMD includes thinning of the layer of RPE cells in the patient's macula, the central portion of the retina and the anatomic location of central vision. With RPE cell death comes the loss of macular photoreceptors and loss of central vision. Currently both conditions are untreatable and often lead to legal blindness over a multi-year course. ACT's Stargardt's and dry AMD therapies treat these conditions by transplanting RPE cells in the patient's eyes before the RPE population is lost.

"Today - 13 years after the discovery of human embryonic stem cells - the great promise of these cells is finally being put to the test," said Dr. Lanza. "The initiation of these two clinical trials marks an important turning point for the field. While we will continue writing research papers and carrying out more research, it's time to start moving these exciting new stem cell therapies out of the laboratory and into the clinic. Tens of thousands of people continue to die every day from diseases that could potentially be treated using stem cells. In the meantime, we intend to accelerate our efforts to translate new embryonic stem cell (ES) and induced pluripotent stem (iPS) cell therapies into the clinic. It has taken years of extensive research to get to this point. Our research and preclinical studies have demonstrated the safety and effectiveness of such therapies. We hope these cells may provide a treatment option not only for degenerative eye diseases, but for a wide spectrum of other debilitating conditions, ranging from diabetes to vascular and autoimmune diseases. Our team remains committed to moving the field of regenerative medicine forward from bench to bedside."

About SMD, Dry AMD and Degenerative Diseases of the Retina

Stargardt's macular dystrophy (SMD) is one of the most common forms of macular degeneration in the world. Stargardt's causes progressive vision loss, usually starting between 10 to 20 years of age. Eventually, blindness results from photoreceptor loss associated with degeneration in the pigmented layer of the retina, called the retinal pigment epithelium or RPE cell layer.

Degenerative diseases of the retina are among the most common causes of untreatable blindness in the world. As many as 30 million people in the United States and Europe suffer from macular degeneration, which represents a \$25-30 billion worldwide market that has yet to be effectively addressed. Approximately 10 per cent of people ages 66 to 74 will have symptoms of macular degeneration, the vast majority suffering from the "dry" form of AMD - which is currently untreatable. The prevalence increases to 30 per cent in patients 75 to 85 years of age.

Source: Advance Cell Technology

New Trial for Retinal Stem Cell Treatment Gets Go-ahead

London, UK - 22 September 2011

Trials of an experimental new treatment that could offer hope to young people suffering from a currently untreatable inherited eye condition will get under way soon at Moorfields Eye Hospital following approval on 22 September by the Medicines and Healthcare Products Regulatory Agency (MHRA).

The trials, due to start in the next few months, will investigate the safety of using retinal cells derived from stem cells to treat people with advanced Stargardt disease, a form of macular degeneration that causes disabling loss of sight in young people. The cells will be injected into the retina during an operation lasting up to an hour.

"There is real potential that people with blinding disorders of the retina, including Stargardt disease and age-related macular degeneration, might benefit in the future from transplantation of retinal cells," says retinal surgeon Professor James Bainbridge, who will be conducting the trials at the National Institute for Health Research (NIHR) Biomedical Research Centre based at Moorfields and the UCL Institute of Ophthalmology.

"The ability to regenerate retinal cells from stem cells in the laboratory has been a significant

advance and the opportunity to help translate such technology into new treatments for patients is hugely exciting. Testing the safety of retinal cell transplantation in this clinical trial will be an important step towards achieving this aim.”

The technology has been developed by US company Advanced Cell Technology (ACT), who got the go-ahead to run similar trials in the United States in November 2010.

Source: Moorfields Eye Hospital

AMD Researcher Named Recipient of Stem Cell Research Prize

London, UK - 11 October 2011

The New York Stem Cell Foundation (NYSCF) has awarded the inaugural New York Stem Cell Foundation-Robertson Prize in Stem Cell Research, a \$200,000 prize awarded annually for extraordinary achievement in translational stem cell research, to a British scientist working to cure blindness.

Professor Peter J. Coffey, DPhil, Director of the London Project to Cure Blindness and Professor of Cellular Therapy and Visual Sciences at the Institute of Ophthalmology in London, received the award for his pioneering work in the use of human embryonic stem cells to cure Age-Related Macular Degeneration (AMD), a common form of blindness.

Professor Coffey's research has demonstrated that stem cell-based therapy halted visual deterioration in models of AMD, a currently untreatable form of blindness affecting millions of people across the globe. Clinical trials using the therapy are expected to begin in 2012.

“On behalf of the London Project to Cure Blindness team, I am honoured to accept this prize,” said Professor Coffey. “This financial award will go directly to support this critical research that offers hope for preventing blindness, re-storing sight, and improving the quality of life for sufferers of AMD. I thank The New York Stem Cell Foundation for recognising this important work and for its support of translational stem cell research.”

Professor Coffey's research focuses on replacing retinal pigment epithelium (RPE), cells at the back of the eye thought to be damaged in AMD. His team was able to transform human embryonic stem cells into healthy, fully functioning RPE cells, which was not possible using adult stem cells. Professor Coffey and his team also successfully demonstrated that visual function could be restored in an animal model.

