

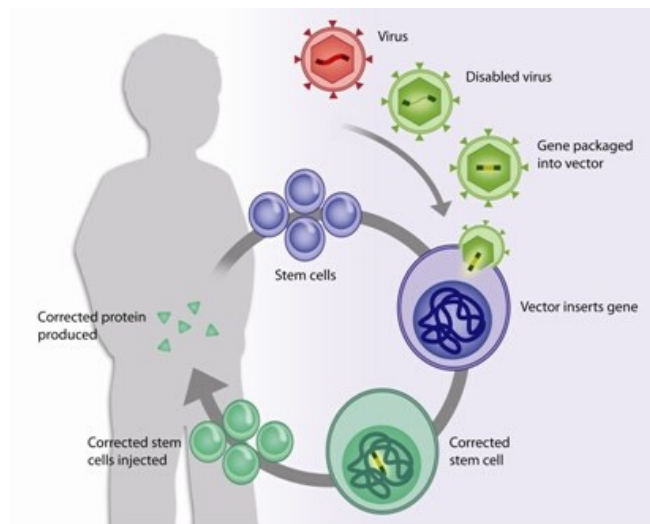


# GENE THERAPY

M A R K E T R E A D

## WHAT IS GENE THERAPY?

Gene therapy is primarily an experimental technique that replaces a faulty gene or adds a new gene in an attempt to cure disease or improve one's ability to fight disease. Gene therapy holds promise for treating a wide range of diseases, such as cancers, cystic fibrosis, hemophilia, and AIDS.<sup>1</sup>



Credit: Boston Children's Hospital  
<https://vector.childrenshospital.org/2011/04/moving-gene-therapy-into-high-gear/>

<sup>1</sup> <https://www.drugs.com/mcp/gene-therapy>

## HOW DOES GENE THERAPY AFFECT SELF FUNDED PLANS?

Before a company can market a gene therapy product for use in humans, the gene therapy product has to be tested for safety and effectiveness so that FDA scientists can consider whether the risks of the therapy are acceptable in light of the benefits. As FDA approvals occur, self funded plans may find that their plan language covers these expensive treatments that were previously excluded from coverage under experimental and investigational procedures.

## APPROVED GENE THERAPIES

Therapy	Manufacturer	Approval Date	Indication	Cost*
ZOLGENSMA® (onasemnogene abeparvovec-xioi)	AveXis, Inc	5/24/2019	spinal muscular atrophy (SMA)	\$2,125,000 for treatment course*
LUXTURNA (voretigene neparvovec-rzyl)	Spark Therapeutics, Inc.	12/19/2017	biallelic RPE65 mutation, associated retinal dystrophy	\$850,000 per a one time treatment*
KYMRIAH (tisagenlecleucel)	Novartis Pharmaceuticals Corporation	8/30/2017	B-cell precursor acute lymphoblastic leukemia (ALL)	\$475,000 for one treatment course*
YESCARTA (axicabtagene ciloleucel)	Kite Pharma, Incorporated	10/18/2017	large B-cell lymphoma	\$373,000 per treatment regimen*

\* cost estimates from Drugs.com as listed at time of FDA approvals.

## HOW CAN SELF FUNDED PLANS PROACTIVELY MANAGE RISKS?

KRMA recommends the following risk management actions:

1. Select top-tier Third Party Claims Administrators that can customize your complete health plan, monitor triggers, and negotiate claims.
2. Carve pharmacy benefits out to a Third Party Pharmacy Benefit Manager (PBM) that offers specialized prior authorizations and case management services
3. Design your benefit plan to include
  - a. Clearly defined definition of Gene therapy, including Criteria for Use and Limitations such as Genomic Testing requirement for medical necessity of Gene Therapies
  - b. Steerage to PBM or direct procurement
4. At KRMA we understand the evolving world of healthcare and self insurance and have access to a variety of subject matter experts; we can help develop plan language to ensure healthy plan management, while still supporting member advocacy; and our carrier partners offer policies that are robust and adaptable to our clients' needs.

