CONVERTING RODS TO CONES:

SEEING IS BELIEVING

FOCUS: *Developing a treatment to prevent blindness and compromised sight in patients with retinitis pigmentosa (RP).*

Dr. Reh's years of dedicated work on retinal regeneration started with his fascination at how amphibians regenerate eyes and limbs. But stimulating the regeneration process in patients with retinal disease is still some years off, and now he and his team may have found a faster way to help patients with retinitis pigmentosa.

RP is a group of rare genetic disorders that involve a breakdown and loss of cells in the retina, with vision worsening until usually only a small area of central vision remains—akin to looking through a straw. Often all vision is eventually lost.

Studies of the developing retina showed that the rods and the cones originate from the same progenitor, but a molecular switch, called NR2E3, makes the cells become rods. “Since RP starts in the rods, we thought that if we could convert the rods to cones, the newly formed rod-cone hybrid cells might not die from the disease,” Dr. Reh says. “So we looked for drugs that could inhibit the function of NR2E3, and discovered a small molecule we dubbed Photoregulin3 that seems to work.”

In mice, Photoregulin3 partly converted the rods to a more cone-like state, and sure enough this stopped them from degenerating. Funding from the Harrington Discovery Institute allowed Dr. Reh's team to acquire the key data to start a biotech company, whose aim is to make the molecule more potent, and if possible, adapted to work as a pill.

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AN ESTIMATED
100,000
PEOPLE IN THE U.S. HAVE
RETINITIS PIGMENTOSA

IMPACT WISH:

“With a pill taken perhaps once a week, people with RP will retain their eyesight throughout their lives.”

Harrington Discovery Institute
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