Blind rats' vision restored by genetic modification, humans next?

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A new technique that has the potential to treat inherited diseases by removing genetic defects has been shown for the first time to hinder retinal degeneration in rats with a type of inherited blindness, according to a Cedars-Sinai study.

A research team at the Cedars-Sinai Board of Governors Regenerative Medicine Institute focused on inherited retinitis pigmentosa, a degenerative eye disease with no known cure that can lead to blindness. The researchers used a technique known as CRISPR/Cas9 to remove a genetic mutation that causes the blindness disease. CRISPR/Cas9 is adapted from a strategy used by bacteria to fight invading viruses. Although the study involved rats, it is an important milestone because of its potential implications for humans.

"Our data show that with further development, it may be possible to use this gene-editing technique to treat inherited retinitis pigmentosa in patients," said Shaomei Wang, MD, PhD, a research scientist in the institute's Eye Program and associate professor of Biomedical Sciences. Wang was the senior author of the study, published in the journal *Molecular Therapy*.

Retinitis pigmentosa is a class of diseases in which patients experience night blindness in the early stages, along with atrophy and pigment changes in the retina, constriction of the visual field and eventual blindness, according to the National Institutes of Health. While rare overall, it is one of the most common inherited diseases of the retina, affecting about one in 4,000 people in the U.S. and Europe.

Read full, original post: Gene editing technique improves vision in rats with inherited blindness