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

García Pérez, L.

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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)
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)
3. “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)
5. “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)
6. “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.
8. “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.
9. “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).

