THE ALLIANCE
SIGNATURE
SEMINAR

INSIGHTS REPORT:

CELL AND GENE THERAPY POLICY

allhealthpolicy.org

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ALLIANCE
FOR HEALTH POLICY

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I. ACKNOWLEDGMENTS

Seminar Sponsors and Supporters

The Alliance for Health Policy recognizes and appreciates AstraZeneca, Alnylam Pharmaceuticals, and Eli Lilly and Company for their support of the Signature Seminar program, which makes this work possible.

II. SIGNATURE SEMINAR: CELL AND GENE THERAPY POLICY

Cell and gene therapies represent a transformative frontier in medicine. These technologies have the potential not only to change treatments for individual patients but also to reshape the systems by which care is evaluated, paid for, and delivered. While early therapies target relatively small populations, broader platforms are emerging, and experts anticipate a rapid expansion of potentially curative treatments, provided the policy and regulatory environment can keep pace with innovation.

Over the next decade, experts say the future holds enormous promise and some important risks. One aspect of cell and gene therapies that does not always make headlines is how these therapies, many aiming for curative or near-curative outcomes, could reshape the broader health care system. If therapies succeed in eliminating the need for chronic or catastrophic care in conditions such as type 1 diabetes or hypertension, for example, how does that shift system priorities and operations?

The current structure of the delivery system for many of these medicines, and the unique role of patients in cell and gene therapies often results in challenging patient experiences. Requirements for highly specialized infrastructure and staffing, intensive clinical oversight, and prolonged monitoring requirements concentrate care in a small number of facilities. Combined with challenges in securing coverage for treatment, the scarcity of locations presents logistical challenges for many patients seeking treatment. As is common for patients with rare diseases—and, to some extent, for anyone navigating a complex diagnosis requiring specialized treatment—patients and their families often must travel, sometimes across state lines, to access care, adding cost, friction, and complexity. Additionally, and unique to these therapies, it is patients' own cells or genes that ultimately contribute to their cure, embedding the individual patient directly into the manufacturing and delivery process and creating a unique demand on patients as part of their own therapy.

Cell and gene therapies also present unique technical and regulatory considerations. Cell therapies involve modifying or using living cells, grown or engineered outside the body, to deliver therapeutic effects, while gene therapies target the underlying genetic causes of disease by introducing, replacing, or inactivating genes within cells. Both require extensive clinical evaluation, upfront investment, and long-term monitoring, but differ in regulatory emphasis: cell therapies focus on manufacturing consistency, donor eligibility, and immune response risks, whereas gene therapies are subject to rigorous preclinical testing, vector-specific safety assessments, and ongoing patient follow-up due to permanent genomic changes.

Rapid scientific and technological change presents a challenge for the U.S. policy process. According to the Congressional Research Service, fewer than 9 percent of members of Congress have backgrounds as scientists, engineers, or health professionals, which can make integrating complex scientific concepts into legislation challenging. In conversations with congressional staff, many acknowledged the importance of cell and gene therapies but noted limited opportunities or support to deepen their understanding. Helping to bridge this knowledge gap is central to the Alliance's mission of supporting informed policymaking.

