Adenoviral vectors as genome editing tools : repairing defective DMD alleles

Maggio, Ignazio

Stellingen behorende bij het proefschrift:

Adenoviral vectors as genome editing tools: repairing defective *DMD* alleles

1. Adenoviral vectors (AdVs) constitute a valuable platform for introducing RNA-guided nuclease-encoding genes into human somatic cells regardless of their transformation phenotype. (this thesis).
2. The nature of donor DNA templates greatly affects the specificity and accuracy of designer nuclease-assisted genome editing. (this thesis).
3. AdVs encoding designer nucleases can be tailored for achieving efficient 'template-free' *DMD* gene repair in patient-derived muscle progenitor cells. (this thesis).
4. In addition to Duchenne muscular dystrophy, research on AdV-mediated gene editing might also be worth pursuing in the context of other genetic disorders, including those caused by premature stop codons or aberrant splicing. (this thesis).
5. "The success of CRISPR/Cas9 also highlights another general tenet of science: basic, fundamental research can lead to transformative discoveries." (Zhang Feng, *Human Gene Therapy.* 2015, 26: 409-410).
6. "The nuclease field is advancing at a breathtaking rate. Translation of this newfound technical ability into the creation of breakthrough therapeutics that profoundly alter disease outcomes for patients is no longer science fiction; in fact, it is likely that this will be accomplished in the foreseeable future." (Bolukbasi M.F. *et al*, *Nature Methods*. 2016, 13:41-50).
7. "Advances in 'vectorology' may ultimately also cross-fertilize and benefit the field of gene editing as well". (VandenDriessche T. & Chuah M.K. *Molecular Therapy.* 2016, 24:414-416).
8. Recent evidence suggests that Duchenne muscular dystrophy (DMD) is also a stem cell disease. This strengthens the view that satellite cells should be considered as preferential targets for DMD therapies. (Dumont N.A. *et al*, *Nature Medicine*. 2015, 21:1455-1463).
9. One day you will wake up and there won't be any more time to do the things you've always wanted. Do it now. (Inspired by *The Alchemist*, Paulo Coelho, 1988)
10. PhD training, propositions and reality have a lot in common: they are never as you expect.
11. Universities should reward passionate learners and brave dreamers because they are the ones that can change the future.
12. Since body and mind are inseparable, scientists need to do more physical activity in order to reach their highest intellectual potential.

Ignazio Maggio
Leiden, 17 November 2016