benefits MAGAZINE

It Could Happen to Your Health Plan:

Managing the Rising Costs and Risks of Rare Disease

by | **Ryan A. Siemers, CEBS**

New therapies for rare diseases and disorders offer life-preserving treatment but carry price tags that can reach into the millions of dollars. How should health plan sponsors manage this emerging risk?

The ongoing rise in health care costs is a well-established challenge for employers and plan sponsors as they continue to focus on quality, wellness, price of services and administrative efficiencies to address this challenge.

But many plan sponsors face a new challenge: managing the outsized financial impact—if not catastrophic risk—created by a single plan participant diagnosed with a rare disease or life-threatening illness. New therapies offer life-preserving treatment for previously untreatable diagnoses but at costs in the hundreds of thousands or even millions of dollars, with some recurring annually. These high-cost treatments elude traditional health plan controls. Being aware of and managing this risk is crucial for all health plan sponsors, particularly those with self-funded plans, where one claimant alone can severely impair the plan sponsor's finances.

Prior to the passage of the Affordable Care Act (ACA), most health plans had individual lifetime if not also annual dollar limits on benefits. According to the Kaiser Family Foundation 2009 *Employer Health Benefits Survey,* on the eve of ACA, 59% of covered workers had such lifetime limits, often $1 million or $2 million. ACA's removal of those limits garnered little attention relative to all of the other changes, since a truly catastrophic claim of $1 million or more was a relatively rare occurrence. Providers, especially hospital systems, knew this common limitation as well and rarely billed beyond those amounts. It seemed like an invisible fence.

While some ACA components have met administrative or political challenges and have been curtailed or eliminated, the removal of dollar limits has become quietly entrenched. Health care providers, led by hospitals, soon recognized that former billing limit maximums had been eliminated. Treatment no longer required transferring the patient to Medicaid and its lower reimbursements, because it was now fully covered by the plan sponsor—at a more agreeable commercial reimbursement rate to the provider.

takeaways

- Under the Affordable Care Act (ACA), health plans can no longer set individual lifetime or annual dollar limits on health benefits.
- The removal of these limits has left health plan sponsors vulnerable to financial risk from participants who have rare diseases and disorders and may require high-cost treatments.
- Pharmaceutical companies are developing new drugs and other therapies to treat these conditions, and costs can be in the millions of dollars.
- Evolving financing solutions that health plan sponsors may consider include installment payments, risk pooling and outcomes-based pricing.
- Medical stop-loss insurance also can protect plan sponsors, but they must be careful to select the right provider and coverage.

A New Financial Runway for Rare Disease and Disorder Therapies

The ACA removal of lifetime limits on nearly all health plans, both individual and group, began with plan years after September 20, 2010. Annual limits were prohibited as of January 1, 2014. When experts projected the costs of ACA, removal of annual and lifetime limits did not generate much concern initially. According to a PricewaterhouseCoopers study prepared in March 2009, "the aggregate cost increase for all companies with lifetime limits would be 0.4% to 0.6%."[1]

Meanwhile, medical stop-loss insurance, the traditional protection against catastrophic medical claimants in self-funded health plans, was not governed by ACA. Stop-loss insurance is not health insurance; it insures (or reinsures) a self-funded medical plan, not an individual. However, stop-loss providers were now tasked with covering this newfound, unlimited health plan liability. Faced with this sudden increase in coverage demand, stop-loss underwriters were eventually receptive to providing stop-loss policies with unlimited maximums, which were previously uncommon. Unlimited coverage is now a largely universal stop-loss policy provision.

In addition to hospitals with costly intensive and specialty care units, specialty drug providers took notice of this new, unlimited funding "runway" for rare or less common medical conditions—many of which are life-threatening. Immediate beneficiaries included patients with Factor VIII hemophilia, a condition that already challenged traditional lifetime limits. Some of these patients may have previously been transferred to Medicaid or other social programs, but now health plans funded treatment regimens of several hundreds of thousands of dollars a year with no lifetime ceiling on benefits.

Pharmaceutical firms also took notice and recognized the expanding opportunity to develop rare disease therapies. Previously, the small patient populations affected by these rare diseases challenged the ability to earn adequate return on the

necessary investment and research. The Orphan Drug Act of 1983, named after the term used to describe these rare diseases and their small populations, previously sought to incent the development of such therapies by providing extended seven-year patent protections and tax credits. However, pre-ACA health plan lifetime limits stymied the ability of health plans to pay the necessary, and often ongoing, costs of a therapy. Patients would hit their limits. Suddenly, there was an existing, if not dusty, incentive for drugmakers to develop therapies alongside an unlimited funding platform by health plans to reimburse the costs. Those earlier estimates of overall plan cost increases of less than 1% were soon challenged.

