Viral gene therapy approaches for CRB1 retinal disease

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Stellingen behorende bij het Proefschrift:

Viral Gene Therapy Approaches for *CRB1* Retinal Disease

1. Understanding and utilizing the cellular and genetic differences between rodents and humans remains crucial for future research (field).

2. Loss of mouse CRB1 and ablation of CRB2 in rods results in visual function impairment and to an exacerbation of the retinitis pigmentosa phenotype (this thesis).

3. Spectral domain optical coherence tomography is essential to analyze the progression in morphological changes in the diseased retina (this thesis).

4. In *CRB1* patient-derived retinal organoids, the CRB1 protein is largely diminished while *CRB1* transcript levels are similar to the isogenic control (this thesis).

5. The relatively low levels of human *CRB2* in Müller glial cells could be the cause for the inner retinal phenotype of human *CRB1*^{KO} retinal organoids (this thesis).

6. Different adeno-associated viral vector (AAV) capsids display unique cell tropisms; therefore, it is essential to characterize the AAV tropism in the specific research model being employed (this thesis).

7. The utilization of human-induced pluripotent stem cell-derived retinal organoids enables access to previously unavailable materials for a better understanding of retinal disease field).

8. Treatment within the correct window of opportunity is essential for the success of AAV-mediated gene augmentation therapy (field).

9. One should consistently bear in mind the distinction between statistically significant differences and biological relevance (field).

10. The publication of peer-reviewed well-executed experiments with undesired outcome is crucial, as it helps in preventing the repetition of unsuccessful experiments.