San Diego Scientists Tweak Gene Editing Tool To Target RNA

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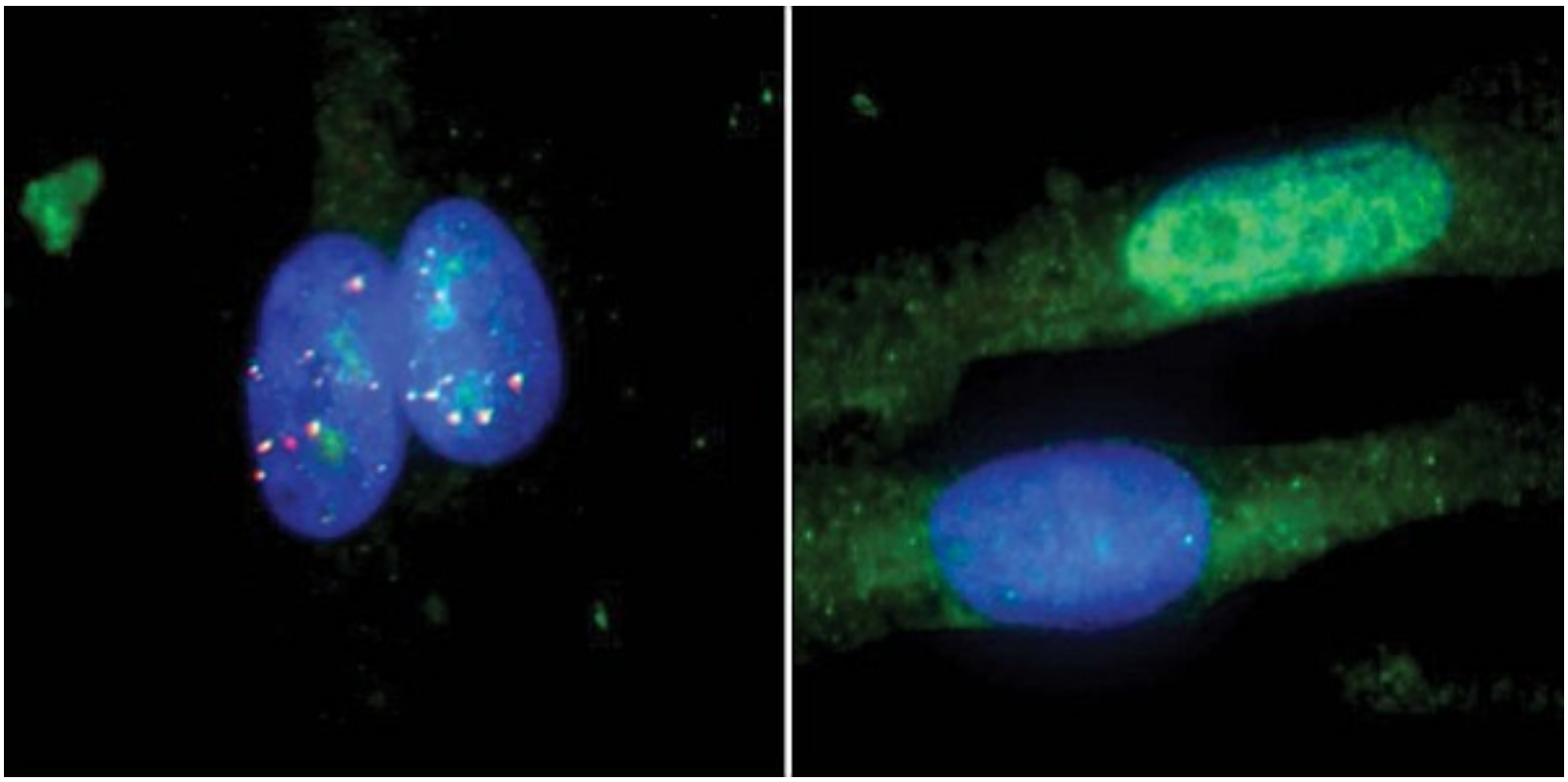
By David Wagner



Credit: UC San Diego Health

Above: Muscle cells from a patient with myotonic dystrophy, used in recent UC San Diego experiments, are seen in this undated photo.

In recent years, scientists experimenting with plants, animals and even human embryos have relied on the powerful gene editing tool CRISPR to make precise changes to DNA.

But a team of UC San Diego scientists have been repurposing CRISPR to target RNA, the molecule crucial for translating the genetic information stored in DNA.

In a new study published Thursday in the journal Cell, the researchers report using their technique to prevent the kinds of RNA errors involved in diseases like Huntington's and certain forms of ALS.

"It's a programmable system, so we could change the sequence of our guide RNAs to target different mutations that cause different diseases," said Ranjan Batra, who co-authored the study during his time as a postdoctoral researcher in Gene Yeo's lab at UC San Diego.

The researchers carried out their experiments in human cells. Batra said the researchers hope to test their approach in mice, and eventually move into human trials. Yeo, the senior author on the new paper, has co-founded a company called Locana to develop the technique.

Batra said when it comes to developing human therapies, targeting RNA could end up being preferable to editing DNA. In some experiments, CRISPR has been associated with "off-target effects," or unintended changes to genes scientists were not trying to alter.

"If you're targeting RNA, you're actually not interfering or messing up the DNA that's in the cell already," said Batra. "Any changes we make to DNA are permanent. Any changes that we make to RNA stay for only a few hours until a new RNA is made."

Scientists who were not involved in the study were impressed by the researchers' proof of concept. But they told KPBS more work is needed before we will know whether this technique can successfully combat disease beyond cells in a dish.

"It's tempting to imagine how this exciting capability might be used to treat genetic diseases one day, although many additional studies are needed to test this possibility," said Harvard University professor David Liu.

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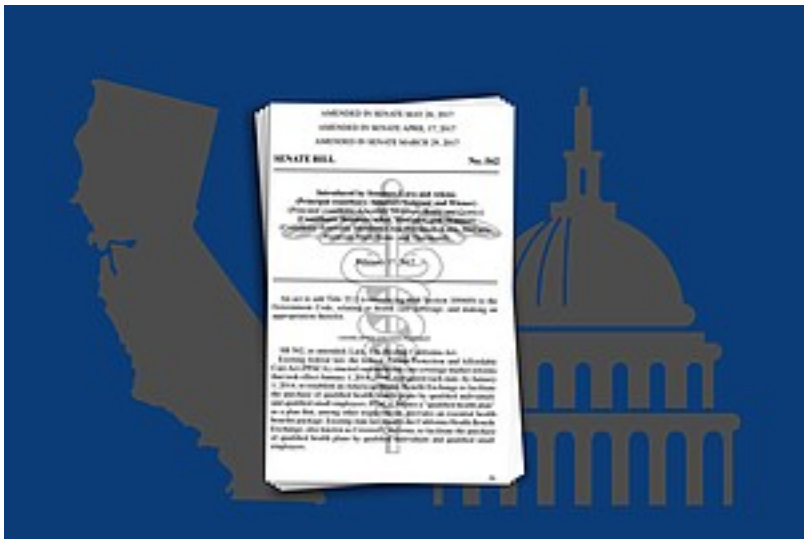

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