Emerging Therapies

With unlimited medical plan coverage and the opportunity for extended patent protection, development of therapies for rare diseases and congenital conditions has sprouted. In addition, injectable medications for people with cancers have played an increasing role in oncology treatment. Life-preserving or -enabling, the advent of these sophisticated therapies is welcome.

Most recently, emerging gene therapies that can cure devastating diseases—potentially in a single treatment—have come to market. In their deployment, these therapies destroy a damaged or mutated DNA sequence and replace them with a normal or properly modified sequence. These therapies are clinically administered in a health care setting and share no similarity to those purchased at the neighborhood pharmacy.

One example, Luxturna®, treats a rare genetic disorder that can cause

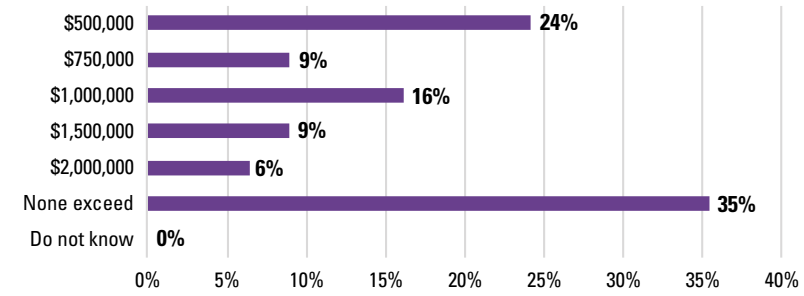
Stop-Loss Insurance: What Does It Cost? How Prevalent Are Catastrophic Claims?

In partnership with Aegis Risk, the International Society of Certified Employee Benefit Specialists cosponsors the *Aegis Risk Medical Stop-Loss Premium Survey,* an annual survey on premiums and aspects of stop-loss coverage. The 2019 survey represents the 13th edition of the survey and gathered data on 539 policies covering more than 940,000 employees.

The survey found that 31% of respondents reported catastrophic claimants in excess of $1 million in the last two policy years.

Highest Paid Claimant, in Excess

In One Policy Year, Over Last Two



Other key observations in the survey include average monthly premium by stop-loss deductible and contract type.

Average Monthly Premium by Deductible and Contract Type

Individual Deductible	Paid
$100,000	$142.69
$200,000	$67.96
$300,000	$44.04
$400,000	$32.37
$500,000	$25.50
$1,000,000	$8.47

Note: $1 million is average of actual responses; n = 13. Premium reflects a mature paid contract. Other contract terms may vary.

A complete copy of the survey can be found at www.iscebs.org. Search "2019 stop-loss survey" to find a link. The site allows visitors to register for notification of next year's survey. All survey respondents receive an initial copy upon its release in late summer.

blindness. First approved in 2017, it has a one-time cost of $850,000. Another, Zolgensma®, recently obtained Food and Drug Administration (FDA) approval as a cure for spinal muscular atrophy. In its most severe form, this disease affects approximately 300 U.S. newborns per year and is typically fatal. Novartis, the drug manufacturer, has set a price of $2.125 million per patient. It competes with an existing therapy, Spinraza®, which costs $750,000 initially with annually recurring treatments that cost approximately $375,000.

The development of chimeric antigen receptor T-cell therapies, known as *CAR-T,* for certain cancers is accelerating. Described as functioning like a "living drug," these therapies orchestrate an immune response and destroy pathogen cells. One example is Kymriah®, a CAR-T therapy with FDA approval that can cost nearly $500,000. Related side effects and inpatient admission can bring the total claimant expense to near $1 million.

The pipeline of these therapies will not slow down anytime soon. In January 2019, FDA announced that it expects to approve ten to 20 gene and cell therapies a year by 2025. In the recent 2020 *Large Employers' Health Care Strategy and Plan Design Survey* by the National Business Group on Health (NBGH), the No. 1 pharmacy benefits concern for plan respondents is how to finance these FDA-approved treatments with million-dollar price tags. "The pipeline is looming—There are an estimated 14 new therapies in excess of $1 million each that are on the docket for FDA approval in the coming months and years," reported Ellen Kelsay, NBGH chief strategy officer, at a press briefing announcing the results.[2]

A Price Inelastic

The price and potential revenue of gene therapies have fueled intense development the last several years. Those initial therapies are now hitting the market, and most are developed by small, startup biotech companies. The research and development is often a jointly funded effort with nonprofits as well as governmental entities like the National Institutes of Health (NIH). Private equity and venture capital funding also play a part.

Instead of developing new products internally, established pharmaceutical firms have found it more attractive to acquire these biotech startups, in deals often worth billions. These purchase prices are not to offset debt from research and development but rather to capture the future projected revenue stream of these therapies. These firms, and their shareholders, will expect to recoup their investment. Health plan sponsors should not expect to receive price concessions on these drugs.

