

EXPLORING GENE THERAPY

WHAT IS GENE THERAPY?



Each person inherits features from his or her parents in the form of genes¹.



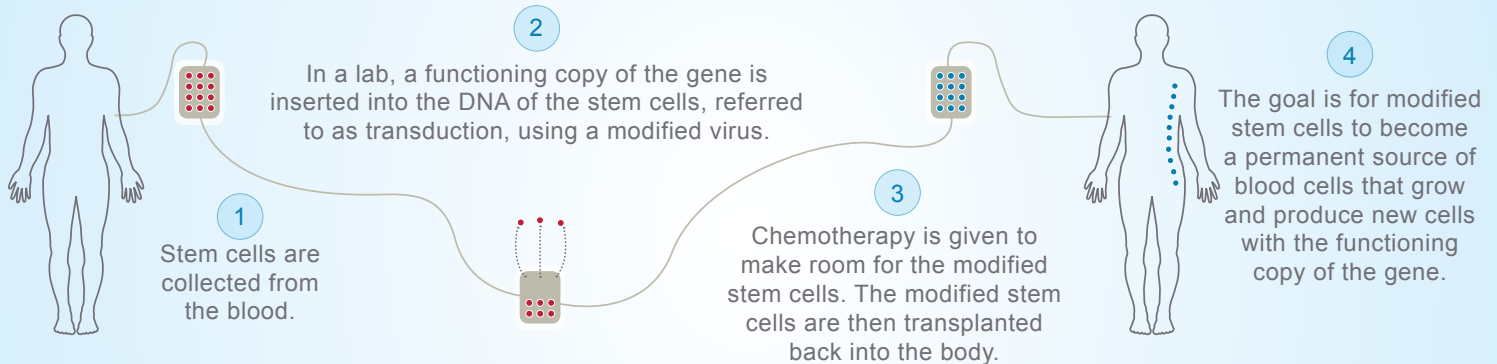
Genes are made up of a molecule called DNA (deoxyribonucleic acid)¹.



When a gene does not work properly, the gene is said to be malfunctioning and may cause a genetic disease¹.

GENE THERAPY USES FUNCTIONING GENES AS MEDICINE TO HELP CORRECT A GENETIC DEFECT².

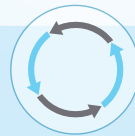
HOW DOES GENE THERAPY WORK?



WHY GENE THERAPY NOW?



In 2012, the first gene therapy was **approved** in Europe⁴.



Significant advances have been made in **gene transfer technology**, safety and how the transfer vehicles are manufactured.



Long term safety and efficacy has been observed for more than 5 years and **positive data is growing**.

For **30 YEARS,**

Gene therapy has been studied as a potential treatment for genetic disease².

~2,000

clinical trials have been conducted using different types of gene therapy in cancers, blood diseases, central nervous system disorders and immune system diseases^{2,3,5}.

THE GOAL

**TRANSFORM THE TREATMENT OF GENETIC DISEASES
BY PROVIDING FUNCTIONING COPIES OF THE MALFUNCTIONING GENES.**

Sources:

1. www.ornl.gov/scitech/resources/Human_Genome/medicine/genetherapy.shtml (Accessed Dec. 4, 2012).
2. Sheridan, C., 2011. Gene therapy finds its niche. *Nature Biotechnology*, 29(2), pp. 121-8.
3. Kay, M. A., 2011. State-of-the-art gene-based therapies: the road ahead. *Nature*, 12(5), pp. 316-28.
4. www.uniquere.com/news/1671889/uniQure-s-Glybera-First-Gene-Therapy-Approved-by-European-Commission.html (Accessed Feb. 18, 2013).
5. Kohn, D.B. & Candotti, F., 2009. Gene therapy fulfilling its promise. *The New England Journal of Medicine*, 360(5), pp. 518-21.