January 22, 2024

The Honorable Bill Cassidy, M.D.
Ranking Member
U.S. Senate Committee on Health, Education, Labor, and Pensions

Via email: GeneTherapyCoverage@help.senate.gov

Re: Request for Information, Access to Gene Therapies

Dear Senator Cassidy,

On behalf of the Health Care Delivery Committee, the Individual and Small Group Markets Committee, and the Active Benefits Committee of the American Academy of Actuaries ("the committees"),[1] we appreciate the opportunity to respond to the Committee on Health, Education, Labor, and Pensions' (HELP) request for information (RFI) on access to gene therapies for patients with an ultra-rare disease.[2] Our comments will primarily consider a commercial marketplace perspective and may not address or apply to public programs or other coverage options.

Introduction

Gene therapy is in its infancy. Relatively few therapies have been approved thus far, with most affecting a small number of patients. This is poised to change. Significant growth in the number of approved therapies is expected over time, including some that could benefit a substantial number of patients.[3]

To date, most approved gene therapies share several common characteristics:

- A small potential patient pool per therapy (ultra-rare or rare diseases).
- Pricing per course of treatment at several hundred thousand dollars or higher.
- Treatment for a chronic rather than acute condition.
- Recent U.S. Food & Drug Administration (FDA) approval with a limited track record on potential long-term effects.
- A low number of providers who are trained on and experienced in the delivery of the therapy. Some delivery locations may fall outside the geographic area of a patient, or be considered out-of-network (OON) for the patient's health plan.

[1] The American Academy of Actuaries is a 20,000-member professional association whose mission is to serve the public and the U.S. actuarial profession. For more than 50 years, the Academy has assisted public policymakers on all levels by providing leadership, objective expertise, and actuarial advice on risk and financial security issues. The Academy also sets qualification, practice, and professionalism standards for actuaries in the United States.
[2] https://www.help.senate.gov/imo/media/doc/gene_therapy_rfi.pdf
[3] https://milkeninstitute.org/article/gene-therapies-next-frontier-drug-development

While these characteristics are not unique to gene therapies, the high costs per treatment and the potential for future gene therapies that will treat more common conditions has created significant emphasis on the current state of coverage of these therapies. The RFI signals a desire to accelerate gene therapy coverage for these ultra-rare conditions in commercial markets while contemplating an increased leadership role for Congress.

The net impact of coverage expansion for new gene therapies in the current environment and in the future is likely an improvement in the quality and/or duration of life for affected patients as well as an increase in the cost of health care. While the net cost impact of covering any given therapy is likely to be modest in relation to the current total cost of health care, the cumulative cost of all approved therapies could be significant. Efforts to fast-track coverage are also likely to accelerate cost increases.

Each therapy/use case is unique and the path to commercial coverage is equally unique. As is true of any new treatment, widespread coverage and rapid adoption of the therapy is much more likely when the new treatment offers an improvement in health outcomes or in cost when compared to existing options.

To support the development of new therapies, accelerate coverage, and promote equitable access and coverage of existing treatments, HELP has several policy levers to consider. Increasing the pace of coverage for gene therapies for ultra-rare conditions through formal mandates could bring these new treatments to patients more quickly and accelerate innovative research. However, this may also lead to higher costs overall, and to the individual patients, due to reduced leverage in price negotiations between providers, health insurance plans, and manufacturers, reduce the appeal and/or use of alternative therapies that are equally efficacious, or promote the adoption of therapies with less obvious improvements in health outcomes.

What is the Current Practice for Patients with Ultra-Rare Diseases or Disorders?

8. What, if any, are the cost-sharing mechanisms that patients are typically subject to when paying for and accessing these therapies?

Cost-sharing for covered gene therapy services are typically expressed as a combination of copays, coinsurance, and/or annual deductibles. Cost-sharing is likely to be modest in richer plans, while leaner plans average may exceed 30% of allowed charges.

Within current plan design, there are policy levers intended to protect consumers from catastrophic financial impacts. One such lever includes the ACA, annual out-of-pocket (OOP) maximums. These maximums limit the overall financial exposure of the covered individual for essential health benefits (EHBs). In the 2025 plan year, the OOP maximum is $9,200 for an individual.[4]

[4] 2025 PAPI Guidance (cms.gov)

If a health plan emphasizes the use of coinsurance, a gene therapy patient is more likely to meet the OOP maximum due to the high overall cost of the therapy. However, this is only true when the gene therapy services are considered covered benefits and therefore apply to the OOP calculations.

