NOVEL GENE THERAPY CURES BLINDNESS

Professor MacLaren (right) *at work in the operating theatre*

Transcription of an interview with Robert MacLaren, Professor of Ophthalmology

'If you're suffering from an incurable disease, to be told that there's nothing that can be done and no one is doing any research into it is one of the worst things that we can possibly say. At least if I can say to people, 'There isn't yet a cure, but we're working on developing a cure through this clinical trial,' it just changes their whole concept; it gives them hope.

Choroideraemia is prevalent in about one in 50,000 people, all round the world as far as we know, and these people go blind. It affects men, and the reason they lose their sight is because they're missing a vitally important gene which is at the back of the eye, and it's important for keeping the eye healthy. What we're doing with our gene therapy research is that we're actually using a virus – a tiny viral particle – to deliver the correct gene back into the cells at the back of the eyes in these patients.

The eye is a fantastic target organ for gene therapy, because the genes that cause retinal degeneration are well documented, there are plenty of them, and we can know exactly what single gene to put back. We also have a control eye, so we can look at the degeneration in one eye, and see how it changes compared to the other. And although it's a little bit early to talk about the results, the results that we've seen so far in terms of the visual improvement have been very, very encouraging.

The disease itself is relatively rare, but the concept of what we're doing, the technology, everything we're doing in thi trial, will be relevant to other causes of blindness – and we can think of retinitis pigmentosa, which is a disease that causes blindness in about one in 4,000 people in the UK, through similar genetic mechanisms. And also, potentially, age-related macular degeneration, which has similarities to choroideraemia, and it is a disease that causes blindness in 300,000 people in the UK, and is increasing each year with the increase in the elderly population.

One of the benefits of being in academic research rather than a pharmaceutical company or something like that is that, is the fact that we can be open and collaborative with our work. There is no financial incentive for us in what we're doing, we're all working together for the same goal. And I think that makes a very good work environment.

It's fantastic to see the realisation of what I've been working on for many, many years. We're not just talking about technology – I have patients in my clinic who've got choroidaeremia. To see the expression on their face when they know that we're developing this clinical trial is something that I will not forget. Mothers with affected sons, people of working age, people of our age, who are in the process of losing the very last remnants of sight, to suddenly know there is potentially something out there … as a clinician, to be able to help people, particularly when it's something that has up until this time been incurable, that's something I find very rewarding and very satisfying.'

To view the video interview visit: www.ox.ac.uk/annual-review

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