

JUNE 2023 NEWSLETTER

Dear friends and supporters

I was thrilled to show Nigel Atkinson Esq, HM Lord-Lieutenant of Hampshire, and Mrs Atkinson some of the work being undertaken in our laboratories. They are both hugely interested in our research and it was lovely to see them again. I also enjoyed meeting guests at Little Court in Crawley earlier this month and will look forward to catching up with many more of our supporters at some of our future events.

Every year eye researchers have an opportunity to meet up at the largest eye research Conference in the world. This provides an excellent chance to interact with colleagues from



L-R: Catherine Robertson, HM Lord-Lieutenant, Mrs Christine Atkinson, Dr Jörn Lakowski and Prof Andrew Lotery.

around the globe and to develop collaborations to push our research forward. This year the meeting was in New Orleans and I was delighted that three of our junior researchers presented their work, which was well received. Our study of macular degeneration called the Pinnacle study had seven presentations at the meeting. It was gratifying that other researchers referenced our work in their talks. A report on the Pinnacle study was also highlighted locally by the National Institute of Health Research on "International Clinical Trials day" in May, as an important research project.

I am delighted at recent significant successes from our team which are reported in more detail later in this newsletter, including:

- Dr Jörn Lakowski's success in securing an academic grant of £548,535 from the Biotechnology & Biological Sciences Research Council.
- Dr Jay Self and a wider team from within the University of Southampton have collaborated to develop an app to help children with amblyopia (lazy eye) which will help thousands of young patients throughout the UK.
- Dr Adnan Khan has been awarded a Fulbright Commission UK Scholarship and will be taking up his appointment in Iowa in September.

All these scientists have been directly supported by the Gift of Sight appeal and have been positively impacted by this help.

Gift of Sight's mission is both to train the next generation of eye researchers and also to translate research into better treatments for patients. I think the above examples demonstrate how this is being achieved. Your support has been directly responsible for our success. Thank you.

With my very best wishes.

Andrew Lotery

Andrew Lotery MD, FRCOphth Professor of Ophthalmology University of Southampton

The Gift of Sight Appeal is managed by the University of Southampton which is an 'Exempt Charity' (HMRC reference number X19140) under the terms of the Charities Act 2011.

Dr Jörn Lakowski, Lecturer in Vision Sciences

Inherited retinal dystrophies, featuring the progressive degeneration of the rod and cone photoreceptors, lead to irreversible blindness. Collectively, they represent the most frequent inherited forms of human visual disability, with an estimated prevalence of 1 in 3000 worldwide. While rods enable vision in dim lighting conditions, human high acuity and colour vision depends on cone photoreceptors. In addition, rods provide indispensable trophic support to the latter. Even though cone photoreceptors are a rare population, forming only 2-4% of total retinal cells, diseases effecting them, either directly or through the loss of rod



derived trophic signalling, have an incapacitating impact on the lives of those affected. Unfortunately, current treatments are very limited and can only delay the onset of sight-loss, but not stop or reverse it. Thus, there is a great impetus to develop new and more effective treatment options.

One prominent therapeutic approach, called "cell replacement therapy" involves the transplantation of lab-grown photoreceptors into the retina in order to replace those lost in the disease, and thus preventing further visual deterioration. Following transplantation, donor cells have to navigate the host environment and integrate into the existing retinal circuitry making the correct turns along the way and forming functional synaptic connections.

Over the past 10 years, great strides have been made in the field, using rodent models to work out the necessary protocols and parameters. However, it has become clear that many significant hurdles remain to translate this approach from lab-bench to bedside. For example, while the premise of the cell therapy approach rests entirely on transplanted cells "finding" their way in this new environment, we do not actually know how this process works and how we can optimize it to improve the outcome.

The human pluripotent stem cell derived retinal organoids, or human "mini-retinas", which we grow in our Gift of Sight supported stem cell lab, present the perfect model systems to study how human photoreceptors naturally migrate during development and assume their final position within the retina. This is essentially the process that cell therapy aims to emulate and that currently hampers efficient translation into the clinical setting.

As reported in my last newsletter contribution, pump-prime funding from Gift of Sight and the Academy of Medical Sciences helped us to generate several fluorescent photoreceptor reporter stem cell lines. These allow us to visualise and track in real-time the rods and cones within our retinal organoids as they grow in the cell culture dish. I should add that these tools are really only available in a handful of labs in the world and they now provide us with unique research opportunities in both basic biological research and therapy development. As you may now appreciate, there is a seamless transition between basic research and therapy development here, and this is generally true.

