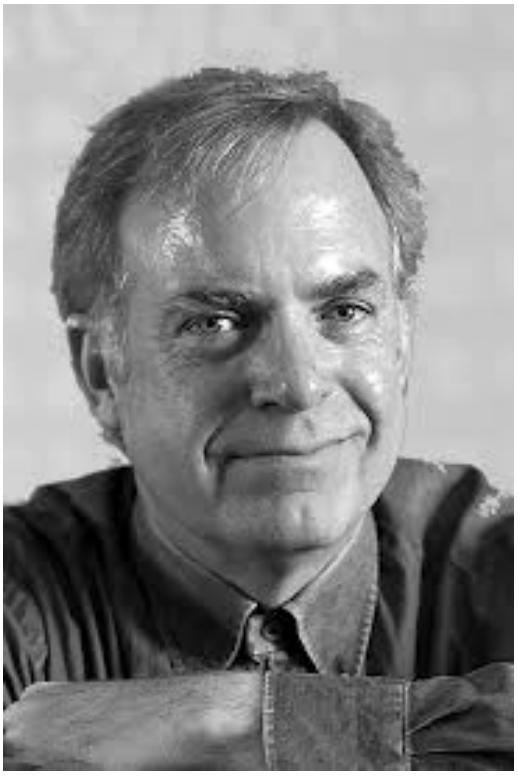
Joan & Gordon Bergy, MD

Inaugural Lecture in Vision Science

11 am, Friday January 11, 2013

Hogness Auditorium

UW Health Sciences, A420



John Flannery, PhD

Molecular and Cellular Biology, UC-Berkeley

Directed evolution of viral vectors for retinal gene therapy

Gene therapy has vast potential for treating and potentially curing a number of retinal diseases including glaucoma, age-related macular degeneration, and inherited photoreceptor diseases. However, gene delivery technologies require significant improvements in cellular targeting, efficiency, and safety before promising findings in animal studies are translated to the clinic. In particular, for retinal gene therapy it would be highly advantageous to transduce a single cell type that spans the entire retina after an intravitreal injection of a gene delivery vehicle for the subsequent secretion of a general neuroprotective factor throughout the retina. Unfortunately, there is no vector capable of efficiently infecting the cell type that meets these needs, Müller cells. Vectors based on adeno-associated virus (AAV) have proven themselves to be highly promising in numerous retinal disease models, but they are also incapable of Müller cell infection. We have developed novel lentiviral vectors with new properties, including altered receptor binding, which are capable of efficient Müller cell transduction. In parallel, the basic mechanisms of AAV transduction of Müller cells will be explored in order to develop new AAV pseudotypes capable of Müller cell transduction. The novel approaches developed in this work will have general impact for the molecular engineering of enhanced viral gene delivery vehicles, and future work will focus on testing these vectors in an animal model of retinal disease.

Reception precedes the presentation in the Hogness Lobby

To schedule an appointment, email: mneitz@uw.edu