INSIGHT GUIDE

Cost vs. Cure

Gene Therapy's Financial Blueprint

Goodroot

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From the shade of our eyes to the arch of our brow, genes shape the subtle nuances that define us. Beyond physical characteristics, they silently govern our body's functions, dictating our vulnerabilities and resilience to many diseases. Sometimes a gene is defective or is missing when a person is born. This is known as an inherited mutation. In other situations, healthy genes change or mutate as we age, or from environmental exposure. While some mutations are benign or even advantageous, others lay the foundation for serious genetic disorders.

Now, imagine if we could rectify these genetic misprints. Envision a world where genetic conditions, once deemed incurable, are now resolvable with the flick of a molecular switch. The exciting reality is: we're standing on the cusp of such a world.

Gene therapy, the cutting-edge realm of medical science that seeks to treat or prevent diseases by modifying or replacing faulty genes, has made leaps from theoretical musings to tangible solutions. Validated by the U.S. Food and Drug Administration's endorsement, gene therapies are now at the forefront, signaling not just transformative outcomes for cancer and rare diseases but also reshaping the very essence of medical treatments and disease categorization.

However, like every groundbreaking innovation, gene therapies bring their own set of challenges – not the least of which are their monumental costs—many in the millions of dollars. With the influx of these multimillion-dollar therapies, we are faced with a paradox. They offer unparalleled hope to patients otherwise devoid of curative options, yet simultaneously strain our healthcare system's financial backbone. The enormity of these costs isn't just a concern for large institutions—consider the self-insured company grappling with the potential of a single $3 million gene therapy claim. The financial stakes are staggering, and the concern is no longer in the distant future – it is here and now.

So, how do we navigate this promising yet perplexing landscape? This whitepaper seeks to explain the intricacies of gene therapies, unravel their economic implications and provide actionable strategies for stakeholders to ensure that the promise of gene therapies doesn't become an insurmountable burden. Keep reading to explore the new gene therapy frontier, its promises, its challenges and the roadmap to harnessing its potential responsibly.

These insights are brought to you through the collaboration of Goodroot and affiliate companies, RemedyOne, AlignRx and Nuwae.

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About Goodroot

Goodroot is an interconnected community of companies making every interaction between human and health accessible, affordable and seamless. Goodroot's community, which includes AlignRx, Breez, CoeoRx, Emry, Penstock, RemedyOne, Sola, Nuwae and Empara — delivers transformative tech and proactive services that enable clients, partners and patients to reap more value from the money spent on health. We're laser-focused on holistically reinventing healthcare, one system at a time, to improve affordability and accessibility for patients. To date, we've removed over $1 billion in wasteful and unnecessary spending from the industry.

About AlignRx

AlignRx partners with pharmacy benefit consultants, employer groups, TPAs and health plans to offer pharmacy solutions and insights that improve performance, drive savings and deliver better outcomes across the pharmacy benefits value chain. Our robust Rx consulting team—made up of industry experts, data analysts and clinical pharmacists with decades of industry expertise and complete pharmacy landscape command—are in your corner year-round. We develop creative cost-containment opportunities, optimize pharmacy spend and value, recover owed dollars and ensure PBMs are meeting contractual guarantees. Our PBM procurement process is the most meticulous in the industry, ensuring every important detail is accounted for within the contract.

About RemedyOne

RemedyOne is a formulary and rebate optimization company that cuts through the complexity that exists within modern pharmaceutical programs. Our clinically driven approach enables our team, which includes veteran pharmacists and an in-house Pharmacy and Therapeutics Committee, to assess new drugs, understand their cost and implications and make formulary recommendations based on research, insight and efficacy. We work with PBMs, health plans, employer groups and TPAs to provide clinical guidance and cost savings at every stage of the formulary and rebate management process.