Patients may be subject to higher cost-sharing if the gene therapy is offered only through OON providers. This also introduces the potential for balance billing, should the treatment be performed by individuals or facilities who are not in-network (INN) or the therapy is considered experimental by the health plan and not a covered benefit.

How Do Plans and Payers Currently Manage Financial Risk?

11. What does coverage for these therapies typically look like? What does the landscape look like for coverage of these therapies?

The following response describes criteria that may be used to evaluate whether a particular patient qualifies for gene therapy treatment in plans where this is a covered benefit.

Generally, coverage eligibility is dependent on the criteria of the therapy and will vary by condition. The eligibility determination process is usually initiated by the patient's physician and in coordination with the medical management team of the physician's hospital/facility/office, which may be part of a Center of Excellence (COE). During this step, a review of the insurer's drug formulary is likely to take place. The role of drug formularies in determining coverage of gene therapies is evolving. Most gene therapies are currently administered by a physician, which is generally considered a function of the medical benefit and less affected by prescription drug formulary construction. As gene therapies become more prevalent and potentially require less direct involvement from medical personnel, formularies may begin to play a more significant role in determining coverage. Prior authorization requirements are also common, which may limit access, particularly for patients on the margins of the targeted treatment group.

As part of the eligibility determination stage, most medical teams also review demonstrated health outcomes and any clinical trial safety results to assess whether the treatment is appropriate for the patient. If there are recommended standards of care from professional medical societies or generally accepted protocols from credentialed organizations, such guidance will be included in the assessment. The team will also review the patient's experiences with alternative treatments, should alternatives exist.

The landscape may continue to evolve, as language within the proposed 2025 HHS Notice of Benefit and Payment Parameters (NBPP)[5] would eliminate the state financial responsibility and instead allow any state or federal benefit mandate that has been implemented since 2012 to be eventually considered an EHB, with the associated OOP maximums and coverage requirements.

[5] https://www.federalregister.gov/documents/2023/11/24/2023-25576/patient-protection-and-affordable-care-act-hhs-notice-of-benefit-and-payment-parameters-for-2025

12. What are the typical elements of a benefit design that includes coverage of these therapies?

Typical cost-sharing features for covered gene therapy treatment are described in the response to Question 8. Other typical plan provisions for covered gene therapy are described in the response to Question 11.

For self-insured employer sponsored insurance, or fully insured employer sponsored insurance if there is no mandate to cover these therapies, leaner plan options (such as those targeting actuarial values near the 60% minimum value threshold) are more likely to exclude coverage of these therapies. These plan options are designed to provide minimum essential coverage while limiting the costs of the insurance coverage through high cost sharing, utilization management, and/or a narrower list of covered services than is typical of more generous plans. Leaner plan options can be found in all product network types (e.g., PPO, POS, EPO, HMO).

13. What factors do benefit consultants consider when designing benefits that include coverage of these therapies?

Benefit consultants consider the following when designing benefits that include coverage of gene therapies:

- Cost of the coverage, including the physician and facility costs involved.
- Expected workforce turnover.
- Whether alternative treatments exist for a given condition that can be treated with gene therapy.
- Coverage decisions among peer employers.
- Efficacy of the treatment.

Health plan sponsors may consider the following additional factors:

- Care management approaches and their value.
- Network considerations, such as whether to employ a COE approach with travel coverage, which could improve health outcomes and reduce overall costs.
- Health equity.
- The financial risk in including each particular treatment.
- Legal risks, such as nondiscrimination.
- The impact to premiums, especially relative to competitors.
- Selection risk, particularly for sponsors with multiple plan offerings.

15. What contract options exist for health plans and other payers to mitigate the cost of covering these therapies?

While we are not aware of a single common approach for health plans and other payers to mitigate the costs of covering gene therapies, potential options include:

- Requiring prior authorization and/or attempting alternative therapies to ensure that the patient is likely to benefit from the gene therapy.

