

## **Cartilage pathology in MPS VI: disease modeling using iPSCs and CRISPR/Cas9**

1. “Identification of disease-associated variants and understanding their mechanism of action is becoming increasingly important to confirm the diagnosis, for genetic counseling, and for development of novel therapies.” — this thesis
2. “While the scientific developments in the field of gene editing are continuing with dazzling speed, it will be important to provide education in the field and to closely monitor and regulate clinical developments.” — this thesis
3. “Although cartilage is one of the major affected tissues in MPS VI patients, the development of cartilage pathology remains poorly understood.” — this thesis
4. “For the development of treatment options, human patient derived model systems that reflect the cartilage pathology in MPS VI are essential.” — this thesis
5. “In contrast to mesenchymal stem cells, hiPSCs have high capacity for self-renewal and are suitable for gene editing, which makes them more suitable for disease modeling of cartilage pathology.” — this thesis
6. “The joy of gene editing is: DNA becomes a drug target.” — Fyodor Urnov
7. “Stem cell research can revolutionize medicine, more than anything since antibiotics.” — Ronald Reagan
8. “There is still debate about whether iPSCs are functionally equivalent to ESCs. This question should be answered only by science, not by politics or business.” — Shinya Yamanaka
9. “All models are wrong, but some are useful.” — George E.P. Box
10. “Our job is improving the quality of life, not just delaying death” Robin Williams
11. “All we have to decide is what to do with the time that is given us.” — J.R.R. Tolkien

Mike Broeders, June 1<sup>st</sup>, 2021