About Nuwae

Nuwae is leveling the playing field and changing the game when it comes to controlling the cost of prescription drugs. We believe that there should be one price for a drug, no matter who is paying. Our cutting-edge solutions are designed to lower the cost of prescription drugs by redeeming the tarnished "pharmaceutical rebate" into a force for good. We're solving the issues that drive drug prices to astronomical levels, ultimately lowering out-of-pocket costs and providing equitable access and outcomes for patients. Our mission is grounded in the belief that access to important medications should not be seen as a luxury or a benefit for select patients, and that no one should have to ration their medication due to cost.

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Setting the Stage for Gene Therapies: From Clinical Foundations to Practical Applications

Understanding Gene Therapy: Beyond the Blueprint of Life

Our bodies function based on a complex interplay of cellular mechanisms. At the heart of these operations are genes, which serve as the architects, designing and overseeing the production of proteins essential to our health and well-being. But when these blueprints have errors—whether inherited from birth or acquired later in life—disruptions can lead to an array of health complications, from mild disorders to severe diseases.

Traditional medications have often served as band-aids, temporarily covering symptoms without addressing the underlying genetic anomalies. However, gene therapy offers a transformative approach. It seeks to correct genetic misprints at their source, promising not just symptom management, but a more profound, long-term resolution, often with just a single intervention.

The Mechanisms Behind Gene Therapy: Rewriting Our Genetic Code

Rather than offering a short-term solution, gene therapies fundamentally seek to rectify underlying genetic anomalies. They achieve this by changing our genetic makeup in various ways:

Replacement
Introducing a healthy gene to take the place of a malfunctioning one.

Addition
Incorporating a new gene into the body to fortify its defense against diseases.

Suppression
Silencing a problematic gene that's causing trouble.

These interventions require precision, ensuring the right genetic modifications reach the right cells. Here's where vectors come into play. Through the marvels of genetic engineering, vectors, often derived from viruses, are tailored to move desired genetic material to specific cells efficiently.

Delivery Modes of Gene Therapy: Ex Vivo vs. In Vivo Approaches

The actual delivery of gene therapies can be conceptualized in two primary ways, each with its distinct methodologies and implications:

Ex Vivo (Outside the Body) Gene Therapy:

Cells are extracted from the patient and cultured in a controlled laboratory environment. Here, vectors introduce the therapeutic genes into these cells. Once modified, these genetically altered cells are re-infused into the patient, where they can now execute their functions correctly. By effectively replacing or augmenting the patient's faulty or missing genes, these reintroduced cells can substantially alter disease progression.

In Vivo (Inside the Body) Gene Therapy:

Instead of removing cells from the body, the therapeutic genes (via vectors) are directly introduced into the patient. These vectors move through the body to target specific cells, delivering the genetic material which then integrates with the patient's DNA, enabling the cell to produce the required proteins or correct its malfunctioning processes.

The allure of gene therapy lies in its direct approach. By addressing the root genetic cause behind diseases, it offers hope for more definitive and lasting treatments. As science and technology continue to progress, the horizon of gene therapy keeps expanding, redefining the way we perceive and address health challenges.

Are Gene Therapies Safe and Effective?

Gene therapies are often met with a mixture of awe and skepticism. As with any innovative medical treatment, the balance between potential benefits and risks is a focal point for both healthcare professionals and patients.

At the outset, it's essential to acknowledge that, like all medical treatments, gene therapies are not devoid of risks. Given that they involve alterations at the genetic level, there are concerns about unintended consequences, which might include a heightened risk of certain cancers, adverse allergic reactions or potential damage to organs or tissues, especially if the therapeutic process requires an injection.

But it's also important to emphasize the progress made in recent years to enhance the safety profile of these therapies. Refinements in methodologies, meticulous research and advancements in technology have bolstered their reliability. These strides in safety have not gone unnoticed. The U.S. Food and Drug Administration (FDA) has recognized and validated them by approving certain gene therapies for clinical use in the United States.

The journey of gene therapy from a theoretical concept to a clinically approved treatment is long and rigorously scrutinized. Every therapy must undergo a series of clinical trials, which serve as checkpoints. These trials meticulously evaluate both safety and effectiveness, ensuring that any approved therapy meets the highest standards of care.

