

## Seminar

### *Gene Therapy, from Hollywood to the bench to the clinic*

Inherited genetic diseases are individually rare but, collectively, affect over 400 million people worldwide; over 75% are children. For many young people with these conditions there is little hope. Most rare genetic diseases are untreatable, causing significant disability, with almost a third dying before their fifth birthday. Licensed genetic treatments are currently available for less than 0.1% of patients with these diseases, leading to significant morbidity and premature mortality. In this lecture, I will explore the public perception of gene therapy and detail one of our clinical-trajectory gene therapy programmes.

Tuesday, 11 November 2025

15h00 - 16h30

CPD Points  
Available

PVT Resource Centre, Ground Floor,

PVT Building, 29 Princess of Wales Terrace, Parktown

**RSVP HERE FOR CATERING**

Prof Simon Waddington



Simon grew up in a declining mill town in the north of England and moved to Imperial College London to do a PhD in kidney disease with Dr Vicky Cattell. On finishing his PhD he still didn't know which direction he wished to take but was incredibly lucky to be invited to a postdoctoral position in a preclinical gene therapy group under the mentorship of Professor Charles Coutelle. First working on cystic fibrosis and Duchenne muscular dystrophy before working on haemophilia in mice which ultimately led to human trials and beyond. He then focused upon gene therapy for early onset childhood diseases.