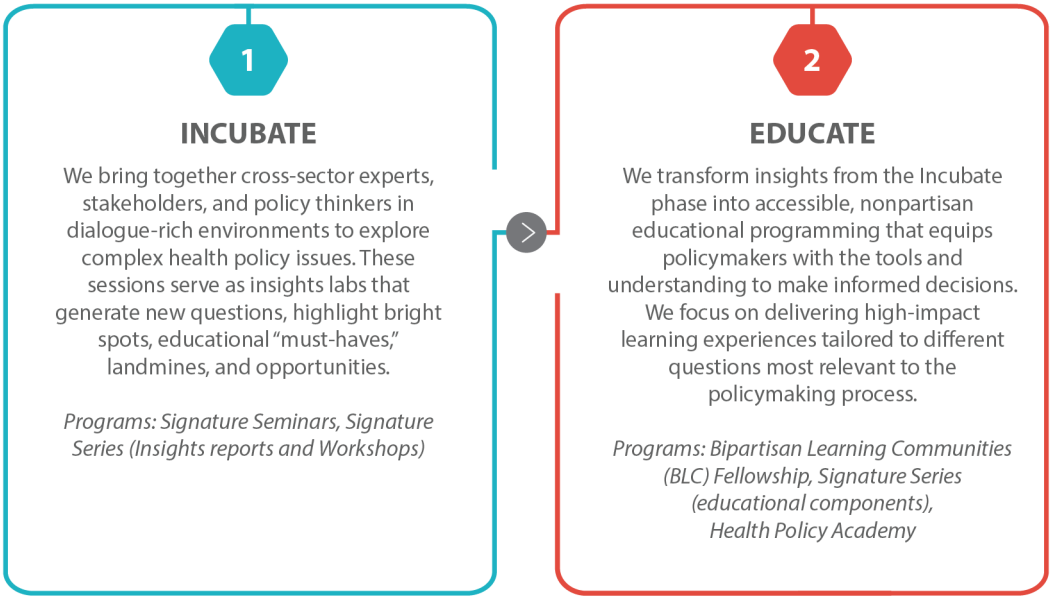
This insights report summarizes findings from the Alliance for Health Policy's 2025 listening process on cell and gene therapy policy. Drawing on interviews with experts from multiple perspectives, it highlights current trends, priority issues, and deeper-level concerns, offering Congress and agency policymakers a foundation for understanding how these rapidly advancing therapies connect to policy questions that will influence the structure of the U.S. health care system.

About the Alliance for Health Policy

The Alliance for Health Policy is a nonpartisan, nonprofit organization dedicated to helping policymakers and the public better understand health policy, the roots of the nation's health care issues, and the trade-offs posed by various proposals for change.

THE ALLIANCE'S INCUBATE TO EDUCATE MODEL

The Alliance applies a unique two-part "Incubate to Educate" model to its programming.



The Signature Seminars mark the first stage of our program lifecycle, "Incubate." This stage focuses on gathering insights and convening experts to provide guidance on educational opportunities around key policy issues. Each seminar brings together a diverse set of voices from across the health care policy community, including government staff, academics, patients, providers, payers, innovators, and technical experts.

These insights directly inform the second stage of our program lifecycle, "Educate," during which the Alliance develops and delivers targeted educational programming for legislative staff and the broader health policy community.

III. BACKGROUND

Listening Tour Summary

From September through November 2025, the Alliance for Health Policy conducted 17 in-depth interviews (IDIs) to inform this report. The findings provide an overview of the current cell and gene therapy policy landscape, highlight pressing gaps and priorities, and identify opportunities for future discussion.

Design and Methods

Participants represented a broad cross-section of the health policy community, including patient advocates, current and former state and federal policymakers, nonprofit organizations, physician-researchers, public and private payers, and private-sector stakeholders. Selection prioritized bipartisan and multi-stakeholder perspectives, as well as policy and political expertise that reflects the diversity of the Alliance community. Each 30-minute IDI was conducted via Zoom using a semi-structured format. Findings are qualitative and intended to provide directional insights. Attribution throughout the report is designed specifically to give insight into the interviewee's role and organization without specificity that may jeopardize confidentiality of the conversation.

