

Cell and Gene Therapy: a New Era of Medicine¹

Cell and gene therapies could help reduce or eliminate the need for treatments that have to be taken continuously, often for life^{2,3}

Novartis is reimagining medicine with one-time, transformative cell and gene therapies that only need to be administered once for patients with serious, rare, and life-threatening diseases. These therapies present an opportunity to reexamine how our healthcare system manages diagnosis, treatment, care, and associated costs for these patients.^{1,3-5}

Conventional Therapy



Uses molecules, peptides, proteins

Treatment contains a small (most drugs) or large (biologics) molecule that mimics or disrupts processes associated with a condition or disease^{6,7}



Chronic therapy

Many conventional treatments must be taken by pill, injection, or infusion on a continual basis, and the effects of treatment may not continue once the medication is stopped⁶⁻⁸

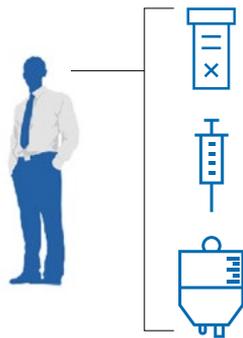


Manages or treats symptoms long term

Usually relieves the signs and symptoms of disease⁸

Delivered in vivo

Traditional medicines are ingested, injected, or infused, and take action within the body^{6,7}



Similar for all

Uniform treatment designed to benefit larger groups of patients, targeting common disease processes or specific disease pathways^{6,9}



Uses broader knowledge about diseases to treat many patients^{6,9}

Cell and Gene Therapy



Uses DNA, RNA, cells

Reprograms the body to directly treat disease^{3,10}



One-time treatment

Effect of treatment may be lasting after a single administration³



Long-term effects

Potential to transform medicine, halting the progress of a disease or alleviating the underlying cause of a disease³

Delivered ex vivo or in vivo³

Ex Vivo outside the body

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



In Vivo inside the body

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



New gene is packaged inside vector

New gene is inserted or altered inside patient

Genetically focused

Designed to treat each patient at the genetic level³

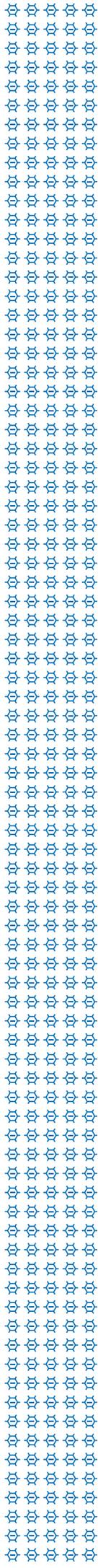


Uses information about a patient's cells and genes, along with the individual characteristics of their disease³



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Reimagining Medicine



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