Experimental approaches to treat blinding diseases of the retina

Degenerative diseases of the retina are major causes of blindness worldwide. Age-related macular degeneration (AMD) for example is estimated to affect 196 million people in 2020. No treatment options are available with the exception of anti-VEGF compounds for the less frequent exudative form of the disease. Underlying causes are manifold as diseases can be multifactorial (like AMD) or inherited (mutations in more than 200 genes can lead to retinal degeneration). Even though gene therapy has been established in the clinics for a rare form of Leber’s Congenital Amaurosis, strategies applicable to a large number of patients are needed. Our lab investigates molecular pathways of retinal degenerative diseases and focuses on non-mutating gene therapy strategies that may be applicable to a large number of patients. Specifically, we develop an AAV-mediated RNAi gene therapy for the common dry form of AMD and a CIRSPRa-based approach to induce an endogenous neuroprotective pathway to prolong survival of photoreceptor cells.