Optimization of Adeno associated virus (AAV) vectors for the treatment of inherited retinal disease

Shannon E. Boye, Ph.D., 273-9342, shaire@ufl.edu

Two major focus areas in the lab are:

1) Developing novel AAV vectors that are capable of transducing photoreceptors following intravitreal injection- Because most inherited retinal degenerations are caused by mutations in photoreceptor-specific genes, there is a great need to develop photoreceptor-targeted gene therapies. Of equal importance is the need to develop an injection procedure which is less invasive than the state of the art (subretinal injection), particularly when an underlying genetic defect leads to a degenerative process and a fragile retina prone to further damage upon surgically induced retinal detachment. Using both rational mutagenesis and directed evolution techniques, the Boye lab seeks to develop AAV vectors that possess an enhanced ability to transduce photoreceptor cells, notably foveal cones, following intravitreal delivery.

2) Expanding AAV vector technology- The Boye lab is actively developing novel, dual AAV vector platforms which are capable of delivering large transgenes. Once thought to be a limiting factor for AAV gene delivery, this technology will allow for the treatment of many diseases associated with mutations in large genes (>~5kb). Specific emphasis is placed on Myosin7a Usher syndrome (USH1B), a debilitating condition characterized by profound deafness at birth and loss of sight within the first decade.

An MSRP student will have the opportunity to work in either of these focus areas.
