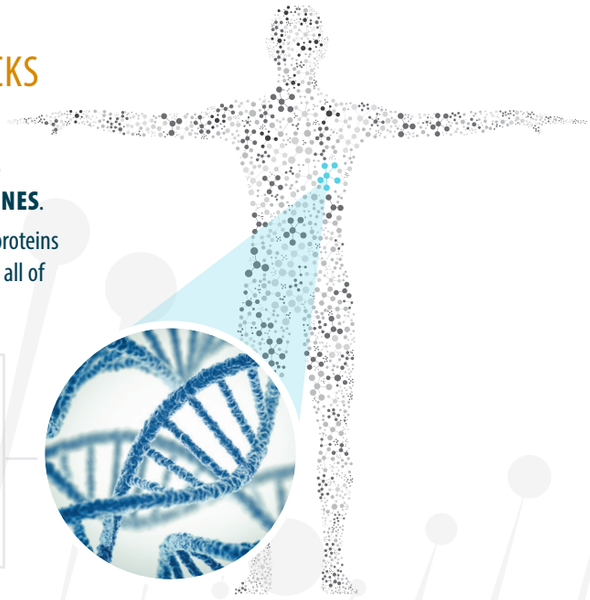
# **Gene Therapy:** Tiny Target, Colossal Costs

## **CELLS** ARE THE BUILDING BLOCKS OF **ALL LIVING THINGS.**

The body is comprised of **TRILLIONS OF CELLS**.

Within these cells are **THOUSANDS OF GENES**.

Genes provide information about the production of proteins and enzymes that make blood, bones and muscles – all of which support the body's functions.[1]



## **GENES** MAY BE MICROSCOPIC, BUT THEY PLAY AN **ENORMOUS ROLE IN A PERSON'S HEALTH.**

In fact, many life-threatening illnesses are derived from defective genes. Knowing this, scientists have worked for decades to find ways to **modify or replace faulty genes** with healthy ones to prevent, treat or cure diseases or conditions.[1]

## WHAT IS **GENE THERAPY?**

**Gene therapy modifies cells by either:**

- Replacing faulty genes with good ones
- Adding genes that can help to fight diseases
- Turning off genes that cause issues[1]

## HOW ARE THE **NEW GENES DELIVERED?**

Currently, scientists use a "**vector" to insert new genes directly into cells**. Viruses that have been modified can be used as vectors. Cells can be modified inside of the body or outside of the body and then returned.[1]

## WHAT PRODUCTS ARE CURRENTLY **FDA-APPROVED AND AVAILABLE?**

| Therapy | Launch | Commonly Associated Disease | Cost* |
|---|---|---|---|
| **Kymriah®** (CAR T-cell therapy) | August 30, 2017[2] | Acute Lymphoblastic Leukemia (ALL)[2] | **$475K** per treatment regimen[6] |
| **Yescarta®** (CAR T-cell therapy) | October 18, 2017[3] | Non-Hodgkin Lymphoma (NHL)[3] | **$373K** per treatment regimen[7] |
| **Luxturna®** | December 19, 2017[4] | Specific type of blindness that is rare[4] | **$850K** per one-time treatment[8] |
| **Zolgensma®** | May 24, 2019[5] | Spinal Muscular Atrophy (SMA)[5] | **$2.1M** per one-time treatment[9] |

## HOW QUICKLY ARE GENE THERAPIES **DEVELOPED/COMING TO MARKET?**

**According to the FDA:**

- In 2019, **more than 800** active cell-based or directly administered gene therapy investigational new drug (IND) applications were on file
- By 2020, **more than 200** investigational new drug (IND) applications will be received per year
- By 2025, **approval will be given** to 10 to 20 cell and gene therapy products per year[10]

## WHAT CAN SELF-FUNDED GROUPS DO **TO HELP MANAGE COSTS?**

- **Clearly address** use of gene therapy in the plan document, defining limitations and criteria for use
- **Know your population risk**
- **Identify potential risks** through claims for genetic testing procedures
- **Know how the plan will be billed** – directly or through provider buy-and-bill (where mark-up can occur)
- **Prepare for potential expenditures** following therapy
- **Notify the Stop Loss carrier** if gene therapy is excluded from covered benefits

## THE IMPORTANCE OF **STOP LOSS PROTECTION**

With high-dollar claims tied to pharmaceutical expenses on the rise, Stop Loss insurance is more important than ever in helping to guard the financial health of self-funded groups. Smart Stop Loss carriers demonstrate expertise with pharmacy cost containment best practices and are responsive in paying claims quickly and accurately. With HM Stop Loss, you can have confidence in the quality of what we deliver.

To learn more about HM Stop Loss protection, contact your HM sales representative or visit **hmig.com**.

*Cost listed is for the gene therapy only at the initial time of FDA approval; it does not include any mark-up by the provider or facility or any additional related costs, nor does it reflect any treatment expansions for the therapy that may have been approved following the initial release or the costs related to that expanded use.

**Sources:** [1]"What Is Gene Therapy? How Does it Work," U.S. Food & Drug Administration; [2]"FDA approval brings first gene therapy to the United States," U.S. Food & Drug Administration; [3]"FDA approves CAR-T cell therapy to treat adults with certain types of large B-cell lymphoma," U.S. Food & Drug Administration; [4]"FDA approves novel gene therapy to treat patients with a rare form of inherited vision loss," U.S. Food & Drug Administration; [5]"FDA approves innovative gene therapy to treat pediatric patients with spinal muscular atrophy, a rare disease and leading genetic cause of infant mortality," U.S. Food & Drug Administration; [6]"What Is the Cost of Kymriah," Drugs.com; [7]"What Is the Cost of Yescarta,"Drugs.com; [8]"How Much Does Luxturna Cost," Drugs.com; [9]"Zolgensma Approval History," Drugs.com; [10]"Statement from FDA Commissioner Scott Gottlieb, M.D. and Peter Marks, M.D., Ph.D., Director of the Center for Biologics Evaluation and Research on new policies to advance development of safe and effective cell and gene therapies," U.S. Food & Drug Administration; all information accessed August 9, 2019.



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