
BIO SHEET

Jean Bennett, M.D., Ph.D.

Jean Bennett, M.D., Ph.D., tenured professor of Ophthalmology and Cell and Developmental Biology, and vice chairman for research in Ophthalmology at the University of Pennsylvania (Penn) School of Medicine, is a pioneer in retinal gene therapy and internationally recognized for her work in this field. 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. In addition, her laboratory has developed gene transfer approaches to treat syndromic diseases resulting in vision loss, studies the immune response of the eye to viral vectors, and characterizes and utilizes cell specific promoters for the delivery of therapeutic nucleic acids. Recently she was named the F.M. Kirby Professor of Molecular Ophthalmology. Her research, conducted at Penn over the past 16 years, has established the scientific underpinnings which made it possible to test the first potential definitive retinal gene therapy treatment for patients with blinding retinal degenerations. Dr. Bennett is the principal investigator of the Foundation Fighting Blindness-funded The Children's Hospital of Philadelphia (CHOP)-Penn Pediatric Center for Retinal Degenerations and the Scientific Advisor of a clinical trial that evaluates the safety and efficacy of gene augmentation for a Leber Congenital Amaurosis. This study was initiated in the fall of 2007 at The Children's Hospital of Philadelphia.

Katherine A. High, M.D.

An internationally prominent hematologist and researcher, Katherine A. High, M.D., is the director of the Center for Cellular and Molecular Therapeutics at The Children's Hospital of Philadelphia. She is the William H. Bennett Professor of Pediatrics at the University of Pennsylvania School of Medicine, and a Howard Hughes Medical Institute investigator. She also is a past president of the American Society of Gene Therapy and is a member of the Institute of Medicine. Dr. High studies novel therapies, in particular gene transfer, for genetic disorders such as hemophilia at The Children's Hospital of Philadelphia. She leads a National Institutes of Health-funded laboratory and has contributed scores of papers to the scientific literature.

Albert M. Maguire, M.D.

Albert Maguire, M.D., is an associate professor of Ophthalmology at the Scheie Eye Institute at University of Pennsylvania's School of Medicine. He is a senior member of the Retina Service at the Scheie Eye Institute and in the Department of Pediatric Ophthalmology at The Children's Hospital of Philadelphia. He is also a senior investigator at Penn's F.M. Kirby Center for Molecular Ophthalmology. Dr. Maguire has had a long-standing interest in retinal gene transfer/gene therapy and developed surgical approaches with which to deliver genes in proof-of-concept studies involving gene therapy. He has trained numerous investigators at dozens of institutions nationally and internationally on technical aspects of these procedures and has extensive experience performing subretinal injections of viral vectors. Dr. Maguire has published widely on applications of gene transfer approaches for retinal degenerative and ocular neovascular diseases. Dr. Maguire has participated in numerous clinical trials for retinal diseases. His research, conducted at Penn over the past 16 years, has resulted in approval and implementation of gene therapy clinical trials in the United States for Leber Congenital Amaurosis. He is currently the principal investigator of a Phase 1 Safety Study in Subjects with Leber Congenital Amaurosis (LCA) Using Adeno-Associated Viral Vector to Deliver the Gene for Human RPE65 into the Retinal Pigment Epithelium. This study was initiated in the fall of 2007 at The Children's Hospital of Philadelphia.

Alberto Auricchio, M.D.

Alberto Auricchio, M.D., is associate professor of Medical Genetics at the “Federico II” University in Naples and Full Investigator at the Telethon Institute of Genetics and Medicine in Naples. Dr. Auricchio’s research is focused on gene therapy of retinal and metabolic diseases using adeno-associated viral vectors. Dr. Auricchio received the 2006 Outstanding New Investigator Award of the American Society of Gene Therapy and is a member of the Editorial boards of *Human Gene Therapy* and *Molecular Therapy*.

Francesca Simonelli, M.D.

Francesca Simonelli, M.D., is the principal investigator of a grant from the Telethon Institute of Genetics and Medicine (TIGEM) to select, study and evaluate Italian patients with LCA treated in the clinical trial of gene therapy performed at The Children’s Hospital of Philadelphia. Dr. Simonelli is an associate professor of Ophthalmology at the Second University of Naples, an institution with long experience in researching inherited retinal diseases. She received her medical degree in 1983 from the Second University of Naples, where she also served her residency in ophthalmology. The president since 2004 of the Italian Society for Genetic Eye Diseases, Dr. Simonelli also serves on the Eye Working Group of TIGEM. She has contributed dozens of publications to ophthalmology journals, and has received a certificate of excellence from the European Association for Vision and Eye Research.