One year in, 9 boys with muscular dystrophy show remarkable progress from gene therapy

Nine boys aged 6 to 12 who have been living with [Duchenne Muscular Dystrophy] since birth received a gene therapy treatment from pharmaceutical giant Pfizer, and a year later, 7 of the boys show significant improvement in muscle strength and function.

Though the treatment's positive results are limited to a small group, they're an important breakthrough for gene therapy, and encouraging not just for muscular dystrophy but for many other genetic diseases that could soon see similar treatments developed.

DMD is a genetic disorder that causes muscles to progressively degenerate and weaken. It's caused by mutations in the gene that makes dystrophin, a protein that serves to rebuild and strengthen muscle fibers in skeletal and cardiac muscles.

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[Researcher Jude] Samulski's gene therapy treatment for DMD used an adeno-associated virus to carry a healthy copy of the dystrophin gene; the virus was injected into boys with DMD, broke into their muscle cells, and replaced their non-working gene. Samulski said of the adeno-associated virus, "It's a molecular FedEx truck. It carries a genetic payload and it's delivering it to its target."

It's only been a year, we don't yet know whether these treatments may have some sort of detrimental effect in the longer term, and the treatment itself can still be improved. But all of that considered, signs point to the DMD treatment being a big win for gene therapy.

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