

Stellingen behorende bij het proefschrift

Stem Cell Based Gene Therapy for Pompe's Disease

Gentherapie voor de ziekte van Pompe met stamcellen

door

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1. Successful clinical implementation of stem cell gene therapy for Pompe's disease paves the way for a variety of other inherited diseases (this thesis);
2. Preclinical evaluation in mice of stem cell based gene therapy as a single intervention strategy for Pompe's disease confirms the high enzyme levels required for optimal efficacy of enzyme replacement therapy in patients (this thesis);
3. Stem cell based gene therapy is unique in clearing glycogen storage in all organs, including the brain (this thesis);
4. The current licensing process for gene therapy products is too complicated to be of direct benefit to patients (Mavilio, Nature 2012; 490, 7; this thesis);
5. As a single intervention, stem cell based gene therapy for Pompe's disease is cost-effective as an alternative to the current perpetual enzyme replacement therapy;
6. Gene editing of disease-related single mutations will replace the retroviral integration strategy for some monogenic disorders in the future;
7. The financial crisis does not only affect scientific research in terms of funding, but it also affects the proper education of the general public;
8. It is remarkable that delicious food such as mussels also provides a life-saving tissue glue (In: Haller et al., Acta Biomaterials 2012; 8(12):4365-70);
9. The occurrence of totipotent cells observed after in vivo reprogramming might open new avenues for regenerative medicine (In: M. Abad et al., Nature. 2013 Sep 11. doi: 10.1038/nature12586);
10. Stem cell based gene therapy treated Pompe mice are able to "run from Rotterdam to Paris in 28 days" (this thesis);
11. Life is not like water. Things in life don't necessarily flow over the shortest possible route (In: H. Murakami, 1Q84, 2009).