- Managing one-time payment terms and discounts, whether with the manufacturer for the treatment or with the facility, for bundled payment terms that includes the treatment cost as well as facility and professional costs.
- Seeking contracts with many gene therapy providers to reduce the risk of OON charges and risk of up-charged treatment costs. There are several vendors operating in this space to help health plans achieve this strategy.
- Seeking outcomes-based reimbursement arrangements, although these require multiple years of information and there is no guarantee that a patient will remain on a given plan for multiple years.
- Excluding certain treatments from the plan, although some plans also provide direction to enrollees regarding financial support through clinical trial participation, charitable organizations, or the applicable manufacturers.

It should be expected that a course of gene therapy treatment for an ultra-rare condition will continue to be quite costly even after mitigation efforts. Many gene therapies are the most effective treatment for specified patients with the targeted conditions, and the limited patient pool makes it unlikely that other companies will prioritize development of competing therapies. This gives pharmaceutical companies significant leverage in negotiations. To date, there has not been a significant reduction in consumer prices, which can be an unintended consequence of coverage mandates.

17. What factors or challenges do health plans and other payers typically consider when determining whether to cover one or all of these therapies?

When determining whether to cover gene therapies, health plans and other payers consider:

- Efficacy and safety of the treatment, including expected long-term outcomes.
- Alternative treatment options (if any) and their costs/outcomes.
- Expected utilization.
- The likely ability to deliver most care INN, reducing the risk of up-charging and overall treatment costs.
- Cost and benefits of risk mitigation options, such as stop-loss insurance, reinsurance or other risk pools that would cover these therapies.

19. How do health plans and other payers currently evaluate the long-term financial benefit of covering these therapies compared to the high up-front cost? Interested parties should include information on how they evaluate these therapies in relation to existing clinical treatments.

In considering whether to cover a specific gene therapy, both short- and long-term financial expectations are considered by health plans and other payers in addition to the factors described above. The evaluation includes the expected cost of the therapy, as well as the reduced costs for existing treatment alternatives, the time value of money, and expected changes in utilization. Note there may be a fair amount of uncertainty about long-term costs and benefits.

Payers might not consider indirect costs in their evaluations. For example, a health plan offering coverage on the ACA Individual Marketplace might not attach much weight to an expected reduction in sick days, since this has little to no impact on the cost of the coverage.

After deciding to cover a specific therapy, the price for the upcoming year of coverage is likely to anticipate the higher upfront, net short-term cost with no immediate credit for an expected long-term benefit.

20. How do health plans and other payers currently evaluate the financial benefit of covering these therapies in instances where a patient might switch health plans?

As noted in the response to Question 13, plan sponsors or their advisors might consider potential health plan switching when making the initial decision about whether to cover a specific gene therapy. Other factors, as noted previously, are far more prominent in decision-making processes. Once it is decided that a specific treatment is a covered benefit, health plans and other payers do not usually consider future switching. When evaluating an individual patient's request for coverage of a specific treatment, health plans and other payers do not consider potential future switching. Comparatively, stop-loss providers may consider the health of existing patients, as well as the potential of future switching, when deciding to renew coverage and/or pricing of coverage.

21. If separate from financing the cost of the therapy, such as for associated wrap-around services, please describe how health plans and other payers manage the costs of administering the therapy and associated care.

In order to manage the costs of administering therapies and associated care, some health plans are investing in their own provider locations in order to exercise more direct control of treatment. To some extent, other providers are incentivized to be INN with specific health plans in order to access the facilities.

Where needed, some health plans are contracting with providers for specific treatments, even when the providers are otherwise OON. This gives the health plan improved control of drug cost and treatment reimbursements. Providers share the benefit of such an arrangement, given the potential differences in costs between INN and OON reimbursement designs. For example, an OON provider that would normally target 250% of typical INN rates to cover their costs may prefer to accept a guaranteed payment of 150% of typical INN rates.

Associated care is typically administered using existing provider contracts for the treatments, including facility and professional costs. There may be treatment bundles that are inclusive or be paid on a fee-for-service basis. In addition, access to and/or coverage of care delivered OON may be limited.

What is the Future of Access for These Therapies?

41. What are the anticipated costs or savings to health systems, plans, payers, or patients as a greater number of these therapies become available?

