

Induced liver regeneration enhances CRISPR/Cas9-mediated gene repair

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Use of thyroid hormone to boost hepatocyte proliferation enhanced the efficiency of CRISPR/Cas9-mediated gene correction in the mouse liver. This dietary induction of hepatocyte regeneration may be a viable clinical strategy to enhance gene repair in the liver, according to the peer-reviewed journal *Human Gene Therapy*.

The study was done in a mouse model of tyrosinemia type 1. "In neonatal mice, a [gene correction](#) frequency of ~10.8% of hepatocytes was achieved," said Qing-Shuo Zhang, Oregon Health & Science University and coauthors. "The efficiency in adult mice was significantly lower at ~1.6%."

Use of thyroid hormone T3 to temporarily induce hepatocyte division in the adult mice led to a significant increase in the gene correction efficiency to 3.5%.

"The promise of [gene editing](#) for [human gene therapy](#) is being realized initially with ex vivo manipulation of stem cells and lymphocytes and in small organ targets like the retina. If gene editing becomes efficient enough to correct [genetic defects](#) in vivo in the liver, it could then be used to treat a much wider variety of disorders. The work in this paper moves the field closer to that goal," according to Editor-in-Chief of *Human Gene Therapy* Terence R. Flotte, MD, Celia and Isaac Haidak Professor of Medical Education and Dean, Provost, and Executive Deputy Chancellor, University of Massachusetts Medical School.

More information: Qing-Shuo Zhang et al, Induced Liver Regeneration Enhances CRISPR/Cas9-Mediated Gene Repair in Tyrosinemia Type 1, *Human Gene Therapy* (2020). [DOI:](#)

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