Cystic fibrosis, sickle-cell anemia could be corrected in embryos with new CRISPR variant

Since the discovery of the genome-editing tool CRISPR/Cas9, scientists have been looking to utilize the technology to make a significant impact on correcting genetic diseases. Technical challenges have made it difficult to use this method to correct disorders that are caused by single-nucleotide mutations, such as cystic fibrosis, sickle-cell anemia, Huntington's disease, and phenylketonuria. … [Researchers] have just used a variation of CRISPR/Cas9 to produce mice with single-nucleotide differences. The findings from this new study were published recently in *Nature Biotechnology* in an article entitled "Highly Efficient RNA-Guided Base Editing in Mouse Embryos."

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The most frequently used CRISPR/Cas9 technique works by cutting around the faulty nucleotide in both strands of the DNA and cuts out a small part of DNA. In the current study, the investigators used a variation of the Cas9 protein (nickase Cas9, or nCas9) fused with an enzyme called cytidine deaminase, which can substitute one nucleotide into another—generating single-nucleotide substitutions without DNA deletions.

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"The next goal is to correct a genetic defect in animals. Ultimately, this technique may allow gene correction in human embryos," [remarked senior study investigator Jin-Soo Kim].

The GLP aggregated and excerpted this blog/article to reflect the diversity of news, opinion, and analysis. Read full, original post: An Efficient Single-Nucleotide-Editing CRISPR