Health Plan Insurer and Administrator Response—So Far

Plan sponsors and their health plan administrators are actively seeking solutions to finance the high price of these therapies, with hopes of better managing the costs. However, the response so far has been largely reactive rather than proactive. Furthermore, no plan sponsor (or its advisors) with a stop-loss policy should take a simple, carefree approach, thinking that "stop loss will just cover it." Stop-loss insurance reinsures the health plan, which is governed by plan documents that further describe medical necessity and the role of a qualified plan administrator. Each component is crucial.

Financing solutions to these therapies evolve around versions of risk pooling, as well as outcomes-oriented payment structures, including:

- **Installment payments.** An approach led by the drugmakers, this allows plans to allocate payment for a therapy over multiple years. While discussed as a viable solution by some, including employer groups, think tanks and consultants, it merely spreads out the cost of the therapy and does not address the underlying cost. This likely explains its genesis from the drugmakers. This financing approach is uncommon for any other compo-

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nent of health plan expense. It's also unclear how a stop-loss policy would address the annual expense compared with a one-time claim. It's possible that each payment would be subject to another year's stop-loss deductible, which could mean the plan pays the full expenses of the therapy with no costs covered by stop-loss insurance if it doesn't exceed the deductible.

- **Risk pooling.** Risk pooling seeks to share the projected expense across a larger population than one plan and is the dynamic that forms the basis of fully insured health plan underwriting, let alone the basis behind a medical stop-loss policy. Health plan administrators Cigna, CVS Health (Aetna) and Anthem (a Blue Cross Blue Shield company) have all announced initial development of programs that offer coverage of gene therapies above a likely cost threshold for a per member per month fee. In a recent *Wall Street Journal* article, Dr. Steve Miller, Cigna's chief clinical officer, said the company is targeting the fee to be less than $1 per member per month.[3] Any plan that may participate in such a program should ensure that any concurrent stop-loss policy acknowledges this external assumption of risk and gain a stop-loss underwriting price credit. Aside from risk protection, these health plan administrator programs would include the clinical review process and specialty pharmacy, as needed, to dispense. Insurers also hope to obtain outcomes-based pricing, which would bring some component of quality into the equation. "We're looking for money-back guarantees," said Dr. Miller.
- **Outcomes-based pricing.** Similar to other, evolving value-based provider reimbursements that reward quality, not just volume, this approach seeks to tie ultimate reimbursement to the clinical effectiveness of therapies. However, this approach has challenges, including that it doesn't address the high cost set by drugmakers. Furthermore, determining clinical effectiveness may require long-term results, which means a patient may no longer be a participant of the plan that funded the therapy, and his or her outcomes would be unknown. Rebates may be offered as the mechanism for not meeting clinical outcomes, but those may be limited since they cannot exceed the "best in market" rebates guaranteed to Medicaid, which are about 23% at best.

Stop-Loss Insurance—An Established Protection, but Buyer Be Mindful

The role of medical stop-loss insurance remains to protect a self-funded medical plan as a policyholder from the infrequent and sporadic financial impact of a catastrophic health plan claimant. The monthly premium paid for coverage is in many ways a budgeting tool for the plan sponsor to allocate that unpredictable expense across all plan periods. Through the purchase of a stop-loss policy, a plan sponsor is effectively pooling its risk with all others held by its stop-loss underwriter. Ideally, an underwriter is sizeable enough to both anticipate and fund these expenses by spreading the costs throughout its entire block of policyholders. In that manner, the claim is not a surprise but an expectation that the underwriter has already forecasted.

Having a catastrophic medical claimant has been a long-held risk of any self-funded plan sponsor. Since the ACA establishment of unlimited health plan liability nearly ten years ago, there have been more frequent occurrences of traditional claimants in the millions of dollars, if just from removal of the prior, common limits. From claims for premature infants and patients who need multiple surgeries to Factor VIII hemophilia regimens, all of the larger medical stop-loss writers have already approved stop-loss policy reimbursements in excess of $5 million or more. Stop-loss providers scrutinize these claims to confirm that they meet the required eligibility and are medically necessary and that the reimbursement is for the usual, customary and reasonable (UCR) charge per the underlying health plan document. If administered properly, these claims are reimbursed.

It's likely that some stop-loss writers, perhaps facing a reimbursement that is a significant percentage of their overall book-of-business premium, have opted to deny or make an incomplete reimbursement based on one of those terms. Contrary to a still-persistent belief in the market, stop-loss insurance is not a commodity. Buyers should beware, perhaps more than ever.

