Article

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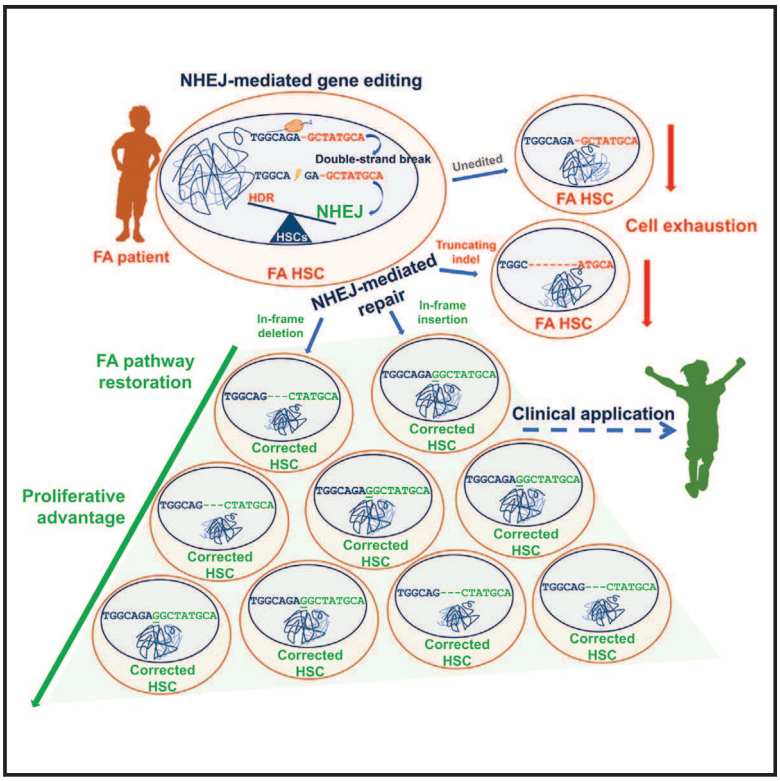
NHEJ-Mediated Repair of CRISPR-Cas9-Induced DNA Breaks Efficiently Corrects Mutations in HSPCs from Patients with Fanconi Anemia

Graphical Abstract



Highlights

- NHEJ-mediated gene editing enables highly efficient editing in human long-term HSCs
- NHEJ-mediated editing restores mutant coding frames across FA complementation groups
- Corrected FA-HSCs have a marked proliferative advantage *in vitro* and *in vivo*

Authors

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In Brief

NHEJ is an error-prone DSB repair mechanism typically exploited to create gene knockouts. Román-Rodríguez and colleagues show efficient CRISPR-Cas9-mediated repair of mutated Fanconi anemia genes using NHEJ to generate compensatory mutations that correct the phenotype of FA patient HSCs, suggesting a simple and feasible clinical approach for monogenic hematopoietic diseases.

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