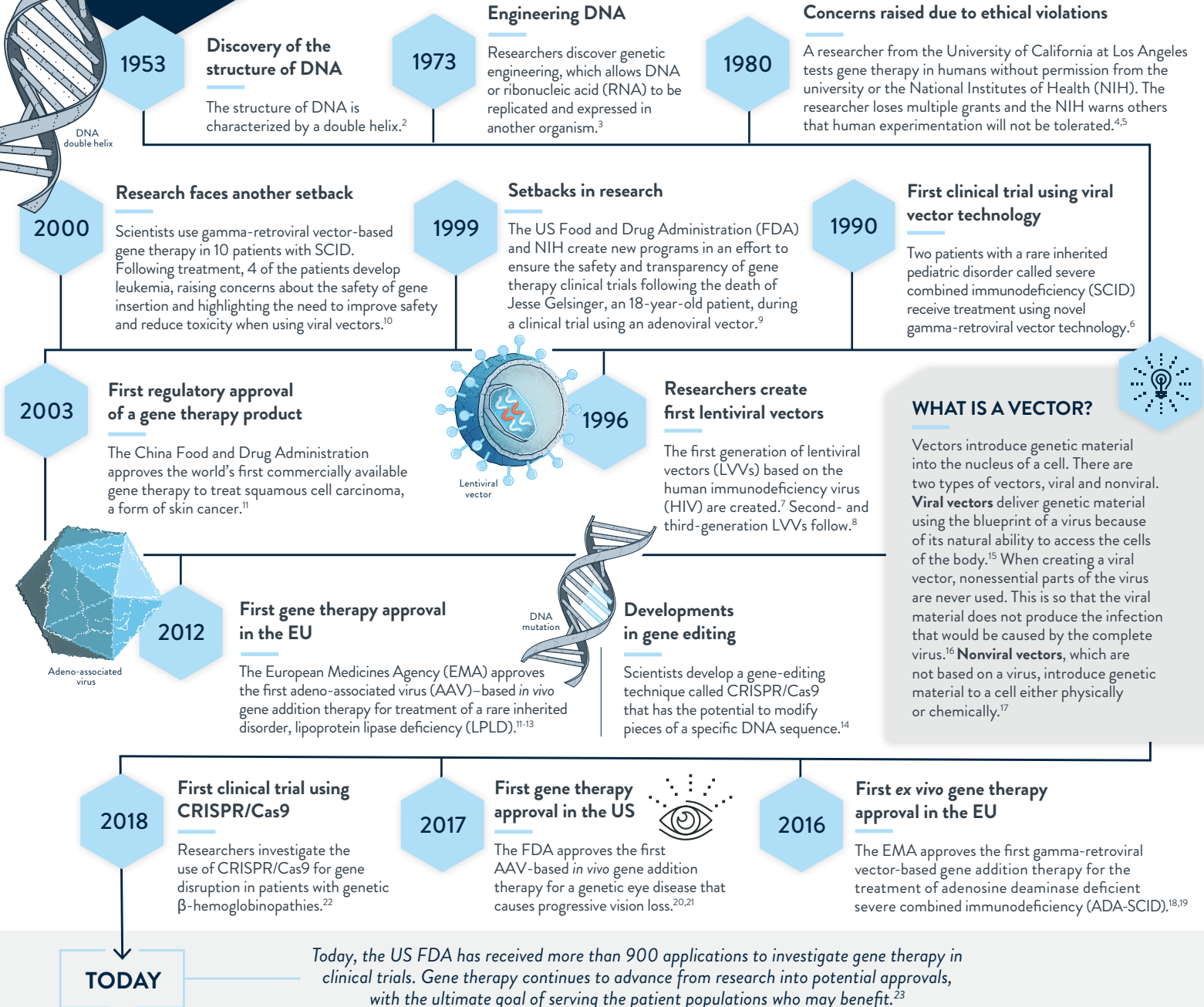


The EVOLUTION of GENE THERAPY

From the early days of gene therapy research, many researchers believed that treating inherited diseases at the genetic level would unlock a new potential for treatment. Over time, researchers have gained a better understanding of genetics and discovered techniques that have led us to the world of gene therapy today.¹

Take a look at some key milestones in the evolution of genetic research and gene therapy



WHAT IS A VECTOR?

