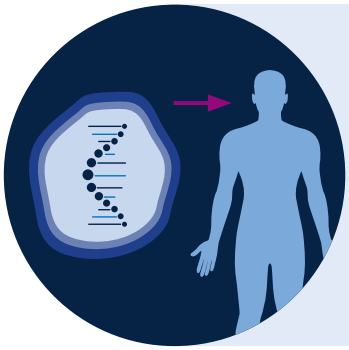
GENE THERAPY: CHANGING THE MANAGEMENT OF DISEASE

GENE THERAPY IS HERE NOW

Already a treatment option for patients in many disease areas, gene therapy has the potential to treat genetic disorders by replacing a defective gene with a working copy, thus helping the body to recover functionality.[1]

AS GENE THERAPY HAS DEVELOPED, DIFFERENT APPROACHES HAVE EVOLVED



- Unlike **gene editing**, where faulty elements within existing DNA are corrected, the process of **gene therapy** intends to leave the original genetic material in the chromosomes unchanged, and so the new functional gene has not been seen to be passed on to future generations.[2]
- The new gene provides instructions for the body to make a protein that it requires and can potentially alter the course of a disease, reducing or eliminating the need for ongoing treatment.[4]

GLOSSARY

GENE EDITING

A procedure that aims to make changes to a patient's original DNA.

GENE THERAPY

Treatment that intends to leave the original genetic material unchanged, introducing new genetic material into the patient's cell for therapeutic purposes.

EX VIVO GENE THERAPY

With cells taken from a patient's body, this type of gene therapy occurs outside of the body, in a lab, before being transferred back into the patient's body.[5] First, affected cells are removed from the patient's body via a biopsy. In the lab, functional genetic material is introduced into the cells, which are then delivered back into the patient's body.[5]

IN VIVO GENE THERAPY

Inserting a functional gene into a patient's cell, this type of gene therapy occurs inside the body.[5] A functional gene is placed inside the shell of a neutralised virus, such as an adeno-associated virus (AAV).[5] The therapy containing the functional gene is then delivered into a patient's cells, often through an IV injection.

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GENE THERAPY IS HERE NOW[6]

Gene therapy is the result of more than

50 years

of collaboration, discovery and research in genetic science.[8]

Proven and effective gene therapies available are now being used by clinicians to treat people across Europe living with genetic conditions.[3] As of September 2021,

11 gene and cell therapies

have been granted marketing authorization in Europe, and are available to patients for conditions including cancer and vision loss.[6]

In the next decade, up to

60 gene and cell therapies

are predicted to be developed and available to patients.[9]

GENE THERAPY HAS THE POTENTIAL TO CHANGE LIVES IN A WAY WE HAVE NEVER SEEN BEFORE

- **Gene therapy** is an entirely new approach to managing disease and could change the meaning of what 'treatment' achieves for people living with genetic conditions, with the potential to **improve their quality of life** in ways which may not have seemed possible in the past.[7]
- As with all medications, **responses to gene therapy may vary across patients**, and how long the treatment effect may last is being evaluated in ongoing clinical trials and data collection.[7]

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