Why gene therapy’s potential is attracting intense interest from Big Pharma

Big Pharma has been struggling for years to produce drugs that are major breakthroughs in medicine. For these companies, gene therapy may be the promised land.

Gene therapy—treatments designed to replace faulty genes with healthy ones—offers potential cures for hundreds of genetic diseases, like hemophilia and muscular dystrophy. That promise, and its rich commercial potential, has led to a land grab among the drug giants.

The latest is Roche Holding … which agreed [in February] to pay $4.8 billion for Spark Therapeutics (ONCE), a maker of gene therapy for a rare eye condition and hemophilia. …

“Big drug companies are waking up and saying this is a real technology and that they need to be there,” says Marshall Gordon, senior research analyst at ClearBridge Investments… .

So far, the only replacement gene therapy that has been approved by the Food and Drug Administration is Spark’s treatment for a retinal disease that can lead to blindness.

… Many gene therapies have demonstrated impressive results in clinical trials, including the Novartis treatment for spinal muscular atrophy and Sarepta Therapeutics ’ (SRPT) treatment for Duchenne muscular dystrophy, or DMD.

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