

Joan & Gordon Bergy, MD

Lecture in Vision Science

4 pm, Friday
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UW Health Sciences, D209



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Gene therapy for retinitis pigmentosa: to knockdown and/or to replace can sometimes be the question

To schedule an appointment,
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Inherited retinal degenerations constitute a major cause of blindness in dogs and humans worldwide, and share a common feature: the degeneration and death of the photoreceptor cells. Although a significant number of genes associated with retinitis pigmentosa (RP) have been identified, there is currently no treatment available.

Dr. Beltran's research is focused at examining the cellular and molecular mechanisms of photoreceptor death in canine models of retinal degeneration, and developing novel therapeutic approaches aimed at curing or slowing the progression of this group of diseases in dogs and human patients.

Studies currently conducted in Dr. Beltran's laboratory include:

- Investigating the signaling pathways of cell death in canine models of X-linked RP caused by mutations in the *RPGR* gene.
- Elucidating the pathogenesis of light-induced retinal degeneration in the T4R rhodopsin mutant dog, a model of human autosomal dominant RP.
- Testing neuroprotective agents for their potential photoreceptor rescue properties.
- Developing and testing corrective gene therapy approaches for *RPGR-XLRP*, and *RHO-ADRP* in collaboration with the Aguirre (Univ. Penn), Jacobson and Cideciyan (Univ. Penn), Hauswirth and Lewin (Univ. Florida) laboratories.
- Testing optogenetic tools as a potential strategy for recovering visual function in patients with late stages of retinal degeneration.