The anticipated net cost impact may vary by therapy and use case, but expanded coverage of gene therapy will almost certainly increase overall costs, net of savings, both near term and long-term. Many therapies may not result in net savings, as they are often lifesaving or life-extending. Some patients may be cured, which may offset a lifetime of medical costs. Many patients may see a significant improvement in quality of life. The overall financial impact will vary, depending on the disease itself, its prevalence, and the specific gene therapy.

The net cost impact of a specific therapy for an ultra-rare disease is likely to be relatively small in relation to the overall health care spend. However, over time, the cumulative increase in costs for all gene therapies may not be small. This is likely to prove true particularly as gene therapies for more common conditions arise.

44. How can future payment or coverage models for these therapies be designed in a way that drives down total health costs for the patient?

The gene therapy treatments introduced to date are costly. For many therapies, it seems unlikely that any payment or coverage model will reduce the cost of care relative to the cost for existing alternatives.

Under any model, the patient will be responsible for a small portion of the total cost of a covered treatment. This portion could still be viewed as unaffordable, depending on the underlying benefit design. While the OOP maximum provision is likely to limit the patient's financial exposure for covered treatment, the maximum has historically been more than $1,000. Patients can also incur meaningful OOP costs beyond the cost of the treatment itself, such as travel costs to and from treatment or a need for home modifications. There may also be significant patient financial exposure when care is a non-covered service or offered by OON providers/facilities. This is a critical policy aspect of patient finances for gene therapy since treatment centers are limited and may not qualify as INN for many gene therapy patients.

Many patients that could benefit from gene therapy are currently incurring OOP expenses in connection with alternative treatment. The immediate impact on patient financial exposure of receiving gene therapy will vary by patient. Over the longer term, annual recurrent costs could be reduced to the extent that a one-time gene therapy treatment is effective.

48. What other considerations should be made around benefit design to ensure access to these therapies (e.g., deductibles, cost-sharing)?

As noted above, health plan coverage almost always includes limits on patient cost-sharing (for covered, INN services), sometimes at the service level, with a cap on overall OOP costs. Depending on the plan, supplemental benefits such as transportation and lodging may be covered. Also, as mentioned previously, some therapies are currently only offered in a very limited number of facilities across the nation. Outside of these direct patient costs, there are also indirect costs that may not be immediately considered when designing benefits. This could include additional costs related to lodging, transportation, or childcare that result from the limited number of participating facilities. Supplemental benefit design, which has seen increasing interest in the commercial and public program marketplaces, is something that could be considered. Additional issues include the impact of delayed or uncertain approval process timelines, the evolving process of developing eligibility criteria.

Other considerations within benefit design discussions might include (but are not limited to):

- Should a separate OOP limit for costs directly related to gene therapy (such as facility charges, any related testing, costs of the therapy itself, etc.) be considered and how would that impact actuarial value and broader affordability constructs? Would a separate OOP limit apply solely to specific, innovative gene therapies or to all gene therapies?

- What limitations, if any, should be placed on utilization management mechanisms to ensure that reasonable access is maintained?
- If federal approvals and coverage determinations are considered coverage mandates, how quickly is coverage effectuated? When determining associated costs and risks, will the government provide the data that is needed? Would all new mandates apply in the next plan year or are mid-year changes anticipated, which would likely necessitate some additional actuarial guidance?

How Should Lawmakers Seek to Evaluate and Accomplish these Policy Goals?

58. What are the tradeoffs of expanding coverage of certain therapies in one market over another? For instance, interested parties could contemplate the strategic benefit of prioritizing a new coverage model for a certain market (e.g., small group or large group) prior to deploying it more broadly.

