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.3

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

The structure of DNA is characterized by a double helix.

Scientists use gamma-retroviral vector-based gene therapy in 10 patients with SCID. Following treatment, 4 of the patients develop leukemia, raising concerns about the safety of gene insertion and highlighting the need to improve safety and reduce toxicity when using viral vectors.20

The FDA approves the first AAV-based vector for gene therapy to treat a genetic eye disease that causes progressive vision loss.22

Scientists develop a gene-editing technique called CRISPR/Cas9 that has the potential to modify pieces of a specific DNA sequence.54

The China Food and Drug Administration approves the world’s first commercially available gene therapy for a genetic eye disease that causes progressive vision loss.20,21

Scientists discover genetic engineering, which allows DNA or ribonucleic acid (RNA) to be replicated and expressed in another organism.3

The US Food and Drug Administration (FDA) and NIH create new programs in an effort to ensure the safety and transparency of gene therapy clinical trials following the death of Jesse Gelsinger, an 18-year-old patient, during a clinical trial using an adenoaviral vector.9

The FDA approves the first lentiviral vectors (LVVs) based on the human immunodeficiency virus (HIV) are created. Second- and third-generation LVVs follow.9

The structure of DNA is replicated and expressed in another organism.3

Gene therapy to treat squamous cell carcinoma, a genetic disorder, lipoprotein lipase deficiency (LPLD).11-13

Two patients with a rare inherited pediatric disorder called severe combined immunodeficiency (SCID) receive treatment using novel gamma-retroviral vector technology.6

The first clinical trial using viral vector technology

First gene therapy approval in the EU

The European Medicines Agency (EMA) approves the first adeno-associated virus (AAV)–based in vivo gene addition therapy for treatment of a rare inherited disorder, lipoprotein lipase deficiency (LPLD).13-15

The first generation of lentiviral vectors (LVVs) based on the human immunodeficiency virus (HIV) are created. Second- and third-generation LVVs follow.9

The first clinical trial using viral vector technology

First gene therapy approval in the US

The FDA approves the first AAV-based in vivo gene addition therapy for a genetic eye disease that causes progressive vision loss.22,27

Developments in gene editing

Scientists develop a gene-editing technique called CRISPR/Cas9 that has the potential to modify pieces of a specific DNA sequence.54

First gene therapy approval in the EU

The EMA approves the first gamma-retroviral vector-based gene addition therapy for the treatment of adenosine deaminase deficient severe combined immunodeficiency (ADA-SCID).26,27

First gene therapy approval in the EU

The European Medicines Agency (EMA) approves the first adeno-associated virus (AAV)–based in vivo gene addition therapy for treatment of a rare inherited disorder, lipoprotein lipase deficiency (LPLD).13-15

First gene therapy approval in the EU

The FDA approves the first AAV-based vector for gene therapy to treat patients with a rare form of inherited vision loss.22,23

The China Food and Drug Administration approves the world’s first commercially available gene therapy for a genetic eye disease that causes progressive vision loss.20,21

Today, the US FDA has received more than 900 applications to investigate gene therapy in clinical trials. Gene therapy continues to advance from research into potential approvals, with the ultimate goal of serving the patient populations who may benefit.21