Report of 21 July 2016 Meeting
Royal Society
Southern Highlands Branch

Speaker: **Dr David Suhy B.Sc.PhD, Chief Scientific Officer, Benitec Biopharma**

Topic: **The Age of Genomics**

Dr David Suhy arrived to deliver his exciting lecture on the eve of his departure to the USA after spending one week at the Sydney Benitec offices. He lives and conducts his research in San Francisco. The audience of 40 was most appreciative of the fact that he managed to schedule time in the Southern Highlands of NSW to bring the latest updates from Benitec research in its quest to "silence genes for life".

In this relatively small company of fifteen scientists, Dr David Suhy leads the development of all the therapeutic programs. Benitec's new technology combines the application of RNA interference with gene therapy delivery approaches. Long gone are the days when scientists had to set up exhaustive screens of tens of thousands of compounds to identify new drugs to treat human diseases. The "Age of Genomics" has ushered in the unique ability to develop new drugs simply by knowing the sequence composition of the gene.

RNA interference, a technology that awarded its inventors the Nobel Prize for Physiology or Medicine in 2006, is showing remarkable progress in treating a wide variety of human maladies in clinical trials. In this lecture, Suhy explored how this technology can selectively silence disease causing genes. In addition, he described the advances that his team at Benitec Biopharma have made in pairing up this technology with gene therapy based delivery, thus creating the possibility of therapeutics that may significantly impact and improve human health with a single treatment.

Benitec believe their technology has the potential to be the "one shot" cure for a wide range of diseases that are currently addressed by strict ongoing regimens or that have no effective treatment or only palliative care options. Their RNA interference, called DNA-directed RNA interference, or ddRNAi, is being used to develop their pipeline of product candidates for the treatment of several chronic and life-threatening human diseases such as hepatitis B, age-related macular degeneration, or AMD, and oculopharyngeal muscular dystrophy, or OPMD. These diseases have large patient populations, with the exception of OPMD which is a rare disease.

In addition, the company has licensed their ddRNAi technology to other biopharmaceutical companies whose pipeline programs are progressing towards, or are in, clinical development for applications including HIV/AIDS, retinitis pigmentosa, Huntington's disease, cancer immunotherapy and intractable neuropathic pain.

David Suhy holds a BS in Biochemistry and Biophysics from the University of Pittsburgh, earned his PhD in Biochemistry, Molecular Biology and Cell Biology at Northwestern University, and conducted his post-doctoral work at Stanford University. His lecture, despite the intricacy of the subject field, was delivered so well that his excitement for his research flowed on to everyone in the Chevalier Performing Arts Centre, particularly when he quoted an informal note he had written in 2014. It read as follows:

"Today I was fortunate enough to see a gene therapy product that I had invented with two others in 2003, and worked on for the last 11 years, be dosed into the first human subject for clinical testing. This is the first time that RNA interference technology has been administered directly and systematically into man using recombinant viral vectors. Intended as a single shot treatment for hepatitis C, we are evaluating this drug, called TT-034, for safety and efficacy in individuals infected with HCV. What did you do today?"

Anne Wood FRSN