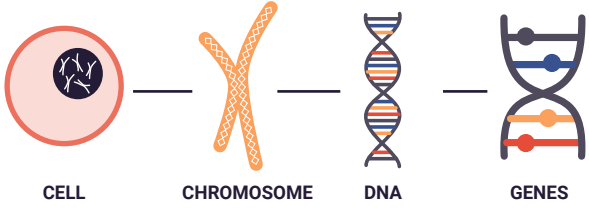


PTC Therapeutics in Gene Therapy

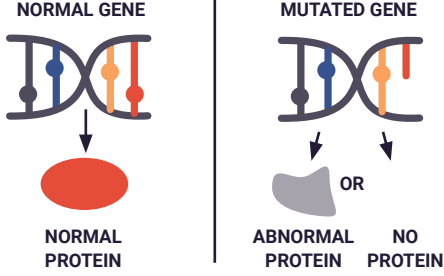
From Lab to Patient

What causes genetic disorders?

Genes are small sections of DNA that provide instructions for the body to make proteins involved in processes essential for life.¹



A change or fault in the gene, called a mutation, may cause a 'genetic disorder'.²



Gene therapy: new hope for previously intractable disease.

Gene therapy aims to treat the genetic disorders by replacing or inactivating genes or introducing corrective genes into cells.³

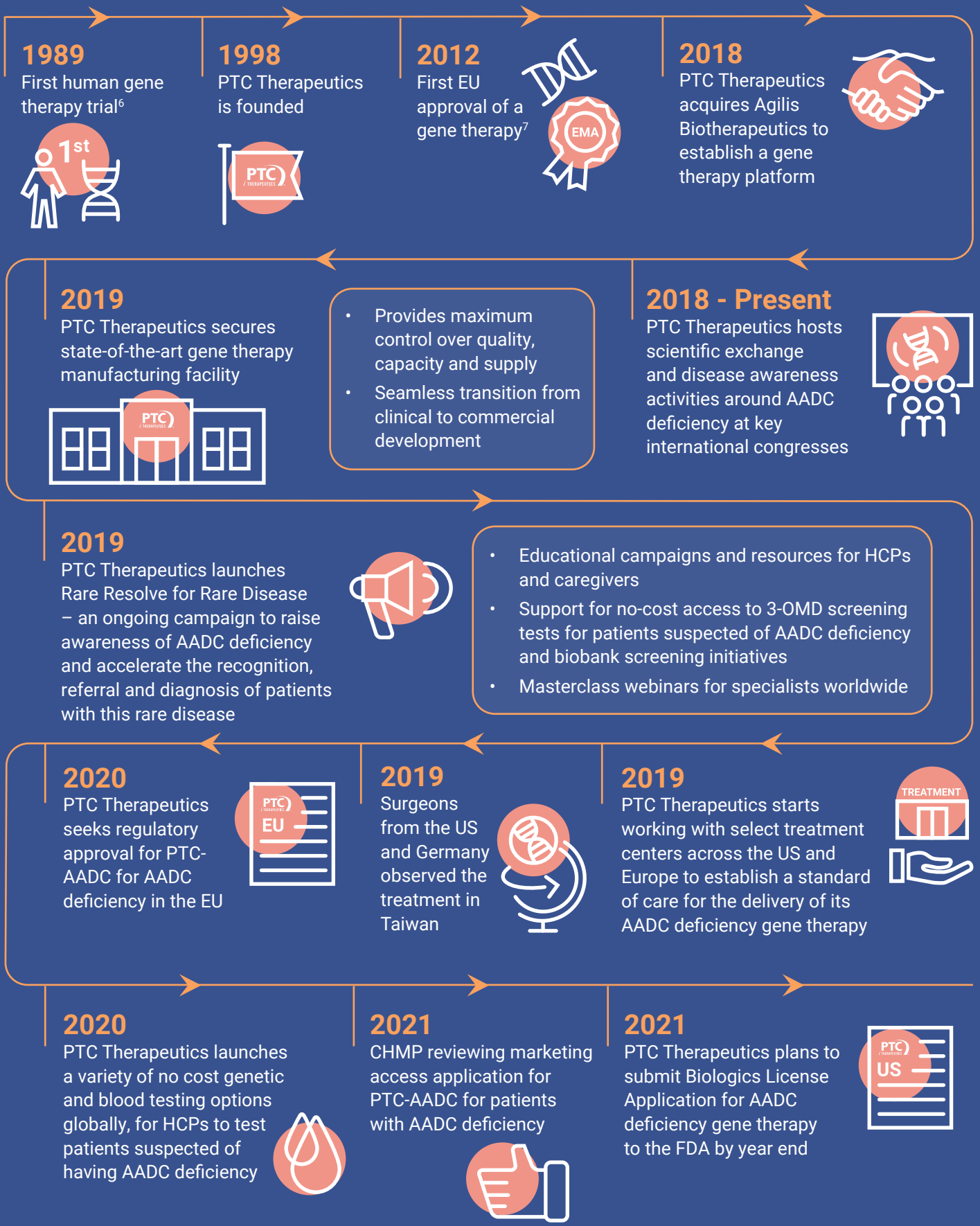
New genes are transferred into cells using transporters known as vectors; made from modified, inactivated viruses that are not harmful.³

In-vivo – directly infused inside the patient³

Ex-vivo – transferred outside the body and then returned to the patient³

By correcting the genetic cause of disease, a single dose of a gene therapy can offer benefits lasting years, if not a lifetime, versus a lifetime of ongoing treatment.⁴ Gene therapy is bringing new hope for thousands of people, many of whom are children with disabling and life-limiting conditions.⁵

Gene therapy holds tremendous promise for some of the most debilitating genetic disorders. PTC Therapeutics is at the forefront of this exciting and transformative area.



PTC Therapeutics has one of the most advanced gene therapy pipelines for rare neurological disorders*

PTC-AADC for AADC deficiency (ADC gene)

AADC deficiency typically causes severe disability and suffering from the first months after birth, affecting every aspect of life – physical, mental and behavioural⁸⁻¹²

PTC-FA for Friedreich ataxia (FXN gene)

Friedreich ataxia progressively robs patients of their ability to walk, speak, see and hear¹³

PTC-AS for Angelman syndrome (UBE3A gene)

Angelman syndrome is characterized by profound intellectual and developmental delays¹⁴

* Information last updated October 2021

Abbreviations

AADC, Aromatic L-amino acid decarboxylase; HCP, Healthcare professional; 3-OMD, 3-O-methyl dopa

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