Cell and gene therapy: A new era of medicine

Cell and gene therapies could help reduce or eliminate the need for treatments that need to be taken continuously, often for life

Novartis is reimagining medicine with one-time, potentially curative cell and gene therapies that only need to be administered once for patients with serious, rare and life-threatening diseases. These new therapies present the opportunity to reexamine how our healthcare system manages diagnosis, treatment, care and associated costs for these patients.

Conventional Therapy

Uses small molecules, peptides, proteins

Treatment contains a small (most drugs) or large (biologics) molecule that mimics or disrupts processes associated with a condition or disease

Chronic therapy

Many conventional treatments must be taken by pill, injection or infusion on a continual basis, and usually the effect of treatment stops once the medication is stopped

Manage or treat symptoms long-term

Usually relieves the signs and symptoms of disease

Cell and Gene Therapy

Uses DNA, RNA, Cells

Reprograms the body to directly fight disease

One-time Treatment

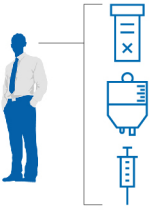
Effect of treatment may be permanent after a single administration

Potentially Curative

Potential to transform medicine, halting the progress of a disease or alleviating the underlying cause of a disease

DELIVERED IN VIVO

Traditional medicines are ingested, injected, or infused, and take action within the body



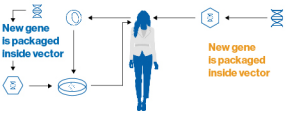
DELIVERED EX VIVO OR IN VIVO[1]

EX VIVO

Genes or cells are modified or replaced outside of the body and then they are returned to the patient

IN VIVO

Genes or cells are inserted or altered directly inside of the patient



IDENTICAL FOR ALL

Uniform treatment designed to benefit larger groups of patients targeting common disease processes or specific disease pathways

Uses broader knowledge about diseases to treat many patients

GENETICALLY FOCUSED

Designed to treat each patient at the genetic level

Uses unique information about a patient's cells and genes, along with the individual characteristics of their disease

References

1. High KA. The Jeremiah Metzger lecture: gene therapy for inherited disorders: from Christmas disease to Leber's amaurosis. *Trans Am Clin Climatol Assoc.* 2009;120:331-359.

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