This view was also shared by the Biotechnology and Biological Sciences Research Council (BBSRC), which recently awarded us a 3-year research grant worth £548,535 (funder contribution). It goes without saying, that this quite significant research funding would not have been possible without the unwavering support of Gift of Sight and its many donors. The infrastructure we created here in

Continued...

the Vision Group with the help of Gift of Sight, such as our human stem cell lab, combined with the expertise we have built over many years convinced the BBSRC review panel to make this strategic investment.

Our new research programme will combine stem cell and genome editing technology at scale to elucidate the cell-signalling cascade involved in controlling cone photoreceptor migration and identify drugs to manipulate this process. We expect that this work will greatly advance our understanding of human cone photoreceptor development, but also reveal common principles guiding the assembly of the complex retinal architecture as a whole. With a view to cell replacement therapy, the pharmacological reagents identified in this study will serve as a starting point towards the development of tools to facilitate migration and synapse formation of transplanted donor cells in a future clinical setting.

Occlusion Therapy (Patching) - New App Launched

In our October 2022 newsletter we shared details of a survey relating to patching therapy. Amblyopia, also known as lazy eye, is the most common cause of preventable visual impairment in children. However, 50% of patients fail the treatment and end up with life long poor vision.

As mentioned by Prof Lotery, we are thrilled that - after many years of work - eye specialists led by Dr Jay Self, mathematicians, and games designers at the University of Southampton have teamed up to develop and launch a smartphone app called 'The Amblios Club'. This is aimed at helping improve vital treatment for children with amblyopia.

The prototype app was launched at the end of May and met with tremendous

media interest, being reported by the BBC, ITV and numerous newspapers. The team have received many enquiries from parents throughout the UK who would like their children to be signed up to use the app. This is an amazing result and has been helped by donations to Gift of Sight. A copy of the University of Southampton press release is <u>here</u>. Please do get in touch with us if you'd like to know more, contact details are given on the back of this newsletter. Thank you.

Dr Adnan Khan, NIHR Clinical Lecturer in Ophthalmology

I am delighted that I was recently announced as the recipient of a prestigious Fulbright Scholar Award from the US-UK Fulbright Commission. My interests include sight-threatening retinal and ocular inflammatory diseases and, over the last four years, I've focused on age-related macular degeneration (AMD) at the University of Southampton with Professor Andrew Lotery and the vision sciences group.

There is increasing evidence that an age-related decline of immune system regulation, known as immunosenescence, contributes to degenerative diseases of the macula (the central part of the retina). A UK-wide shortage of donor eye tissue makes it difficult to undertake the most innovative retinal research. I will use my Fulbright Award to translate areas of good practice from the novel partnership of The University of Iowa with Iowa Lions Eye Bank. I will use ground-breaking technology to further investigate immunosenescence in macular diseases as a basis for translation into curative therapies. <u>https://fulbright.org.uk/our-community/meet-our-fulbrighters/</u>

Thank you Gift of Sight, the De Laszlo Foundation and all donors for your significant help with my work.





THANK YOU FOR YOUR SUPPORT!

Little Court Garden Party 15 June 2023, Crawley, Winchester

Grateful thanks to Mrs Patricia Elkington for kindly inviting us to use her beautiful garden for our 'Drinks and Canapes' evening. We were blessed with glorious summer sunshine and over 60 guests were able to wander around the garden and mingle with researchers from the vision science laboratories. Raffle prizes were donated by generous local companies, including Bombay Sapphire Distillery, Hattingley Valley Wines, Winchester branches of Waitrose, Hilliers Garden Centre and Chococo. The evening raised over £2,000 which is an



Professor Andrew Lotery and Mrs Patricia Elkington

It was lovely to have such fabulously supportive guests and thank you for coming along to join us. You made the evening a huge success and we'll look forward to keeping in touch with you!



amazing sum.

Challenge Adventure Charities

A team of four including David Simpson, Martin Gorman, Simon Martel and James Beaver rode from St Malo to Cahors covering 500 miles in the 2022 CAC Cahors Challenge. The route took the riders through Angers and Limoges and David Simpson kindly presented Ailsa with a cheque to Gift of Sight for £2,500.

David Simpson and Ailsa Walter Thank you all so much for your further support and for your generosity in undertaking these challenges to help many charities!

FORTHCOMING EVENT

A Hoedown with Testvale Squares Foxlease, Clay Hill, Lyndhurst, SO43 7DE Saturday 15th July 2023 7pm—10pm

An evening of fun and dancing to instruction. No experience necessary! Please wear flat or comfortable shoes. Raffle, soft drinks and light refreshments available.

Cost: £10pp (over 12 years only please - sorry but

unsuitable for young children)

Please book online at www.giftofsight.org.uk/events or to pay by card please phone 023 8059 7239



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