Gene and Cell Therapies: Tracing U.S. Milestones

The trajectory of gene and cell therapies in the United States is a testament to relentless scientific inquiry, innovation and the dedication of countless researchers. Each milestone has paved the way for the next, offering hope and opening doors to revolutionary treatments.

December 18, 2017

Luxturna received the FDA's green light, marking its place as a novel gene therapy. This treatment was designed for children and adults grappling with an inherited form of vision loss, which, in the absence of interventions, might culminate in complete blindness.

May 24, 2019

FDA approves innovative gene therapy, Zolgensma, to treat pediatric patients with spinal muscular atrophy, a rare disease and leading genetic cause of infant mortality.

2022: A Year of Multiple Breakthroughs

2022 emerged as a year of proliferation for gene and cell therapies. The year witnessed the approval of five new gene and cell therapies, each promising to address unique medical challenges and expanding the horizons of genetic interventions.

The Future - 2023 and Beyond: A Rich Pipeline

While only a select few gene therapies have received FDA approval, the future holds immense potential. Current indicators, such as ongoing clinical trials and preliminary data, hint at a rich pipeline awaiting regulatory evaluation and approval.

> The FDA projects that
> **by 2025, there may be 10 to 20 such therapies gaining approval each year,**
> solidifying their role in modern medicine.

The Price of Progress: Understanding the Costs of Gene Therapies

Gene therapies represent a medical marvel: the potential to cure devastating diseases often with a single treatment. However, the costs associated with these treatments are causing just as much buzz as their breakthrough potential.

Hemgenix, a groundbreaking one-time treatment for hemophilia B, holds the current title for the priciest drug, coming in at a staggering **$3.5 million**. This price surpasses the previous record-holder, another gene therapy, Skysona, which is priced at **$3 million**. Prior to Skysona, Zynteglo made headlines with its **$2.8 million** price tag for treating a genetic blood disorder. And, as mentioned earlier, in 2019, the world was introduced to Zolgensma, a treatment for spinal muscular atrophy, a fatal genetic disease that strikes infants and young children, at the cost of **$2.1 million**. Other gene therapies carry prices that soar into the hundreds of thousands.

Notably, a report from the Institute for Clinical and Economic Review (ICER) suggests that a more appropriate price tag for Hemgenix would be **$2.9 million**, which is still high, but **$600,000** less than its current market price.

Current Approved Gene Therapies:

Gene Therapy Product	Approval Date	Approved Use	Approximate List Price
Luxturna (voretigene neparvovec-rzyl)	December 18, 2017	Treatment of confirmed biallelic RPE65 mutation-associated retinal dystrophy, a congenital retinal degenerative disorder that leads to blindness	$850,000
Zolgensma (onasemnogene abeparvovec-xioi)	May 24, 2019	Treatment of pediatric patients less than 2 years of age with spinal muscular atrophy (SMA) with bi-allelic mutations in the survival motor neuron 1 (SMN1) gene, a leading cause of infant mortality	$2.1 million
Zynteglo (betibeglogene autotemcel)	August 17, 2022	Treatment of adult and pediatric patients with β-thalassemia who require regular red blood cell (RBC) transfusions	$2.8 million
Skysona (elivaldogene autotemcel)	September 18, 2022	To slow the progression of neurologic dysfunction in boys 4-17 years of age with early, active cerebral adrenoleukodystrophy (CALD), a progressive neurologic disorder	$3 million
Hemgenix (etranacogene dezapar-vovec-drlb)	November 22, 2022	Treatment of adults with Hemophilia B who: • Currently use Factor IX prophylaxis therapy, or • Have current or historical life-threatening hemorrhage, or • Have repeated, serious spontaneous bleeding episodes	$3.5 million
Adstiladrin (nadofaragene firadenovec-vncg)	December 16, 2022	Treatment of adults with with high-risk Bacillus Calmette Guérin (BCG)-unresponsive non-muscle invasive bladder cancer (NMIBC) with carcinoma in situ (CIS) with or without papillary tumors	$60,000 per dose, which can be administered up to 4 times per year. Annual cost $240,000
Vyjuvek (beremagene geperpavec-svdt)	May 19, 2023	Treatment of wounds in patients ≥6 months of age with dystrophic epidermolysis bullosa with mutation(s) in the collagen type VII alpha 1 chain (COL7A1) gene	$24,250 per vial which is about $631,000 per patient per year
Elevidys (delandistrogene moxeparvovec-rokl)	June 22, 2023	Treatment for children with Duchenne muscular dystrophy	$3.2 million
Roctavian (valoctocogene roxaparvovec-rvox)	June 29, 2023	Treatment of adults with severe Hemophilia A without pre-existing antibodies to adeno-associated virus serotype 5	$2.9 million

