

From stem cells to functional lymphocytes: cell differentiation and gene therapy implementation for RAG-SCID

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Stellingen behorend bij het proefschrift getiteld "From Stem Cells to Functional Lymphocytes"

- 1. "T cell lineage fidelity and commitment requires a minimal network with TCF1 as a pioneer transcription factor initiating the T cell lineage program and Gata3 and Bcl11b regulating opposite aspects", (This thesis, Chapter 2)
- "Autologous lentiviral-based gene therapy using a codon-optimized transgene is an efficient and safe approach to correct RAG deficiency" (this thesis, Chapter 4 and Chapter 5)
- "Single cell characterization of the gene therapy product by the DNA branched technique provides a better perception of the potential therapeutic cells and paves the way for an additional criterion to achieve a successful transplantation outcome" (This thesis, Chapter 6)
- 4. There is a pressing and persisting need to reduce toxicity of existing chemotherapy-based conditioning regimens needed for successful hematopoietic stem cell transplants. Combining a reduced dose chemotherapy-based conditioning with mobilizing agents provide only moderate potential for clinical use. (This thesis, Chapter 7)
- "Actual regulatory network studies are not a small add-on to an existing body of work but instead represent a substantial undertaking that is not for the faint-hearted." (Rothenberg, Ellen V. Current Opinion in Hematology. 2021 - Volume 28 - Issue 1 - p 1-10 doi: 10.1097/MOH.0000000000000628)
- "A close partnership of NBS programs' stakeholders, immunologists, geneticists, and pediatricians-immunologists in different countries is required for moving towards universal SCID screening for all infants." (Blom M, J Clin Immunol. 2021 Jan;41(1):99-108. doi: 10.1007/s10875-020-00886-4)
- 7. The development of a gene therapy-based treatment reminds me of a puzzle: creating and putting together the exceptional pieces together (Newborn screening, conditioning, gene correction, gene product, transplantation, monitoring,...) to achieve a successful one time curative treatment.
- "Essentially all models are wrong, but some models are useful" George E.P. Box (1919-2013) Aimed at the field of statistics it can also be generally applied to scientific research models, specifically for all mouse models used in this thesis.
- "There are no mistakes, just happy accidents" Bob Ross (1942-1995) As a way of thinking to avoid too many frustration
- 10. "Balance is not better time management, but better boundary management. **Balance** means making choices and enjoying those choices" Betsy Jacobson (2018)
- 11. "Nos esforzamos por darle un sentido, una forma, un orden a la vida, y al final la vida hace con una lo que le da la gana" Fernando Aramburu, Patria (2020) (we put energy in thinking, making protocols and experiments to make sense of a scientific question, and then science can happen and surprise you).