UNDERSTANDING THE IMPORTANCE OF GENE THERAPY FOR RARE DISEASES

WHAT IS GENE THERAPY?

Gene therapy is a technology that uses genetic material, also known as DNA and RNA, to treat or prevent disease.

WHY IS GENE THERAPY GETTING SO MUCH ATTENTION?

Preclinical and clinical gene therapy studies for many rare conditions are currently in progress. The US Food and Drug Administration (FDA) has recently approved gene therapy products for some rare diseases and cancers.

IS GENE THERAPY A CURE?

Gene therapy might be able to prevent disease progression but generally can't correct damage that has already occurred. Research is ongoing to determine if additional doses of gene therapy will be needed.

CAN GENE THERAPY BE PASSED ON TO FUTURE GENERATIONS?

Gene therapy products are currently being studied in body (somatic) cells but not in reproductive (germ) cells. Somatic cell gene therapy cannot be passed on to future generations.

HOW LONG IS THE TREATMENT, WHAT DOES IT ENTAIL, AND DOES IT REACH EVERY CELL IN THE BODY?

Depending on the condition, gene therapy is given systemically with an IV or injected into the affected tissue or organ. A systemic treatment takes a few hours and can potentially reach all somatic cells but is usually targeted to specific cells affected by the condition.

WHY ARE THERE VARIATIONS IN HOW PEOPLE RESPOND TO GENE THERAPY?

There is variability in the success of gene therapy based on several factors: the genetic background of the individual, their natural antibodies, how effectively the gene is delivered to cells, how many cells need the gene to function normally, and how much normal gene product is produced as a result of the therapy.

IS GENE THERAPY SAFE?

More research is needed to understand the long-term effects of gene therapy. It's important to remember that no drug can be given to humans until there have been preclinical safety studies and the data is reviewed by the FDA.

WHY IS GENE THERAPY SO EXPENSIVE?

Conducting research and developing a safe and effective gene therapy is costly, and it can be difficult and time-consuming to produce a gene therapy on a large scale. It's important to consider that, for some patients, gene therapy could potentially reduce other costs associated with treatment and might even ultimately result in lower cost of care over the long term.

WILL INSURANCE PAY FOR GENE THERAPY?

Some FDA-approved gene therapies have been covered by some insurance companies. Some companies developing gene therapies are exploring ways to connect the cost to how well the treatment is working. We don't know if all gene therapy products will be covered.

WHO IS ELIGIBLE TO RECEIVE GENE THERAPY?

Most gene therapy research is currently focusing on treating patients with serious or life-threatening rare disorders and cancers. Eligibility can also depend on a patient's medical history.

WHAT IS THE DIFFERENCE BETWEEN GENE THERAPY AND GENOME EDITING?

Genome editing is a type of gene therapy that uses technologies such as CRISPR to make targeted changes to the DNA sequence of a gene, either to restore its normal function or, sometimes, to inactivate a malfunctioning gene that is causing harm.

HOW CAN I FIND OUT IF A GENE THERAPY CLINICAL TRIAL IS AVAILABLE FOR MY CONDITION?

The best way to find out is by asking your specialty care provider or by searching on clinicaltrials.gov, which lists privately and publicly funded clinical studies being conducted around the world.