Center on
Health Policy
at BROOKINGS

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Senator Bill Cassidy
United States Senate
Committee on Health, Education, Labor and Pensions

Re: Request for Information on Improving Americans' Access to Cell and Gene Therapies

Dear Ranking Member Cassidy:

Thank you for the opportunity to offer views on how policymakers can ensure appropriate access to cell and gene therapies.[1] This letter offers brief thoughts on why access barriers might arise and how policymakers might address them.[2] In brief, we posit that the most likely driver of access restrictions is not simply that these therapies are expensive, but rather that the nature of the illnesses that these therapies target may combine with the therapies' high prices to create strong adverse selection pressures that will encourage payers to restrict coverage. Avoiding this outcome will require effective enforcement of existing policies that address adverse selection, especially existing benefit regulations, and potentially approaches that relieve plans of some of the cost of these therapies. Additionally, given Medicaid's history as a relatively restrictive payer, ensuring that Medicaid enrollees have robust access to these therapies may require policies that directly change states' options or incentives related to coverage of these therapies.

In themselves, these therapies' high prices are not a reason to expect access restrictions

All else equal, payers are likely to place more restrictions on expensive therapies than cheaper ones. However, payers also care about the (real or perceived) benefits of therapies; private insurers generally must do so to keep their customers happy, while public programs face similar pressures from a range of stakeholders. And while emerging cell and gene therapies are generally very expensive, many will also confer very large benefits. Thus, the fact that these therapies are expensive may not, in itself, be a good reason to expect payers to tightly restrict access.

Nor are the prices of these therapies so high that they will pose novel financing challenges. Insurers already frequently encounter enrollees who incur hundreds of thousands or millions of dollars in costs in a single year. A recent analysis of data for a sample of employers found that 20% had at least one claim in excess of $1 million in the last year, and a survey of another sample of employers found that 76% had at least one such claim in the last three years.[3] Insurers that offer fully insured

[1] The views expressed in this letter are our own and do not necessarily reflect the views of the Brookings Institution or anyone affiliated with the Brookings Institution other than ourselves.

[2] For a more detailed discussion of our view on these issues, with a particular focus on access to cell and gene therapies in Medicaid, see Matthew Fiedler and Richard G. Frank, "Assessing CMMI's Proposals on Medicaid Payment for Cell and Gene Therapies" (Brookings Institution, December 12, 2023), https://www.brookings.edu/articles/assessing-cmmis-proposals-on-medicaid-payment-for-cell-and-gene-therapies/.

[3] Sun Life, "2023 High-Cost Claims and Injectable Drug Trends Analysis_press Release.Pdf," May 17, 2023, https://sunlife.showpad.com/share/qn4HBwhB34l6lpfgmz2Qd; National Alliance of Healthcare Purchaser

coverage can generally spread these costs over a large pool, and self-insured employers generally carry stop-loss coverage that allows them to manage the risk that an enrollee incurs high costs in a single year.[4] The same strategies could, in principle, work with cell and gene therapies.

Similarly, while paying for these therapies will clearly burden consumers and taxpayers, the health insurance system successfully bears far larger costs. To be concrete, one of the papers cited in the press release that accompanied this request for information projects that total national spending on gene therapies will peak at $25.3 billion in 2026.[5] This is about 5% of projected prescription drug spending and less than 1% of projected overall health care spending in that year.[6]

Adverse selection may be a more likely source of access problems

While, on their own, high prices are unlikely to create major access problems, adverse selection against plans that cover these therapies is a more plausible concern. Notably, many emerging cell and gene therapies target chronic conditions with a genetic basis. Patients who have these conditions know that they have them and are likely to place a high value on coverage for relevant therapies when choosing an insurance plan. By contrast, most patients who do not have these conditions generally have little reason to fear developing them in the future and are likely to care little about whether their plan offers coverage for the relevant therapies.

In this environment, plans that offer generous coverage for these therapies are likely to attract many enrollees who need them—and incur correspondingly high costs—without meaningfully increasing their overall enrollment. These dynamics can create incentives for plans to limit access.