Therefore, plan sponsors and benefit managers must be vigilant in selecting a stop-loss underwriter and policy and making sure it and the underlying health plan and administrator work in tandem. Key considerations include the following:

- **Obtain or verify "mirroring" of terms in the health plan document and those in the stop-loss policy.** Stop-loss policies should exclude any parallel language

in the policy and defer to terms in the approved summary plan description (SPD). For example, the basis of UCR reimbursement is one term that should have a shared definition.

- **Pursue a stop-loss policy with no lasering at renewal and a renewal rate cap increase.** *Lasering,* or excluding or limiting (e.g., setting a higher deductible), a specified claimant from coverage under the stop-loss policy is a common underwriter request. This is sometimes unavoidable when purchasing a policy (since any fully known or expected claimant will otherwise be fully priced into the premium). Plan sponsors should ideally avoid this with a "no laser at renewal" provision, whereby a risk that occurred post-policy placement is covered in future periods. With some costly therapies becoming an annual regimen, this can ensure the risk is carried by the stop-loss underwriter as much as possible.
- **Ensure the health plan administrator and/or pharmacy benefit manager has a focused program to authorize and approve administration of gene therapies and other costly specialty drug regimens.** The plan document likely requires medical necessity for these drugs or therapies to be administered. The stop-loss claims analyst will confirm this prior to reimbursing the plan. Therefore, plans should have a program and process in place for confirming medical necessity. The stop-loss carrier may have a gene therapy/specialty pharmacy program as well. Furthermore, it is important for the stop-loss carrier and health plan administrator to have dialogue because it supports the stop-loss insurer reimbursing the eventual expense.
- **Clearly address the use of gene therapy in the plan document, including defined limitations and criteria for use.** Plan sponsors should ensure that any definition of specialty drugs accommodates the plan strategy on gene therapy. They should also seek provisions that permit drug infusions or treatments at home or in an outpatient setting, in lieu of a more costly inpatient setting, when clinically available.
- **Seek financially strong stop-loss underwriters able to withstand and pay claims.** In many ways, bigger is better. The AM Best rating of the insurer should be an "A" or better. These insurers with larger books of business are typically better equipped to handle a claim for $1.5 million, even one as sizeable as $7-$8 million. The largest insurers may have annual stop-loss premiums exceeding $500 million. However, quality underwriters still exist at lower volumes. Plan sponsors also should consider the insurer's combined surplus of assets to liabilities. The larger ones carry $1 billion or more. The goal is for the stop-loss reimbursement to be a "drop in the bucket" as much as possible.
- **Mind the disclosure of high claimants at stop-loss placement and (if required) renewal.** Stop-loss insurance, like any insurance coverage, is written to cover future, unknown risk. Key to that process is the required disclosure of known and existing high-cost claimants to the underwriter, at which point a "firm" proposal is returned. Plan sponsors should not be careless about or overlook claimants who may generate high costs. Often, the health plan has a high-cost claimant listing, which is sufficient documentation. But if the plan omits a participant who

bio

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has just started a costly gene therapy, for which significant expenses have already been incurred, there is a chance that the future claim would not be covered by stop-loss insurance. Plan sponsors should be mindful of the request and its attending disclosure statement, including whether it requests disclosure of known, potential recipients of gene therapy.

- **Seek an informed, experienced advisor on placement of stop-loss coverage.** Notably, stop-loss insurance is not an employee benefit. It covers the plan sponsor. In that respect, it is much more like property and casualty coverage, including the disclosure process and active underwriter review. There are few parallels to the purchase of any employee benefit coverage or service. Plans that do not have someone experienced in stop-loss coverage on their team should seek someone who can provide that knowledge.

In Summary

ACA had impacts both fleeting and lasting on employer health care costs. The removal of all annual and lifetime limits by 2014 opened the pathway to new therapies which, while life-preserving, have also thrust upon plan sponsors a new financial risk. Few can deny the attributes of these medical advancements, but plan sponsors should remain vigilant to ensure that they are properly used and reimbursed. In addition to ensuring proper authorization by their plan administrator, self-funded plan sponsors should continue to seek medical stop-loss protection, further ensuring harmony between it and the underlying plan. Finally, an experienced and knowledgeable advisor always helps.

Endnotes

1. *The Impact of Lifetime Limits,* PricewaterhouseCoopers, March 2009. Prepared for National Hemophilia Foundation on behalf of the Raise the Caps Coalition.
2. "Large Employers Are Concerned About Million-Dollar Treatments," *AIS Health Daily.* Leslie Small. August 26, 2019.
3. "Insurers Pitch New Ways to Pay for Million-Dollar Therapies," *The Wall Street Journal.* Joseph Walker and Anna Wilde Mathews. September 5, 2019.

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