The Looming FDA Pipeline: What's to Come?

There are 65 gene therapies currently seeking FDA approval, with more to come:

Name	Manufacturer	Route	Status	Condition
Exa-cel (Exagamglogene Autotemcel)	CRISPR Therapeutics Vertex	Intravenous	FDA Review (BsUFA - 12/08/2023)	Sickle cell disease and beta thalassemia
Lovo-cel (Lovotibeglogene Autotemcel)	bluebird bio	Intravenous	FDA Review (BsUFA - 12/20/2023)	Sickle cell disease
OTL-200 (Atidarsagene Autotemcel)	Orchard Therapeutics GSK	Intravenous	FDA Review (BsUFA - 1Q 2024)	Metachromatic leukodystrophy
PF-06838435 (Fidanacogene Elaparvovec)	Spark Therapeutics Pfizer Roche	Intravenous	FDA Review (BsUFA - 2Q 2024)	Hemophilia B
Generxx (Alferminogene Tadenovec)	Angionetics Gene Biotherapeutics	Other	Phase III	Angina pectoris
AAV2-REP1 (Timrepigene Emparvovec)	Nightstar Therapeutics Biogen	Intravitreal	Phase III	Choroideremia (CHM)
Invossa (Tonogenchoncel-L)	Kolon TissueGene	Injectable	Phase III	Chronic degenerative joint disease
Engensis (Donaperminogene Seltoplasmid)	ViroMed Helixmith	Intramuscular	Phase III	Chronic diabetic foot ulcers Diabetic neuropathy
RGX-314	Regenxbio AbbVie	Ophthalmic	Phase III	Diabetic retinopathy
PF-06939926 (Fordadistrogene Movaparvovec)	Pfizer	Intravenous	Phase III	Duchenne muscular dystrophy (DMD)
D-Fi (Dabocemagene Autoficel)	Castle Creek Biosciences Paragon Biosciences Fibrocell Technologies Intrexon	Injectable	Phase III	Epidermolysis
AVR-RD-02	AvroBio	Intravenous	Phase III	Gaucher disease
SB-525 (Giroctocogene Fitelparvovec)	Sangamo Therapeutics Pfizer	Intravenous	Phasev III	Hemophilia A
GS010 (Lenadogene Nolparvovec)	GenSight Biologics Genethon	Ophthalmic	Phase III	Leber's hereditary optic neuropathy
RGX-121	Regenxbio	Injectable	Phase III	Mucopolysaccharidosis Type 2
Generx (Alferminogene Tadenovec)	Angionetics Gene Biotherapeutics	Other	Phase III	Myocardial ischemia and refractory angina due to coronary artery disease (CAD)