These incentives will likely be especially acute in insurance markets where patients choose plans individually: the individual market; the Medicare Advantage market; and the Medicaid managed care market. Plans in those markets generally must participate in risk adjustment systems that may dull these selection incentives to some degree, but technical challenges may limit risk adjustment's effectiveness in this setting.[7] Benefit regulations in these markets should also ensure that plans offer *some* coverage for these therapies, but plans may sometimes be able to impede access in more subtle ways, such as by establishing narrow coverage criteria or hard-to-navigate prior authorization regimes, especially if regulators are not vigilant in enforcing existing requirements.

Coalitions, "Rethinking How Employers Address High-Cost Claims," May 10, 2023, https://www.nationalalliancehealth.org/wp-content/uploads/NationalAlliance_HCC-RPT_FINAL.pdf.

[4] Jalpa A Doshi et al., "Is Employment Group Insurance Financing of Expensive Gene Therapies Threatened in the United States?," *Health Affairs Scholar* 1, no. 4 (October 1, 2023): qxad043, https://doi.org/10.1093/haschl/qxad043.

[5] Chi Heem Wong et al., "Estimating the Financial Impact of Gene Therapy in the U.S.," Working Paper, Working Paper Series (National Bureau of Economic Research, April 2021), https://doi.org/10.3386/w28628.

[6] These calculations reflect the most recent National Health Expenditure Projections. The prescription drug calculation encompasses only retail prescription drugs, so projected spending on these therapies would be an even smaller fraction of total prescription drug spending including physician-administered drugs.

[7] For more on the challenges of risk adjustment in this context, see Fiedler and Frank, "Assessing CMMI's Proposals on Medicaid Payment for Cell and Gene Therapies."

Enrollee-driven selection pressures may be somewhat less acute in the group market since enrollees are matched to plans based on where they (or family members) work, and people generally are matched to employers based on many factors other than an employer's health insurance benefits. Nevertheless, these pressures will still exist to some degree, and self-insured and large group plans have greater legal scope to restrict coverage than other plan types. Selection pressures could also create problems in the market for stop-loss coverage. Notably, employers may know how many of their employees are candidates for soon-to-arrive therapies and make decisions about buying stop-loss coverage with that in mind. Some have suggested that stop-loss insurers may respond by excluding these therapies from coverage.[8] If that happens, it could make the cost of these therapies harder for employers to bear and spur more stringent access restrictions.

To be clear, we do not view it as a forgone conclusion that selection pressures will create major access problems. The benefit regulations and risk adjustment systems that exist in the markets where selection pressures are most acute (the individual market, Medicare Advantage, and Medicaid managed care) might be enough to forestall major problems. And selection pressures might be weak enough in the large group and self-insured markets that reputational considerations alone are enough to prevent major problems despite the lack of similar policies. Nevertheless, we view selection as posing risks to patient access that are worth worrying about, especially in markets where patients choose plans individually given the track record of risk adjustment and benefit regulation in those markets and the special challenges that cell and gene therapies present.

Strategies for mitigating selection problems

If selection problems are viewed as significantly imperiling access to cell and gene therapies, then policymakers have a few main options for addressing them. One is to ensure effective enforcement of existing requirements on plan benefits, including the essential health benefit requirements in the individual and small group markets, the requirement to cover all Medicare-covered services in Medicare Advantage, and the requirement to cover all FDA-approved drugs in Medicaid (and, in turn, Medicaid managed care). Policymakers could also consider extending requirements like these to large group and self-insured plans.[9] In practice, it may be hard to fully enforce these rules since plans sometimes erect hard-to-detect administrative barriers to use of high-cost treatments. Nevertheless, the difference between "good" and "bad" enforcement may still be considerable.

Another approach to addressing selection is to dull (or eliminate) plans' selection incentives. One way of doing that is to improve existing risk adjustment systems, but, as we noted above, that may

[8] FoCUS Project, "The Role of Stop-Loss Insurance and Reinsurance in Managing Performance-Based Agreements," September 16, 2019, https://newdigs.tuftsmedicalcenter.org/wp-content/uploads/2019/10/NEWDIGS_FoCUS_Reinsurance_190916.pdf; Michael Ciarametaro et al., "Are Payers Ready To Address The Financial Challenges Associated With Gene Therapy?," *Health Affairs Forefront*, accessed January 12, 2024, https://doi.org/10.1377/forefront.20180626.330036; Caroline Horrow and Aaron S. Kesselheim, "Confronting High Costs And Clinical Uncertainty: Innovative Payment Models For Gene Therapies," *Health Affairs*, November 6, 2023, https://doi.org/10.1377/hlthaff.2023.00527.

