



DREXEL UNIVERSITY

College of
Medicine

Institute for Women's Health and Leadership

2018 Marion Spencer Fay Award Honoree

Jean Bennett, MD, PhD



Jean Bennett, MD, PhD, the F.M. Kirby Professor of Ophthalmology, professor of cell and developmental biology, and co-director of the Center for Advanced Retinal and Ocular Therapeutics (CAROT) at the University of Pennsylvania Perelman School of Medicine, is a physician-scientist with experience/expertise in molecular biology, vector development and gene therapy translational studies. She has developed gene transfer approaches to test treatment strategies for retinal degenerative and ocular neovascular diseases, to elucidate retinal differentiation pathways and to identify pathogenetic mechanisms that lead to blindness.

Her research, conducted at UPenn over the past 26 years, has established the scientific underpinnings that made it possible to test the first potential definitive retinal gene therapy treatment for patients with blinding retinal degenerations. This experience is documented by more than 120 peer-reviewed publications on gene therapy (including the first publication to demonstrate proof-of-concept of retinal gene therapy). Her lab has established a true “from bench to bedside” program, and thus she is familiar with steps necessary to go from proof-of-concept all the way to testing of safety and efficacy in humans with blinding disease, including obtaining the appropriate molecular diagnoses.

Dr. Bennett's work led to a gene therapy trial that demonstrated efficacy and enrolled the first pediatric subject to undergo gene therapy for a non-lethal disease. The team completed both a follow-on (readministration) trial and a randomized, controlled Phase 3 registration gene therapy study for congenital blindness. Dr. Bennett was the scientific director of all three studies, and her longtime collaborator (and husband), Dr. Albert Maguire, was the PI. This work led to the first approved gene therapy drug for retinal disease worldwide and the first approved gene therapy for genetic disease in the U.S. Dr. Bennett's team continues to expand the targets and is excited about the potential for developing gene-based treatments for other devastating inherited diseases.