Name	Manufacturer	Route	Status	Condition
DTX301 (Avalotcagene Ontaparvovec	Ultragenyx	Intravenous	Phase III	Ornithine transcarbamylase (OTC) deficiency
ProstAtak (Aglatimagene Besadenovec)	Advantagene Candel Therapeutics	Injectable	Phase III	Prostate Cancer
EB-101	Abeona Therapeutics	Other	Phase III	Recessive dystrophic epidermolysis bullosa
AAV-RPGR (Botaretigene Sparoparvovec)	MeiraGTx Janssen	Other	Phase III	Retinitis pigmentosa
AGTC-501 (Laruparetigene Zosaparvovec)	Applied Genetic Technologies	Injectable	Phase III	Retinitis pigmentosa
LYS-SAF302 (Olenasufligene Relduparvovec)	Lysogene	Other	Phase III	Sanfilippo syndrome type A, also known as mucopolysaccharidosis type IIIA
Zolgensma IT (Onasemnogene Abeparvovec-xioi)	AveXis Novartis	Intrathecal	Phase III	Spinal muscular atrophy
LBP-EC01	Locus	Oral Other Intravenous	Phase III	Urinary tract infection
DTX401 (Pariglasgene Brecaparvovec)	Ultragenyx	Intravenous	Phase III	Von Gierke disease
UX701	Ultragenyx	Intravenous	Phase III	Wilson's disease
PTC-AADC (Eladocagene Exuparvovec)	Ultragenyx	Injectable	Phase II	AADC deficiency
AAV-CNGA3	PTC Therapeutics	Other	Phase II	Achromatopsia
AAV-CNGB3	MeiraGTx Janssen	Other	Phase II	Achromatopsia
XC001 (Encoberminogene Rezmadenovec)	XyloCor Therapeutics	Injectable	Phase II	Angina pectoris
Ad-RTS-hIL-12	Ziopharm Oncology	Other	Phase II	Breast Cancer Melanoma
BBP-812	BridgeBio Aspa Therapeutics	Intravenous	Phase II	Canavan disease
BBP-631	Adrenas Therapeutics BridgeBio	Intravenous	Phase II	Congenital adrenal hyperplasia
AVR-RD-04	AvroBio	Injectable	Phase II	Cystinosis
SGT-001	Solid Biosciences	Intravenous	Phase II	Duchenne muscular dystrophy
scAAV9.U7.ACCA	Astellas	Intravenous	Phase II	Duchenne muscular dystrophy

Name	Manufacturer	Route	Status	Condition
GALGT2	Sarepta Therapeutics	Injectable	Phase II	Duchenne muscular dystrophy
4D-310	4D Molecular Therapeutics	Intravenous	Phase II	Fabry disease
RP-L102	Rocket Pharma	Intravenous	Phase II	Fanconi Anemia
GBA1	Regenxbio Prevail Therapeutics Eli Lilly	Injectable Intravenous	Phase II	Gaucher disease
AXO-AAV-GM1	Axovant Sio	Intrathecal	Phase II	GM1 gangliosidosis
LYS-GM101	Lysogene	Oral Other Intravenous	Phase II	GM1 gangliosidosis
AXO-AAV-GM2	Ultragenyx	TBD	Phase II	GM2 gangliosidosis (Tay-Sachs and Sandhoff disease)
SPK-8011 (Dirloctocogene Samoparvovec)	Spark Therapeutics Roche	Intravenous	Phase II	Hemophilia A
DTX201	Dimension Therapeutics Bayer Ultragenyx	Intravenous	Phase II	Hemophilia A
SB-FIX	Sangamo Therapeutics	Intravenous	Phase II	Hemophilia B
FLT180a (Verbrinacogene Setparvovec)	Freeline Therapeutics	Injectable	Phase II	Hemophilia B
AskBio009	Baxalta Shire Takeda	Intravenous	Phase II	Hemophilia B
AMT-060	uniQure	Intravenous	Phase II	Hemophilia B
SB-728-T	Sangamo Therapeutics	Intravenous	Phase II	Human immunodeficiency virus (HIV)
SB-728-HSPC	Sangamo Therapeutics	Intravenous	Phase II	Human immunodeficiency virus (HIV)
AMT-130	uniQure	Injectable	Phase II	Huntington's disease
KB105	Krystal Biotech	Topical	Phase II	Ichthyosis
SAR439483	Atsena Therapeutics	Other Intravitreal	Phase II	Leber congenital amaurosis
RP-L201	Rocket Pharma	Intravenous	Phase II	Leukocyte Adhesion Defect Type 1
pIL-12 (Tavokinogene Telseplasmid)	OncoSec	Other	Phase II	Metastatic melanoma
RGX-111	Regenxbio	Injectable	Phase II	Mucopolysaccharidosis Type I

