Viewpoint: We need to know if CRISPR works in monkeys and possible off-target effects before we start human trials

Sometime this year, people in the US and Europe will start getting treated for diseases using the gene-editing tool CRISPR, but a big question remains—will it actually work? Our primate cousins may hold the answer.

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Some of the ongoing CRISPR experiments in monkeys involve blood disorders like sickle-cell disease and beta thalassemia.

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One concern with CRISPR has been the possibility that it will make accidental cuts to other parts of the genome that aren't being targeted. [Researcher Hans-Peter] Kiem says he hasn't yet seen any of these so-called off-target effects in monkeys, but his team is sequencing the genomes of the CRISPR-treated monkeys to make sure.

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Researchers could get a better sense of whether a CRISPR therapy in monkeys will translate to cures for people if the animals had the same disease-causing mutations as humans.

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[Researcher Jon] Hennebold says scientists need to move cautiously into human clinical trials of CRISPR because so little work has been done in monkeys. There's still a question of whether CRISPR will cause immune reactions, for example, and off-target effects are a concern.

"We just don't know enough about what it does outside of the region we want to modify," he says.

Read full, original post: CRISPR trials are about to begin in people—but we still don't know how well it works in monkeys