Refinement of antisense oligonucleotide mediated exon skipping as therapy for Duchenne muscular dystrophy

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Stellingen

Behorend bij het proefschrift:

Refinement of antisense oligonucleotide mediated exon skipping as therapy for Duchenne muscular dystrophy

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1. Expression of a very low level of novel dystrophin protein already has a significant effect
(this thesis)

2. The membrane damage seen in DMD patients is causing the disease as well as facilitating its treatment
(this thesis)

3. Although some targeting peptides may show better results than others, they are not necessarily the most efficient peptides
(this thesis)

4. The many feedback mechanisms in muscle repair show that appropriate timing of repair mechanisms is crucial; in DMD this timing is lost and none of the repair mechanisms can work properly
(this thesis)

5. It is unlikely that changing the level of any single factor disrupted in DMD will largely normalize muscle pathology

6. A method to reduce fibrosis and fat tissue or replace it with muscle tissue would be a welcome addition to the palette of DMD therapies

7. A significant level of dedifferentiation of myonuclei during regeneration could be an additional explanation for the reduced muscle quality seen in DMD patients

8. Fast muscle repair in mice is one of the reasons mice are not as severely affected by dystrophin deficiency as humans

9. Preclinical research into therapies would go faster without patents, but at the stage of translation to the clinic development would come to a halt

10. Living a normal life doesn't mean being able to run for a bus, it means being able to get transport to the place you want to be

11. Often you do not know what you are doing wrong until you do it right

12. A mere friend will agree with you, but a real friend will argue (Assyrian proverb)