Name	Manufacturer	Route	Status	Condition
Reqorsa Quaratusugene Ozeplasmid	Genprex	Injectable	Phase II	Non-small cell lung cancer
XT-150	Xalud Therapeutics	Injectable	Phase II	Osteoarthritis
OXB-102	Axovant Sio Oxford Biomedica	Injectable	Phase II	Parkinson's disease
BMN 307	BioMarin	Intravenous	Phase II	Phenylketonuria
SPK-3006	Spark Therapeutics Roche	Intravenous	Phase II	Pompe disease
VCTX211	CRISPR Therapeutics Vertex	Implant	Phase II	Type 1 diabetes
ST-920 Isaralgagene Civaparvovec	Sangamo Therapeutics	Injectable	Phase II	Fabry disease

The landscape of gene therapies is rapidly expanding, with numerous promising treatments in the FDA's approval pipeline. But the trend in gene therapy pricing is clear—and it's steep. While some pricing isn't yet available, we expect to continue to see high price tags for new gene therapies entering the market in the next few years.

Gene Therapies: Moving Beyond Ultra-Rare Diseases

The majority of current FDA-approved gene therapies primarily target ultra-rare conditions. Luxturna, for instance, addresses retinal dystrophy linked to a biallelic **RPE65 mutation**, a condition affecting an estimated **1,000** to **2,000** individuals in the U.S.

However, the horizon of gene therapy is broadening. Notably, Adstiladrin was approved for high-risk Bacillus Calmette-Guérin (**BCG**)-unresponsive non-muscle invasive bladder cancer (**NMIBC**) with carcinoma in situ (**CIS**). Given that bladder cancer ranks as the 7th most common cancer globally and with approximately **6,100** new cases of NMIBC with CIS diagnosed annually, Adstiladrin has the potential to benefit a significant number of patients.

By late 2023, the approval of two promising gene therapies for sickle cell disease, Lovo-cel by Bluebird Bio and Exa-cel by CRISPR Therapeutics/Vertex, is anticipated. This disease, affecting roughly **100,000** individuals in the U.S.—predominantly within the Black community—could see around **20,000 patients** becoming eligible for treatments like Lovo-cel upon its approval.

The scope of gene therapies is undeniably expanding. As they transition from targeting ultra-rare conditions to addressing more prevalent diseases, it becomes critical for plan sponsors to monitor this space closely, preparing for the potential risks and costs associated with these groundbreaking treatments.

And, while some gene therapies will continue to cater to small patient populations with rare conditions, it's crucial to recognize that genetic disorders don't just affect individuals—they can touch entire families. For small to mid-sized self-insured employers, even one employee with two dependents undergoing gene therapy for hemophilia could significantly burden their health plan.

Strategizing for a New Era: Addressing the Costs and Benefits of Emerging Gene Therapies

The potential advantages of gene therapies cannot be overstated. They have the power to transform lives, liberating people from severe, often debilitating conditions. Plus, the annual costs for these conditions can be astronomical. By comparison, one-time gene therapies, despite their eye-watering price tags, can represent long-term cost efficiencies. But payers are skeptical—and for good reason.

Insurance dynamics are fluid—people shift between jobs and different insurance types throughout their lives. This churn complicates the gene therapy landscape, particularly given the upfront, high costs of treatments. Plan sponsors may hesitate to invest in a therapy requiring a significant one-time payment, especially if the long-term benefits of that therapy accrue to another insurer down the line.

