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MicroRNA-based gene therapy for Huntington's disease : Silencing the villain

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Citation

Miniarikova, J. (2019, January 24). *MicroRNA-based gene therapy for Huntington's disease : Silencing the villain*. Retrieved from <https://hdl.handle.net/1887/68333>

Version: Not Applicable (or Unknown)

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Issue Date: 2019-01-24

Stellingen behorend bij het proefschrift getiteld:

MIRNA-BASED GENE THERAPY FOR HUNTINGTON'S DISEASE: SILENCING THE VILLAIN

JANA MINIARIKOVÁ

1. AAV-delivered RNAi-based gene therapy induces treatment responses across various cell and animal models of HD. (*this thesis*)
2. RNAi has the potential to silence disease-linked genes and provide therapeutic benefit. (*this thesis*)
3. miR-451 precursors showed a preferable safety profile for RNAi-based therapeutics across multiple species by eliminating the passenger strand. (*this thesis*)
4. Exosome-enriched vesicles can export a therapeutic miRNA outside the iPSCs-derived neuronal cell cultures and will be developed further to PK/PD markers in the clinic. (*modified from this thesis*)
5. Therapeutic miRNAs can be designed to lower mutant HTT transcripts in the nucleus. Further investigations should be performed to understand the mechanism of action and future applications. (*this thesis*)
6. Gene therapy will very likely become a tool to treat genetic disorders in a near future. To this end, we must develop a versatile delivery platform applicable for various indications and targeting different tissues.
7. To accelerate drug discoveries and deliver truly patient-focused drug development, we must build stronger bridges between the industry, academia, regulatory, and public stakeholders. As scientists, we shall start aligning the scientific interest between the industry and academia with patients at the heart. We must educate the public and regulatory bodies.
8. Many of us live in a privilege of building our own story, making dreams reality, and fighting nightmares on our own terms. HD patients and their families are deprived of this luxury. Their lives are pre-written as a dark chapter in the book of humankind, without an option to fight for a better end. It is the responsibility of us all to support them and silence this wrong script.
9. *"I am not young enough to know everything."* – Oscar Wilde (1894)
This quote resonates with my belief to stay grounded and realistic to what one knows in order to progress faster.
10. *"Computers will overtake humans with AI within the next 100 years. When that happens, we need to make sure that computers have goals aligned with ours."*
– Stephan Hawking (2015)
This quote serves as a reminder to keep the conversation about AI ongoing.