

Summary of March Address

Guest Speaker: Professor John Rasko
Subject: “Gene and Stem Cell Therapy”

Haematologist Professor John Rasko, the first formal appointee in clinical gene therapy in Australia, , directs Cell and Molecular Therapies at RPA Hospital and heads the Gene and Stem Cell Therapy Program at the Centenary Institute, University of Sydney.

The author of over 100 published papers on this and allied subjects, he has presided over and served on related Australian and international associations, and currently chairs the Gene Technology Technical Advisory Committee of the Gene Technology Regulator for the Australian government. His successful research includes uncovering new mechanisms of leukemia, also clinical trials of new biological therapies for cancer and bleeding disorders, hemopoietic stem cell mobilisation and transplantation.

Introduced by Alistair Brown, John immediately gained our avid attention with his bright, clear and enthusiastic delivery of a very technical subject. The main gist of his presentation covered the principles of Gene and Stem Cell Therapies, and likely future developments, with a belief that this shift to a new form of medicine would replace many pharmaceuticals. By targeting the roots of problems with a single treatment, fewer side effects would result.

His presentation comprised sections including – *Worth the Wait, The Cancer Problem, What’s happening in Cell Therapies, A crash course in DNA/ Genes, Haemophilia, Ethics involved and How we can help*, and was supported by graphic charts throughout.

Health now accounts for approx 9.3% of our GDP, with ever increasing pressures from increased longevity, technology changes and increased patient expectations. Whilst the government funds low risk evolutionary research, funding for more radical research is always in shortfall.

Cancer is now a major problem with 1 in 3 men and 1 in 4 women having cancer in some form by age 75. The incidence is increasing by 1.5% per annum, currently accounting for 27% of all deaths in NSW. The RPA/SCC is treating 10,000 new patients per annum.

John provided an outline of what is currently happening in cell therapy, dealing with cells from one self, a relative or a matched unrelated person, recognising blood as a liquid organ and transplanting cells of that organ. Of interest, every second, 1 million red blood cells are created from blood stem cells. In regard to gene therapy, the term “Ex Vivo” relates to manipulation of cells outside the body, whilst “In Vivo” relates to injection of genes into the body.

Revolutionary research, recognising stem cells in other body organs such as the brain, liver etc., envisages blood stem cells being coaxed to make other such organs. He then outlined the clinical nature of the facility at RPA with its highly filtered air, where the therapies occur.

We were then given a crash course in DNA/genes, using genes as medicine. A cell contains DNA in a 2 strand helix; these DNA units are called genes. These genes are

encoded as Cs, As, Gs, and Ts. And their physical location and order determine what we are.

Genes cause diseases; therefore the aim is to introduce genetic information into human cells to treat such diseases. It sounds compellingly straightforward, and research is on track to achieve this revolutionary new mode of medicine. But it takes a long time as there are many difficulties to overcome.

As an ultimate goal, there are some 4,000 diseases to be cured by gene therapy, the trick being to use viruses as “Trojan horses”. Viruses take over the genetic machinery of cells. As an example, viruses modified as required could be used to treat Cystic Fibrosis.

A watershed disease in this story is considered to be the case of David Vetter - “The boy in the bubble” who lived from 1971 to 1984 and suffered from immune deficiency syndrome. He died following a bone marrow transplant. However, children with the same disease in France were subsequently cured by gene therapy combined with bone marrow transplantation with no immediate side effects. Several of the children treated later suffered from leukaemia as a complication – but the hope is that this feared side effect can now be overcome. John then touched on the history of Haemophilia including how the Russian aristocracy suffered this disease. The human genome needs only one error in the order of 3.2 billion genes to cause haemophilia – a hidden tragedy.

In further advising what this research has to offer, he commented on gene transfer and how a haemophilic dog had been injected with genes and had been basically cured. In the human studies he has pioneered, the early promise of a cure was thwarted by immune complications which are now being addressed in a subsequent clinical trial in Haemophilia.

Next he outlined the Ethics of Inheritable Genetic Modification, and finally expressed the ever present need for investment in this form of research.

In question time, we gained the impression that the pharmaceutical industry was not exactly welcoming this potential replacement of pills with therapeutic treatment (perhaps somewhat reminiscent of the attitude of buggy manufacturers to the new car industry at the start of last century).

Stuart Renwick’s vote of thanks admirably summed up the appreciation of all present.

(Once again, if you Google “John Rasko” you will find a lot of interesting information on the research being done, and an opportunity to contribute to this very worthwhile cause if desired www.cellandgenetrust.org.au.)