Navigating the Financial Complexity of Gene Therapies for Plan Sponsors & Their Partners

The Financial Landscape of Gene Therapies

The substantial initial expenses of gene therapies, combined with the limited long-term efficacy data due to their novelty, pose a significant challenge for healthcare payers to justify the investment. This challenge has been further compounded by small patient populations for many of these diseases, making it difficult to accumulate comprehensive data on long-term benefits, value and costs.

High cost: With individual treatments often exceeding $2 million, a single gene therapy claim can surpass the annual budget for many small to mid-sized employer groups.

It's not just about rare diseases anymore: Though historically targeted at rare diseases, more gene therapies are emerging that aim to treat widespread conditions that impact thousands of people. We expect small-to-midsized plans and employers to be disproportionately impacted. That's why formulating strategies to mitigate financial risks will be critical.

Ensuring Clinical Appropriateness in Gene Therapy Utilization

Prior Authorization

Gene therapies are tailored for very specific indications and come with potential severe side effects. Before embarking on treatment, it's crucial for healthcare providers to validate that patients align with clinical benchmarks such as accurate diagnosis, age compatibility, disease intensity and capacity to endure the treatment. For gene therapies, patients should undergo genetic testing, verifying the presence of the targeted genetic mutation. Incorporating strict prior authorization stipulations for gene therapy claims not only augments safety and patient outcomes but also safeguards the plan from misutilization.

Complex Administration

These therapies often require specialist facilities and clinician administration and are typically covered under the medical benefit. To further ensure quality and value, plan sponsors might consider channeling treatment exclusively through recognized centers of excellence.

Robust Utilization Management

Given the narrow indications and potential severe side effects, plans must implement thorough utilization management strategies, such as precertification or prior authorization, based on FDA-approved indications.

Strategies for Financial Management

Market stop-loss offerings display a range in deductibles, rate caps, and the practice of lasering, or its absence. Lasering, in the stop-loss realm, refers to assigning a particular individual a higher deductible within the policy compared to the rest of the group. Recently, some stop-loss carriers have introduced hybrid solutions, offering overarching stop-loss coverage with elevated limits, complemented by a distinct tier offering protection specifically for certain gene therapies. Examples include Embarc from Evernorth, Optum's Gene Therapy Risk Protection and a program from Stealth Partner Group, which provides supplemental gene therapy coverage for a predictable PMPM fee.

Gene and Cell Therapy Specific Risk Management Products

Some PBMs and insurers offer solutions tailored for gene therapies, ensuring patient access while managing financial risks.

Innovative Payment Models

Both pharmaceutical companies and payers are showing a willingness to explore non-traditional payment models, including outcomes-based programs. These ensure that the payer only fully compensates when the therapy shows desired results.

Examples include:

Novartis' Zolgensma:
Launched with pay-over-time and outcomes-based options.

Spark Therapeutics' Luxturna:
Offers outcomes-based and pay-over-time programs. Additionally, they have direct contracts with insurers to streamline payment processes.

Bluebird Bio's Zynteglo:
Guarantees up to an 80% refund if patients require blood transfusions within 2 years.

Kymriah for leukemia:
Hospitals are only billed if patients achieve remission within 30 days.

Executive Summary

According to a recent survey, while 91% of benefit leaders from employer groups and health plans are aware of gene therapy financial protection products, only 7% currently utilize them. However, nearly half are contemplating their adoption.

With gene therapies ushering in a new era in medical treatment, plan sponsors have an essential role in ensuring these therapies are accessible. By understanding and implementing the strategies above, they can offer these life-changing treatments while ensuring the sustainability of their health plans. It's imperative to advocate for favorable drug-cost arrangements, hold stakeholders accountable for tangible results, and equitably distribute the financial challenges. Coupling this with a holistic and empathetic care approach will not only yield better health outcomes for members but also drive a stronger return on investment. The ultimate goal is a healthcare system where both the well-being of patients and the economic sustainability of the model coexist harmoniously.

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