'Excitement is finally catching on': Why RNA editing may be more promising than CRISPR

CRISPR editing — at least as a therapeutic technique in people — has turned out to be more difficult than initially thought. Researchers have documented ways that Cas9, one of the enzymes used in CRISPR gene editing, could trigger immune responses, or cause accidental changes to the genome that would be permanent. RNA editing, by contrast, could allow clinicians to make temporary fixes that eliminate mutations in proteins, halt their production or change the way that they work in specific organs and tissues. Because cells quickly degrade unused RNAs, any errors introduced by a therapy would be washed out, rather than staying with a person forever.

Excitement over RNA editing is finally catching on. In 2019, researchers published more than 400 papers on the topic, according to data from Scopus, an abstract and citation database. A handful of start-up companies are beginning to use RNA-editing systems to develop potential treatments for everything from genetic diseases such as muscular dystrophy to temporary maladies such as acute pain. And although RNA-based drugs have had difficulty reaching the market owing to challenges in delivery and tolerance, some regulatory approvals in the past few years might help to pave the way for RNA-editing therapies.

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