

V. VINOD MOOHA, MD
 Professor of Ophthalmology
 University of Texas Southwestern



A BREAKTHROUGH IN SIGHT

FOCUS: Precision medicine for age-associated vision loss.

Fuchs' endothelial corneal dystrophy (FECD) is an age-related degenerative disorder in which the inner layer of the cornea degenerates, leading to corneal swelling, scarring, and loss of vision. In the United States, FECD affects one in 25 individuals over age 40 and is the leading indication for corneal transplantation, a procedure with post-op challenges and potentially serious complications.

In 2005, Dr. Mootha was recruited to the University of Texas Southwestern to implement new corneal transplant procedures for FECD. After years of performing corneal transplants, he felt that in the long run he could help more patients by studying the genetic basis of the disease.

Work done by Dr. Mootha's lab and others has implicated toxic accumulation of expanded repeat RNA in the TCF4 gene as

the molecular genetic basis of FECD. "We have developed small molecules called oligonucleotides that block the critical mutant repeat RNA that causes this disease, and in turn reverse the accumulation of this toxic RNA," Dr. Mootha says.

"With the help of our Harrington team, we aim to further optimize our lead compound, develop formulations for safe and effective delivery, and identify biomarkers to facilitate human clinical trials," he says. "If all goes well, we will have a therapy for FECD that's as simple as a topical application or local injection administered in the clinic."

In addition, Dr. Mootha's work may hasten understanding of over twenty devastating neurodegenerative disorders caused by DNA repeat expansions.

"Our Harrington advisors bring vast scientific and industry knowledge to the table."

Harrington Discovery Institute

 University Hospitals | Cleveland, Ohio