In the news

VISIBLE IMPROVEMENT

Two independent studies recently reported significant improvements in the vision of legally blind individuals after gene therapy. The subjects were all sufferers of Leber's congenital amaurosis (LCA), a condition caused by a defect in a gene (*RPE65*) that encodes an enzyme required for rhodopsin production in the retina. People with LCA, who lack this enzyme, have impaired vision from birth that becomes progressively worse with age.

Both studies (one conducted in Britain and one conducted in the United States) used genetically engineered viruses to deliver normal copies of the *RPE65* gene and thus hopefully enable the individuals to express the encoded enzyme. The results were very encouraging: all three subjects of the US study showed improved visual acuity and increased light-sensitivity. "I'd call it a dramatic response," says Jean Bennet, one of the lead authors. (*Washington Post*, 28 April 2008).

Although only one of the three subjects of the British study showed any improvement, the scale of this improvement was impressive: the subject's light-sensitivity increased 100-fold, significantly enhancing his ability to navigate at night. Robin R. Ali, the lead author of this study, says that, "This really paves the way for developing a treatment for people who have so far had no prospect of a cure." (*Guardian*, 28 April 2008.)

Far greater improvements would be achieved by providing the therapy at an earlier stage, when the degeneration is less advanced. "The sooner you can intervene, the better", says Katherine High of the Children's Hospital of Philadelphia (*Science*, 2 May 2008). Furthermore, High adds that the results are "...important for the entire field of gene therapy." (*Bloomberg*, 28 April 2008.)

The studies therefore offer hope that safe and effective treatments for many sight disorders might almost be upon us.

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