Source: UCL Institute of Ophthalmology

Implant of First Ever Commercial Artificial Retina

Lausanne, Switzerland – 2 November 2011

Second Sight Medical Products, Inc., the world's leading developer of retinal prostheses for the blind, announced that the first ever commercial implantation of such a prosthesis was successfully completed on Saturday, 29 October in Pisa, Italy. The company's Argus® II Retinal Prosthesis System (Argus II) was surgically implanted by Dr. Stanislao Rizzo, Director of the University Hospital Ophthalmic Department of Pisa, in a patient suffering from advanced Retinitis Pigmentosa (RP). Argus II, which received marketing clearance in Europe earlier this year, becomes the world's first ever commercial implant intended to restore some vision to a previously blind patient.

"I am very pleased to offer in Italy for the first time ever this approved treatment for blindness due to RP." said Dr. Rizzo. "It is wonderful that medicine can now do something for the blind."

Argus II is Second Sight's less invasive second-generation implantable device intended to treat blind people suffering from degenerative diseases of the outer retina such as RP. The system works by converting video images captured by a miniature camera, housed in the patient's glasses, into a series of small electrical pulses that are transmitted wirelessly to an array of electrodes on the surface of the retina (epi-retinal). These pulses are intended to stimulate the retina's remaining cells resulting in the corresponding perception of patterns of light in the brain. Patients can learn to interpret these visual patterns, thereby gaining some functional vision. The system was tested in a multi-centre international clinical trial that began in 2007.

Dr. Rizzo indicated that the surgery proceeded without incident, and that the patient was recovering very well. Within a week of surgery, testing of the implanted device will begin and the Argus II will be electronically customised for the patient, who could be using the system at home before the end of the month. Typically, patients require a short period of training and low vision rehabilitation to obtain the best results.

Gregoire Cosendai, PhD, head of Second Sight's European subsidiary added: "This is truly a historic event that has been decades in the making. This milestone is significant for the company and the field of vision restoration, but most importantly, for these patients who now have a treatment option." Cosendai further indicated that the Argus II system is currently available in Germany, France, the UK, and Switzerland, and that Second Sight is actively adding more centres throughout the Europe.

Source: Second Sight

Book Review

THUNDER DOG: *The True Story of a Blind Man, His Guide Dog, and the Triumph of Trust at Ground Zero*
by Michael Hingson

Michael Hingson was working in his office on the 78th floor of the World Trade Centre, Tower 1 on 9/11/01 when the terrorist attack happened. Roselle, the three-year guide dog by his side, didn't need to know that a hijacked plane flew into the building. She just knew that her job was to keep her partner safe and out of harm's way. The circumstances were extraordinary, but Roselle rose to the challenge and guided Michael down 1463 steps to safety. According to the author, "Thunder Dog is not a story about 9/11, but rather about trust and teamwork. Readers will be inspired by the bond that Roselle and I shared and how the human-animal bond is enriching and even saving lives." Roselle passed away earlier this year after a long working life and retirement with Michael and his wife. Recently Michael established Roselle's Dream Foundation, which is intended to educate people about blindness and assist blind children and adults in obtaining some of the technologies needed to work in the world. Michael says: "The real handicap of blindness is not a lack of eyesight, but a lack of education about blindness. My dream is to raise funds to help offset the costs of wonderful technologies that can help the visually impaired. It is an honour to create this foundation in Roselle's memory."

The book is available for purchase from www.amazon.com

ABOUT US

Retina Australia (ACT) Inc. is a member of the national body, Retina Australia (RA). Other members of RA are Retina Australia (NSW), Retina Australia (VIC) – which incorporates activities in Tasmania – Retina Australia (QLD), Retina Australia (SA) and Retina Australia (WA). There is also a newly-formed group in the Northern Territory.

Our role, and that of our fellow organisations, is to provide information and support to people and families affected by Retinitis Pigmentosa and other retinal dystrophies. We also raise funds for scientific research into the causes and prevention of these dystrophies.

Retina Australia is a member of Retina International, which has members and affiliates in more than 50 countries. It is estimated that more than 20 million people worldwide are affected by some form of retinal dystrophy.



FIGHTING BLINDNESS

Mission Statement: To provide information and support to people and families affected by inherited retinal dystrophies, and, with the support of the Australian community, to raise funds to finance scientific research into the causes, prevention and cure of retinitis pigmentosa.

Ways You Can Help Retina Australia (ACT)

There are many ways you can help and support us. Listed below are some suggestions.

1. Suggest to family and friends that they hold a special event (such as a sponsored walk or fundraising dinner) to support our work.
2. Include a reference to us when updating your Will.
3. Continue with your much-appreciated cash donations.
4. Tell your local eye professionals (and your family and friends) if you have found the information provided by Retina Australia (ACT) and its support services useful.
5. Offer your services to a member of the executive as there are numerous small jobs that need to be done. Many of these do not require you to be on a committee.
6. If approached, consider allowing your medical information and DNA to be stored on the inherited retinal diseases database so it is readily available to eye researchers both here and overseas.
7. When you hear that someone in your community has been diagnosed with sight loss tell them about Retina Australia (ACT) and the support services offered.

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