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UCSD team adapts CRISPR to edit RNA for disease therapies

The CRISPR gene-editing technique is revolutionizing genetics research. (July 20,2017) (Sign up for our free video newsletter here http://bit.ly/2n6VKPR)

By **Bradley J. Fikes**

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UC San Diego researchers have invented a technology that offers a possible new way to fight genetic diseases, and have built a San Diego biotech company around their discovery.

The scientists adapted the powerful CRISPR/Cas9 DNA editing system, which has transformed the world of biology, to work on RNA, the messenger molecule that carries DNA's instructions into cells.

The system worked in cell cultures to stop production of RNA involved in forms of myotonic dystrophy, ALS and in Huntington's disease. These are all incurable genetic diseases that can be fatal. This RNA can be toxic in itself, or produce abnormal proteins that cause disease.

About 95 percent of the disease-causing RNA was destroyed in the cell cultures.

The scientists have formed a San Diego biotech company called Locana to bring the technology to patients. Several years of development, including animal testing, is expected before that can happen.

The study was published Thursday in the journal *Cell*. Go to j.mp/rnacrispr to get the study. Gene Yeo was the senior author. The first authors were David Nelles and Ranjan Batra, postdoctoral researchers in Yeo's lab.

CRISPR/Cas9 cuts DNA at specified targets, inactivating or altering gene sequences. It has been used for such feats like editing the genome of human embryos. The adapted system doesn't target the genome, but only RNA.

Scientists led by UC San Diego's Gene Yeo modified CRISPR so it doesn't target DNA and instead attacks specific RNA sequences, while leaving others alone. This specificity is vital for therapeutic purposes. It is delivered by an adenovirus, a virus commonly used in gene therapy.

The study expands on previous research that showed the RNA-adapted CRISPR could track RNA as it moves around cells. It didn't affect RNA production, however.

That study included Jennifer Doudna, a UC Berkeley scientist who helped pioneer the CRISPR system. Doudna is on Locana's scientific advisory board, Yeo said. He and Nelles are co-founders of Locana.

The Cas9 component, a protein that destroys the target, was too large to be delivered by the virus. So the team cut the protein's size by removing unnecessary parts used to cleave DNA. The result, RCas9, is guided by an accompanying RNA molecule to the target site.

Yeo said he expects the viral delivery system will remain effective for perhaps five to 10 years. That's important because RNA is continually being produced from DNA, so the new disease-causing RNA must likewise be destroyed.

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