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Retinal progenitor cells for treatment of retinitis pigmentosa

Previous studies have directed towards the isolation and transplantation of retinal progenitor cells (RPCs) to the retina of animal recipients that has supported the therapeutic potential of this approach in the setting of retinal degenerations. One mechanism of action is photoreceptor cell replacement, while another is neurotrophic in nature. Our group has focused on this latter approach, including the production of human RPCs under GMP-compatible conditions and formal IND-enabling preclinical studies, leading directly to a phase ½; an open label safety study of intravitreal RPCs in retinitis pigmentosa. This trial includes two patient cohorts based on relative visual function, as well as a dose escalation component. A total of 24 patients have been enrolled as of June 29, 2016, at four dose levels and the first patient has now completed the study, with one year follow up. Initial clinical experience supports the safety of the approach in late stage RP. Progress will be updated at the time of the meeting.

Biography

Henry Klassen has completed his MD and PhD at University of Pittsburgh, Residency at Yale Eye Center and Fellowship at Moorfields Eye Hospital/Institute of Ophthalmology in London. He is an Associate Professor of Ophthalmology and Director of the Stem Cell & Retinal Regeneration Program at University of California, Irvine. He is also the Founder of jCyte, a startup company formed to commercialize retinal progenitor cell-based technology for use in retinal conditions.

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