Personalized treatment for von Willebrand disease by RNA-targeted therapies

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Stellingen behorende bij het proefschrift

Personalized treatment for von Willebrand disease
by RNA-targeted therapies

1. Current treatment modalities for von Willebrand disease do not benefit all patients, and therefore new treatment options should be explored. *(this thesis)*

2. RNA-targeted therapy is a promising treatment approach for dominant negative von Willebrand disease. *(this thesis)*

3. For a valid comparison between endothelial colony forming cells from patients and healthy controls, cell lines should be matched based on cellular characteristics. *(this thesis)*

4. For a moderate/severe disease like von Willebrand disease, a transient RNA-targeted therapy is preferred over a permanent DNA-targeted therapy. *(this thesis)*

5. Confusion around endothelial progenitor cell identity and function has sometimes let to diminished confidence in the field; this can be prevented when researchers commit to consensus statements. *(modified from: Medina et al. Stem Cells Transl Med, 2017)*

6. Endothelial colony forming cells can provide unprecedented insight into the pathogenesis of von Willebrand disease. *(J. Evan Sadler. Blood, 2013)*

7. The roles of hemostatic components in the vessel wall go far beyond their well-accepted roles in bleeding and clotting. *(José A. López. Blood, 2018)*

8. Investigating the mechanism that cause von Willebrand disease in a patient may better predict the patient's bleeding phenotype and response to treatment than the standard plasma tests that now underlie von Willebrand disease diagnosis.

9. Amid the excitement of the possibility of highly personalized (n-of-1) cures, the goal of improving the health of all must not be forgotten. *(adapted from: Nature Medicine, 2019, in a comment on: Patient-Customized Oligonucleotide Therapy for a Rare Genetic Disease, NEJM, 2019)*

10. You should be judged by your qualities. Being a man or woman is no quality #WomanInSTEM

11. An article has never been rejected for including too many controls. *(adapted from Keith T. Gagnon and David R. Corey. Nucleic acid therapeutics, 2019)*

12. The integrity of modern biomedical sciences is at risk due to the overflow of data. *(adapted from: Siebert, Machesky and Insall. eLIFE, 2015)*