How Casgevy came to be: How researchers found gene editing targets for newly-approved sickle cell drug

The world’s first commercial gene-editing treatment is set to start changing the lives of people with sickle-cell disease. It’s called Casgevy, and it was approved last month in the UK.

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CRISPR is revolutionary because scientists can so easily program it to cut DNA at precise locations they choose.

But where do you aim CRISPR? That’s the lesser-known story of the sickle-cell breakthrough. The disease is caused by faulty hemoglobin, the molecule that carries oxygen in the blood. To cure it, though, Vertex and its partner company, CRISPR Therapeutics, aren’t fixing the genes responsible for the mutation that leaves those molecules misshapen. Instead, the new treatment involves a kind of molecular bank shot—an edit that turns on fetal hemoglobin, a second form of the molecule that we have in the womb but lose as adults.

You can think of how the edit works as a kind of double negative. It adds a misspelling to the turbo-booster of another gene, BCL11A, that is itself what inhibits the production of fetal hemoglobin in adult bodies. Without that booster, there’s less inhibition, and more fetal hemoglobin. Got it?

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Given the media accolades for CRISPR editing, many people don’t realize it’s really best at ripping scars into genes, not making stylish rewrites (although that is coming).

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