

## Viral gene therapy approaches for CRB1 retinal disease

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## Citation

Boon, N. (2023, November 2). *Viral gene therapy approaches for CRB1 retinal disease*. Retrieved from https://hdl.handle.net/1887/3655975

Version:	Publisher's Version
License:	Licence agreement concerning inclusion of doctoral thesis in the Institutional Repository of the University of Leiden
Downloaded from:	https://hdl.handle.net/1887/3655975

**Note:** To cite this publication please use the final published version (if applicable).

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*<sup>KO</sup> 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.