

Despite hype and promise of cures, only a few gene therapy trials have yielded helpful drugs

Most experts in the medical field will tell you that gene therapy has finally come of age, but the numbers tell a different story. Despite 30 years of research and a bigger pipeline than ever, only a small number of gene therapy trials have completed late-stage testing or are currently in late-stage trials.

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Early data from recent trials have shown incredible promise for gene therapies targeting certain inherited diseases: patients have seemingly been [cured of hemophilia](#) and rare types of [inherited blindness](#).

But the majority of such gene therapies are in phase I trials and probably won't reach patients for many years.

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Early clinical trials are meant to establish safety, but data from larger, late-stage trials are needed to convince regulators that drugs are effective for the intended population.

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It's widely speculated that Spark's therapy for blindness will be the first gene therapy approved in the United States. Meanwhile, we're likely to see other research groups try to replicate the successful gene therapies for other conditions.

The GLP aggregated and excerpted this blog/article to reflect the diversity of news, opinion, and analysis. Read full, original post: [Despite the Hype Over Gene Therapy, Few Drugs Are Close to Approval](#)