

# Milestones in the **HISTORY OF GENE THERAPY**

1990 First gene therapy clinical

research in the US

2012 First gene therapy approved in Europe 2022 onwards

Gene therapy research continues

First treatments using genetic-based technologies approved in the US

1965 Discovery of human adeno-associated virus (AAV)



2003 Human Genome Project completed

## 1953

In 1953, scientists James D. Watson and Francis H.C. Crick determined the chemical structure of DNA. This discovery laid the groundwork for the development of future DNA-based technologies, including gene therapy.

#### 1965

In 1965, human adeno-associated virus (AAV) was discovered as a contaminant of adenovirus preparations. It has recently risen to prominence as a vector for gene therapy. AAV vectors are predominately episomal and rarely integrate into the host genome.

### **1980s**

Constance L. Cepko and colleagues reported the development of a murine retrovirus vector system that could be used to carry new genes into mammalian cells. Retroviral vectors insert genetic material into the host cell genome.

#### 1990

In 1990, the first gene therapy clinical trial in the US was started under the direction of Dr. W. French Anderson. Two young girls ages 4 and 9 with adenosine deaminase (ADA) deficiency which causes a form of severe