Prioritizing a new coverage model for only a single market is likely to introduce selection risk. Issues include:

- Plan Sponsor Selection—Plan sponsors may effectively choose to opt-in or opt-out of covering these therapies depending on which market coverage is obtained. Of particular concern to small employers, this could introduce increased friction between those who can afford fully insured coverage through the ACA's marketplace versus a more customized benefit package through self-funded plans. Affordability within this market has been challenging for decades and current debates around level-funding at both the federal and state levels highlight the impact on coverage and cost for employers and employees.
- Mandate Effectiveness—The lack of a mandate in one market will likely lead to lack of coverage, particularly in price-sensitive markets. The cost burden of providing this treatment is unlikely to be diminished in aggregate by these market coverage differences, but the pool on which such costs can be spread will be smaller, leading to higher per capita increases in markets with a mandate.
- Patient Self-Selection—Patients who presently lack coverage but need ultra-expensive, life-changing treatment of chronic conditions, would have motivation to secure coverage in a state or market where such coverage is available. From a patient access perspective, this could translate into further widening the gap between those with financial means and those without, as those with the means to relocate will be able to access treatment. In contrast, the financially disadvantaged will be less likely to be able to move and therefore may experience additional challenges to access appropriate and necessary care. From an actuarial perspective, this self-selection also impacts that risk pool and ability to use the existing knowledge of the community's health and expected utilization. This would likely have fiscal impacts on state budgets, particularly within the individual and Medicaid populations.

59. What variables should lawmakers consider when evaluating which party should bear the greatest financial risk under different contracting or coverage models?

There are multiple financial risks involved in developing and offering coverage of a new gene therapy. For example, pharmaceutical companies take risks in developing a new therapy and securing FDA approval. Our comments focus on coverage or insurance risk.

Few stakeholders beyond health plans and other payers are well situated by expertise and financial structure to assume insurance risk related to gene therapy. If coverage is mandated, government programs to promote market stability could be considered.

Over the long term, the price of covering any treatment will reflect the cost of that treatment, net of savings. For new gene therapies, the short-term insurance risk is principally related to the unknown frequency of these very high-cost treatments. How do the actual costs compare with expected costs? Health plans and plan sponsors that voluntarily choose to cover a given treatment generally understand the financial risk involved. Smaller or risk-averse payers may seek reinsurance or stop-loss coverage to reduce the level of risk assumed. Also, for large self-insurance plans and insurers, there are currently reinsurers and stop-loss carriers who are providing risk mitigation for covered gene therapies.

A coverage mandate for gene therapies could create a significant increase in the risk of high-cost claimants, particularly if the mandate covers a large number of therapies and applies only to a portion of the commercial market. Issuers affected by such a mandate could benefit from public risk mitigation, such as a government-run high risk pool or federal reinsurance programs. These programs help to stabilize the market and can also limit price increases, assuming a sustainable and predictable funding source.

Other variables to consider in the assignment of financial risk for coverage include:

- Uncertainty—Actuaries try to anticipate future costs in rate setting via trend, but trend is, in essence, an average rate of increase. Gene therapies represent ultra-high cost and very-low frequency treatments, with an uncertain long-term outcome. This adds significant uncertainty to trends with the potential for significant year-to-year variability in costs for these treatments.
- Size and financial strength—Larger entities, such as insurance companies or risk pools, have a greater ability and leverage to absorb risk than a typical employer.
- Predictability—It is easier to estimate costs for a mandate that applies to a fixed set of therapies for specified conditions with existing infrastructure (established coding, regulatory form approval, standardized contract language, ability to price for the anticipated coverage period) than an open-ended mandate.
- Funding Risk Mitigation Programs—Should the risk be shared across the entire population? For example, should it be shared with a government-sponsored reinsurance arrangement for all plans and plan sponsors?

For further insights and examination of risk mitigation mechanisms, we would encourage you to review several of our publications, including a recent issue brief on Health Risk Mitigation. Pricing risk can be addressed by risk corridors, plan specific adverse selection risk can be addressed by risk adjustment, and risk of particularly high cost cases can be addressed by reinsurance.[6]

[6] https://www.actuary.org/sites/default/files/files/ACA_Risk_Share_Fact_Sheet_FINAL120413.pdf; https://www.actuary.org/sites/default/files/files/Risk_Adjustment_IB_FINAL_060811.pdf; https://www.actuary.org/sites/default/files/2020-05/IBHealthRiskMitigation.pdf.

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The committees appreciate the opportunity to provide comments on the request for information on access to gene therapies. We would welcome the opportunity to speak with you in more detail and answer any questions regarding these comments. If we can be of further assistance, please contact Matthew Williams, the Academy's senior health policy analyst, at williams@actuary.org.

Sincerely,

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