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DNA-BASED THERAPIES: FROM RECOMBINANT PROTEIN TO GENE THERAPY

Abstract: From the 80s, it became feasible to produce at wish recombinant proteins from cell factories engineered to do so by insertion of genes. It led to the impressive development of biotherapies in several fields of medicine, notably diabetes, cancer, immunological diseases and infectious diseases. DNA is now also being used in the design of new platforms of vaccines as illustrated by the succes of modified adenoviral vectors to protect against Ebola and Covid. Finally, cell modification by addition of a genetic sequence as mediated by viral vectors in vivo or ex vivo has led to success in the treatment of a variety of inherited diseaes such as primary immunodeficiencies, blindness, neuromuscular diseases, hemophilias and hemoglobin disorders. Despite occurrence of adverse events, these therapies reach now for the first one 25 years of protracted efficacy, holding promise to extend application to many more inherited rare diseases. Gene therapy has also transformed immunotherapy of B cell leukemias through the design of CAR (chimeric antigen receptor) T cells. Genome editing, based on modified CRISP,-Cas technology is being tested to provide more precise genetic correction or intervention, offering further perspective of therapeutic development.

About the speaker: Prof. Alain Fischer studied medicine, with a specialization in pediatrics and immunology at the Université of Paris. After completing a postdoctoral fellowship at the University College London, he started independent research in an INSERM unit at the Necker Hospital in Paris. From 2009 to 2016, he was the director of the Institute for Genetic Diseases (Imagine) at Necker University Hospital. Dr Fischer also served as a professor of pediatric immunology at the Université Paris Descartes. From 1996 to 2012, he has served as the director of the pediatric immunology department at the Necker Hospital. Dr Fischer was Professor at College de France (chaire Claude Bernard from 2014 to 2020). Dr Fischer's main research interests are gene therapy, primary immunodeficiency diseases, and the development of the lymphoid system. Dr Fischer received the Louis Jeantet Prize for Medicine in 2001 and the Japan Prize 2015. He is a member of the French and US National Academies of Science and Medicine. He served as president of the French Academy of Science in the years 2023 and 2024.