[9] For more discussion of options to expand benefit regulation for large group and self-insured plans, see Christen Linke Young, "Taking a Broader View of 'Junk Insurance,'" July 6, 2020, https://www.brookings.edu/research/taking-a-broader-view-of-junk-insurance/.

be challenging to do well in practice. Extending risk adjustment to the large group and self-insured markets would also be both operationally and conceptually challenging.

Given the potential limitations of improving benefit regulation or risk adjustment, another approach worth considering is to transfer some responsibility for paying for these therapies from plans to other entities. Depending on the context, this could be achieved in many ways. In individual and group insurance markets, plans could be required to purchase a reinsurance policy that provides some minimum level of coverage for these costs. Depending on policymakers' preferences, that reinsurance policy could be provided by a public entity or a private reinsurer. In Medicare Advantage or Medicaid managed care, a similar reinsurance approach could be used, or responsibility for paying for these services could be retained in the fee-for-service program.

There are precedents for approaches like these. The "high-cost pool" component of the individual and small group market risk adjustment programs is, in effect, a public reinsurance program that pools part of the cost of high-cost enrollees. Similarly, state Medicaid programs commonly "carve out" certain services (e.g., behavioral health care) from managed care contracts and either deliver those services directly through their fee-for-service programs or through a dedicated vendor.

Under any of these approaches, an important question would be who is responsible for managing the cost of delivering these therapies, as these approaches would weaken plans' incentives to do so. Since overuse of many of these therapies appears not to be a major concern, the most important aspect of this would be how to discipline the prices paid to manufacturers. In practice, some public sector role in regulating or negotiating these prices might be desirable, at least under versions of these policies that shifted the entire cost of these therapies away from plans. Stringent forms of benefit regulation may require similar steps to keep prices in check since they also tend to reduce plans' leverage vis-à-vis manufacturers by eliminating plans' ability to decline to cover a therapy.

Other sources of access barriers, especially in Medicaid

While we suspect that selection will be the most important driver of any access restrictions that do emerge, it may not be the only one. One other driver may be the fact that many of these therapies are: (1) administered on a one-time basis but then generate a long-lasting stream of health benefits and cost savings; and (2) at least somewhat deferable, in the sense that patients may realize much (though often not all) of the long-term benefits of treatment even if treatment is delayed. Because patients often move from one payer to another over time, this may encourage payers to implement access restrictions aimed at delaying treatment since such restrictions may shift costs to another payer while depriving patients of only a portion of the benefits of treatment. These dynamics may indeed result in some restrictions, although we note that many existing types of care (e.g., hip replacement surgery) also involve one-time costs, deliver long-lasting benefits, and are at least somewhat deferable. If these dynamics do create access problems, they could be addressed using the same types of tools used to address problems created by adverse selection.

In our view, another likely driver of access restrictions is that some payers may have a general tendency to offer overly stingy coverage that also manifests in their coverage of cell and gene therapies. We see a particular reason to worry about Medicaid, as state programs have historically imposed greater restrictions on access to many types of health care than other forms of coverage,

likely largely because of states' desire to contain the fiscal cost of their programs.[10] The wave of restrictions that states implemented following the arrival of direct acting anti-viral drugs for Hepatitis C offers a cautionary tale in this regard. Many cell and gene therapies are also administered by a relatively small number of providers, so ensuring robust access in Medicaid may often require states to be willing to pay for out-of-state care, which states may be especially reluctant to do.

Addressing these problems will require policies that change states' options or incentives. To that end, federal policymakers could consider several strategies, including strengthening federal enforcement of the requirement that state Medicaid programs cover all FDA-approved drugs, bearing a greater share of the cost of these therapies at the federal level, or finding ways to reduce the underlying prices of these therapies (such as by requiring larger statutory rebates).

Thank you once again for the chance to offer our views on this important topic. We hope that this information is helpful to you. If we can provide further information, we would be happy to do so.

Sincerely,

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[10] For more on the distinctive issues that might affect coverage of cell and gene therapies in Medicaid, see Fiedler and Frank, "Assessing CMMI's Proposals on Medicaid Payment for Cell and Gene Therapies."