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DEVELOPMENT OF A NOVEL AUTONOMOUSLY REPLICATING DNA VECTOR PLATFORM FOR GENE AND CELL THERAPY

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The research of the DNA Vector Lab has most recently focused on the development of a next-generation non-viral DNA vector for gene and cell therapy and the genetic modification of cells. We have successfully built a novel DNA vector platform, which is uniquely suited for this purpose; it provides persistent expression and episomal maintenance without the use of potentially toxic viral components or the risk of insertional mutagenesis. We have demonstrated for the first time that non-integrating non-viral DNA vectors can be used comparably in applications to integrating vector systems such as Lentiviral and Sleeping Beauty vectors and with genome editing systems such as CRISPR; in some instances, our DNA vectors can be used where viruses cannot. We are applying our DNA vectors for gene therapy, for the generation of isogenic cell lines for tumour modelling and the genetic modification and therapeutic correction of clinically relevant human cells. We have recently made a breakthrough in our DNA vector design which allows the application of our vector in stem cells and primary human cells. For the first time, a non-integrating vector system can provide persistent transgene expression in primary human T-cells without the risk of integration-mediated genotoxicity, and we are currently developing a range of novel DNA vectors for anti-tumour immunotherapy. These DNA vectors can also be used to genetically modify stem cells, and we have recently shown that we can generate stable mouse embryonic cell lines and can generate transgenic mice from these modified cells. This presentation will illustrate the features of these novel DNA vectors and how we intend to leverage this new DNA Vector platform in projects which encompass T-Cell immunotherapy, stem cell therapy and prophylactic gene therapy for cancer.

Biography

Richard Harbottle undertook his undergraduate degree in Biochemistry from the University of St. Andrews in Scotland and subsequently joined a Gene Therapy group at Imperial College London where he did his MSc in Human Molecular Genetics and a PhD in the Development of Non-Viral Gene Therapy. He is currently the Principle Investigator and Group Leader of the DNA Vector Research Group at the German Cancer Research Centre (DKFZ) in Heidelberg. His research focuses on the development of DNA technologies for gene and cell therapy with particular focus on the application of minimally sized DNA vectors which can autonomously replicate within cells without causing any genetic or molecular damage.

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