

One-two punch could be key in treating blindness

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András Komáromy and colleagues discovered that a combination therapy restored vision in dogs with an inherited retinal disorder. Credit: Photo by Tom Genarra

Researchers have discovered that using two kinds of therapy in tandem may be a knockout combo against inherited disorders that cause blindness. While their study focused on man's best friend, the treatment could help restore vision in people, too.

Published in the journal *Molecular Therapy*, the study builds on earlier work by Michigan State University veterinary ophthalmologist András Komáromy and colleagues. In 2010, they restored day vision in dogs suffering from achromatopsia, an inherited form of total [color blindness](#), by replacing the [mutant gene](#) associated with the condition.

While that treatment was effective for most younger dogs, it didn't work for canines older than 1 year. Komáromy began to wonder if the older dogs' cones – the [photoreceptor cells](#) in the retina that process daylight and color – might be too worn out.

"[Gene therapy](#) only works if the nonfunctional cell that is primarily affected by the disease is not too degenerated," he said. "That's how we came up with the idea for this new study. How about if we selectively destroy the light-sensitive part of the cones and let it grow back before performing gene therapy? Then you'd have a younger, less degenerated cell that may be more responsive to therapy."

So, Komáromy and colleagues recruited more dogs with achromatopsia between 1 and 3 years old. To test their theory, they again performed gene therapy but first gave some of the dogs a dose of a protein called CNTF, which the [central nervous system](#) produces to keep cells healthy. At a high enough dose, its effect on [photoreceptors](#) is a bit like pruning flowers: It partially destroys them, but allows for new growth.

"It was a long shot," said Komáromy, associate professor in MSU's Department of Small Animal [Clinical Sciences](#).

But it worked.

"We were just amazed at what we found," he said. "All seven dogs that got the combination treatment responded, regardless of age."

While achromatopsia is quite rare, Komáromy said it's a good model disease for other disorders affecting the photoreceptors, conditions that constitute a major cause of incurable blindness in dogs and humans. Those disorders affect individuals of both species in much the same way, so the combination treatment's promise isn't just for Fido.

"Based on our results we are proposing a new concept of retinal therapy," he said. "One treatment option alone might not be enough to reverse vision loss, but a combination therapy can maximize therapeutic success."

Provided by Michigan State University

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