A one day event organised by the Society for Medicines Research

Whilst the concept, application and enormous potential of gene therapy to repair the direct cause of genetically driven diseases has tantalised and fascinated the scientific community over recent years, the development of suitable treatments using this technology has encountered many challenges. Only few gene therapy trials reported clear clinical benefits and in some, severe adverse events including lethality were observed. Overall, concern and scepticism rose over the further deployment of these strategies. However these attitudes are changing with the introduction of new delivery approaches and gene technologies. Several trials in inherited diseases as well as cancers have demonstrated evidence of efficacy and safety. Additional precision gene editing technologies, in particular CRISPR, are now nearing the clinic, further increasing the possibilities of altering the human genome to treat serious diseases.

This meeting brings together experts from academia and industry to discuss the promise, challenges and reality of gene therapy as a therapeutic approach. This promises to be an exciting and interesting meeting with in depth discussion of how the drug discovery and development community has approached gene therapy in the past, how this will evolve in the future to benefit patients and provide an excellent networking environment for everyone interested in this important and rapidly emerging research area.

PROGRAMME OF SPEAKERS:

Prof. Johan Hyllner, Cell and Gene therapy catapult, UK
"Advancements and challenges in gene therapies from an industrial perspective"

Dr. Els Henckaerts, King’s College London School of Medicine, Guy’s Hospital, London, UK
"Understanding AAV and its potential for gene therapy"

Dr. Fulvio Mavilio, Genethon, Évry, France
"Gene editing for the therapy of monogenic blood diseases"

Dr. Jonathan Appleby, GlaxoSmithKline, Rare Diseases Gene Therapy, Stevenage, UK
"Strimvelis: the first ex-vivo stem cell gene therapy to treat ultra rare disease patients with ADA-SCID"

Prof. Stephen Hyde, Oxford University Gene Medicine Research Group, Radcliffe Department of Medicine, John Radcliffe Hospital, Oxford, UK
"Non-viral CFTR gene therapy in cystic fibrosis patients"

Dr. John Murphy, Pfizer Inc., Rare Disease Research Unit, Cambridge, MA, USA