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Abstract

Development of stem cell based-gene therapy for immune deficiencies

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Abstract:

Recent clinical trials using patient's own corrected hematopoietic stem cells (HSCs), such as for primary immunodeficiencies (ADA-SCID, X-SCID, CGD, WAS), have yielded promising results in the clinic; endorsing gene therapy to become standard therapy for a number of diseases. However, the journey to achieve such a successful therapy is not easy and several challenges occur along the way. In my presentation, I will address diverse challenges in the development of gene therapy for immune deficiencies using our own experience with RAG1-SCID as example. We will discuss product development (targeting of the therapeutic cells and choice of a suitable vector and delivery method), the proof-of-concept (in vitro and in vivo efficacy, toxicology, and safety) and the final release steps to the clinic (scaling up, GMP procedures/protocols and regulatory hurdles).

Biography:

Frank Staal received his training at Utrecht University (Netherlands) studying Medical Biology receiving his Bachelor of Science (BSc) and Master of Science degrees both with distinction (cum laude). He obtained his PhD degree from Stanford University Medical School in Genetics under the guidance of professors Leonard and Lenore A. Herzenberg. Besides defending a thesis entitled "Redox regulation of signal transduction and HIV expression in T lymphocytes" he learnt two important lessons: 1.the importance of conducting top notch basic science with a translational focus that directly benefits patients and 2. the importance of scientific collaborations rather than hostile competition. He moved back to his native country working at the Dutch Cancer Institute (Amsterdam) and subsequently at Utrecht University as Fellow of the Dutch Royal Academy of Sciences (KNAW) with professor Hans Clevers

Publication of speakers:

- Frank J.T. Staal et al ; Functional definition of a transcription factor hierarchy regulating T cell lineage commitment ,2020 Jul 31
- Frank J.T. Staal et al ; Successful Preclinical Development of Gene Therapy for Recombinase-Activating Gene-1-Deficient SCID, 2020 Mar 31
- FrankJ.T. Staal et al ; CD4+ T-cell counts and interleukin-8 and CCL-5 plasma concentrations discriminate disease severity in children with RSV infection, 2012 Nov 19
- Frank J.T. Staal et al ; Cell intrinsic regulation of external hematopoietic stem cell stress, 2018 May 22
- Frank J.T. Staal et al ; The Effects of Selective Hematopoietic Expression of Human IL-37 on Systemic Inflammation and Atherosclerosis in LDLr-Deficient Mice, 2017 Aug 9.

6th International Conference on Stem Cell Research, Cell and Gene Therapy; July 20-21, 2020; Paris, France.

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