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A SCIENTIFIC SHOT IN THE DARK PAYS OFF

FOCUS: A new approach for neuroprotection of photoreceptors as a treatment for retinitis pigmentosa.

Retinitis pigmentosa (RP) is a group of inherited retinal disorders that lead to degeneration of photoreceptors (rods and cones) and blindness. With the exception of a costly gene replacement therapy for a subtype of Leber congenital amaurosis, there is no approved therapy for RP.

For decades, Dr. Martin has worked at the interface between chemistry and biology—making molecules and finding those having biological activity. In 2011, he sent a set of compounds to the Psychoactive Drug

Screening Program at the University of North Carolina with the goal of identifying ones that selectively interact with receptors in the central nervous system. “I didn’t know what I was looking for...I just wanted to find something different,” he says.

“We were fortunate to find compounds that bound to the sigma 2 receptor, an enigmatic protein we helped identify as TMEM97,” Dr. Martin says. Although TMEM97 had never been associated with any degenerative retinal condition, he discovered TMEM97-

binding compounds that protect rods and cones in an RP model. “This led us to believe that modulating this receptor would be a unique, gene-independent approach to treat any form of RP.”

With help from the Harrington Discovery Institute, Dr. Martin and his collaborators seek to develop a drug for RP that acts by a completely new mechanism of action and may be eventually applied to other retinal degenerative diseases.

“The consultants Harrington has provided have led us to think of new, potentially better molecules.”

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