

Quest for the cure: towards improving hematopoietic stem cell based lentiviral gene therapy

Tajer, S.P.

Citation

Tajer, S. P. (2024, December 17). Quest for the cure: towards improving hematopoietic stem cell based lentiviral gene therapy. Retrieved from https://hdl.handle.net/1887/4172518

Version: Publisher's Version

License: License agreement concerning inclusion of doctoral thesis in the

Institutional Repository of the University of Leiden

Downloaded from: https://hdl.handle.net/1887/4172518

Note: To cite this publication please use the final published version (if applicable).

Stellingen behorend bij het proefschrift

Quest for the cure: Towards improving hematopoietic stem cell based lentiviral gene therapy

- 1. Recombinant cytokines are frequently added to human HSC cultures, in efforts to promote HSC expansion. While these cultures typically support expansion of total cell count, they do not succeed in expanding functional HSCs (This thesis).
- 2. Quisinostat substantially increases lentiviral transduction efficiency in HSCs, which is critical for achieving successful gene therapy outcomes (This thesis).
- 3. The inclusion of Quisinostat in LV transduction protocols could significantly reduce the cost of gene therapy by increasing transduction efficiency and reducing the amount of viral vector required (This thesis).
- 4. Investigating the epigenetic regulation of hematopoietic stem cells (HSCs) offers valuable insights into the mechanisms controlling their self-renewal and differentiation, potentially leading to new therapies for expanding HSCs and treating hematological disorders (This thesis).
- 5. Hematopoietic stem cells (HSCs) are inherently heterogeneous, with subsets biased towards specific blood lineages. This lineage bias is regulated by distinct epigenetic mechanisms, allowing HSCs to prime for differentiation while maintaining self-renewal and multipotency (*Yiran meng & Claus Nerlov, Trend in cell biology, 2024*).
- 6. Ensuring fair global access to gene therapy products requires innovative drug development, expedited regulation, and early collaboration between key stakeholders to address health inequities and provide cures for unmet medical needs.
- 7. The essence of scientific progress lies not just in uncovering new data, but in developing fresh perspectives and frameworks that transform how we understand and interpret that data.
- 8. Art and science reflect humanity's endless quest for meaning, each shaped by the constraints of human perception.
- 9. The deeper our understanding of the world and ourselves, the greater our freedom becomes. With understanding, we can approach life with awareness, integrity, and free from the constraints of ignorance or emotional turmoil (*The highest activity a human being can attain is learning for understanding, because to understand is to be free. Baruch Spinoza*)
- 10. Humans are not separate beings, but intricately woven into the fabric of life, and true humanity is shown by compassion and empathy for other's suffering. (Human beings are members of a whole, In creation of one essence and soul/ If one member is afflicted with pain, Other members uneasy will remain- Saadi Shirazi).