CRISPR 2.0: Tweaking gene editing tool to revolutionize precision medicine

CRISPR has been hailed as one of the most promising gene editing technologies and promises to revolutionize precision medicine and eradicate genetic diseases. However, the technique is not perfect and needs improvements. A new paper improves this system by creating a novel Cas9 fusion: ExoCas9. This fusion produces a higher rate of overall gene targeting and bias the spectrum of DNA lesions produced to favor much longer deletions.

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We anticipate that this variant will also allow for mutation of a larger spectrum of genomic targets that cannot be targeted with traditional Cas9 in zebrafish, other model organisms, and even humans.

The CRISPR-Cas9 system has the great potential to cure numerous genetic diseases within our lifetime. Gene editing has already been shown to cure the rare skin disease epidermolysis bullosa (EB) in a young boy through gene therapy. Nonetheless, scientists as a whole are still fine-tuning this relatively new method and ExoCas9 is not the first published Cas9 fusion. For example, researchers used a novel Cas9 fusion to the I-TevI nuclease domain (TevCas9) to result in two, non-compatible breaks in the DNA that consistently result in 33-36 nucleotide deletions (Wolfs et al., 2016). Nonetheless, it is through small innovations like ExoCas9 and others that some time in the not so distant future the use of gene editing to cure genetic diseases will be commonplace!

Editor's note: Thomas Clements is a Ph.D. student at Rice University and will be transitioning to a career as a Lecturer in the Biological Sciences Department at Vanderbilt University starting in Fall 2018

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