Gene transfer therapies are the ‘next big thing’ in medicine. Here’s how gene editing works and the companies behind it

The breakthroughs made possible by gene editing were shown in the Jan. 6 news that base editing had repaired a genetic defect in lab mice suffering from progeria, a disorder that prematurely ages and kills children born with the mutation.

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Most first-generation gene therapies use a hollowed-out virus to carry synthetic versions of a gene into cells. The transferred gene isn’t integrated into the cellular DNA, but the cell can still use the instructions to produce functional versions of the missing protein.

Hundreds of such gene-augmentation therapies are in clinical trials. BioMarin Pharmaceutical, UniQure , and Pfizer are each in Phase 3 trials on therapies to treat hemophilia, the bleeding disorder resulting from a mutation in the gene for a blood-clotting protein. Pfizer is also racing Sarepta Therapeutics to treat Duchenne muscular dystrophy with transferred genes that can produce working versions of a muscle protein that patients can’t produce.

Pfizer is making a big bet on these gene-transfer therapies, with three clinical trials that could lead to approvals in the next few years.

These gene-replacement therapies have limitations, however. Their effect wears off as children grow, or in parts of the body with high cell turnover, since transferred genes aren’t integrated in the genome and are left behind as cells divide. As a result, these expensive treatments might need to be repeated every few years.

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