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**
  - Reprogram 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

- **EX VIVO**
  - Bone marrow is removed or reduced outside of the body, cells are treated and returned to the patient
- **IN VIVO**
  - Bone marrow or cells are inserted directly into the patient

### IDENTICAL FOR ALL

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

### GENETICALLY FOCUSED

- Designed to treat each patient at the genetic level

- **Uses broader knowledge about diseases to treat many patients**
- **Uses unique information about a patient’s cells and genes, along with the individual characteristics of their disease**

References:

Novartis Pharma AG
CH-4002 Basel Switzerland
© 2019 Novartis
05/19