Vectors introduce genetic material into the nucleus of a cell. There are two types of vectors, viral and nonviral. **Viral vectors** deliver genetic material using the blueprint of a virus because of its natural ability to access the cells of the body.¹⁵ When creating a viral vector, nonessential parts of the virus are never used. This is so that the viral material does not produce the infection that would be caused by the complete virus.¹⁶ **Nonviral vectors**, which are not based on a virus, introduce genetic material to a cell either physically or chemically.¹⁷

genehome is your space to learn about gene therapy—a go-to source for comprehensive, understandable, and unbiased education.

Learn more about the evolution of gene therapy at www.thegenehome.com

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References: 1. Wirth T et al. *Gene*. 2013;252(2):62-169. 2. Science History Institute. James Watson, Francis Crick, Maurice Wilkins, and Rosalind Franklin. <https://www.sciencehistory.org/historical-profile/james-watson-francis-crick-maurice-wilkins-and-rosalind-franklin>. Accessed March 4, 2020. 3. Science History Institute. Herbert W Boyer and Stanley N Cohen. <https://www.sciencehistory.org/historical-profile/herbert-w-boyer-and-stanley-n-cohen>. Accessed March 4, 2020. 4. Sun M. *Science*. 1981;214(4525):1220. 5. Wivel NA. *Hum Gene Ther*. 2014;25(1):19-24. 6. National Human Genome Research Institute. Results from first human gene therapy clinical trial. <https://www.genome.gov/10000521/1995-release-first-human-gene-therapy-results>. Accessed May 28, 2020. 7. Naldini L et al. *Science*. 1996;272(5259):263-267. 8. Milone MC et al. *Leukemia*. 2018;32(7):1529-1541. 9. Sibbald B. *CMAJ*. 2001;164(11):1612. 10. Hachein-Bey-Abina S et al. *J Clin Invest*. 2008;118(9):3132-3142. 11. Daley J. *Nature*. 2019;576:S12-S13. 12. European Medicines Agency: EMA/H/C/002145 - Glybera. <https://www.ema.europa.eu/en/medicines/human/EPAR/glybera>. Accessed April 29, 2020. 13. Scott LJ. *Drugs*. 2015;75(2):175-182. 14. Jinek M et al. *Science*. 2012;337(6096):816-821. 15. STAT Reports. The STAT guide to viral vectors, the linchpin of gene therapy. STAT News; 2019. 16. Warnock JN et al. *Methods Mol Biol*. 2011;737:1-25. 17. Al-Dosari MS, Goa X. *AAPS J*. 2009;11(4):671-681. 18. Strimvelis Summary of Product Characteristics, GlaxoSmithKline (GSK); 2016. 19. Aiuti A et al. *EMBO Mol Med*. 2017;9(6):737-740. 20. Food and Drug Administration. FDA approves novel gene therapy to treat patients with a rare form of inherited vision loss. <https://www.fda.gov/news-events/press-announcements/fda-approves-novel-gene-therapy-treat-patients-rare-form-inherited-vision-loss>. Accessed May 4, 2020. 21. Luxturna (voretigene neparvovec-ryzl) [prescribing information]. Philadelphia, PA: Spark Therapeutics, Inc.; 2017. 22. Cross R. *Chem Eng News*. 2018;96(2):18-19. 23. Food and Drug Administration. FDA continues strong support of innovation in development of gene therapy products. Press release. <https://www.fda.gov/news-events/press-announcements/fda-continues-strong-support-innovation-development-gene-therapy-products>. Accessed March 4, 2020.

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