Outcomes

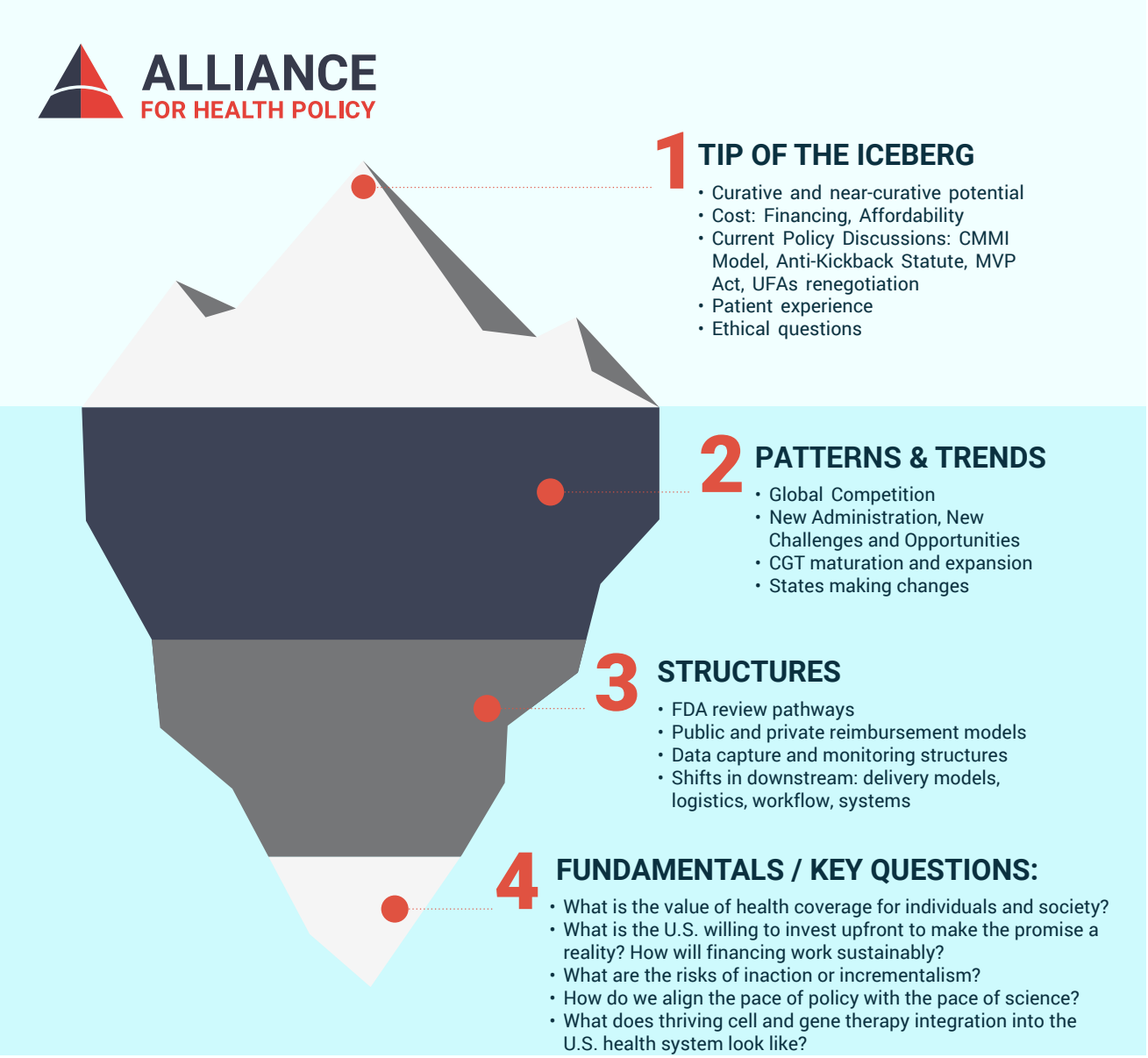
Insights from these IDIs helped shape the Signature Seminar on Cell and Gene Therapy Policy, which brought together a wider set of voices from across the health care policy community to explore this high-priority topic. Interview participants also contributed to identifying potential invitees and discussion topics for the seminar workshops.

The Alliance published an outcomes report from these workshop sessions. The full report, including detailed examples and unattributed quotes, is available on the Alliance website. To view it, visit the Alliance site, click here, or scan the QR code.

See Seminar Report Here:

Scan using your mobile device to read the full report.

IV. THE ICEBERG MODEL: FROM TIMELY ISSUES TO SYSTEMIC AND FUNDAMENTAL INSIGHTS



The Alliance uses a structured framework to organize expert perspectives on cell and gene therapy policy. This approach draws on the "iceberg model" from systems thinking, first introduced by anthropologist Edward T. Hall to illustrate how much of culture and communication lies beneath what is visible. Systems thinking not only identifies individual elements within a structure but also highlights how they interact and influence one another. It has been widely adopted in organizational strategy, business and management, and the public and private sectors.

Applying this model, the Alliance finds that widely discussed issues, such as affordability, financing, and the transformative potential of these therapies, represent only the visible tip of the cell and gene therapy policy conversation. Beneath the surface lie deeper structural and systemic dynamics that often have greater influence on policy outcomes. This framework allows the Alliance to clarify both immediate, high-profile topics and the foundational forces shaping the field.

Tip of the Iceberg: Hot Topics in the Cell and Gene Therapy Landscape

At the tip of the iceberg are the surface-level issues that dominate headlines and congressional debates. These include the curative or near-curative potential of therapies, questions of affordability and financing, ongoing policy initiatives such as the Cell and Gene Therapy (CGT) Access Model and the Medicaid Value-Based Purchasing Act, renegotiations of user fee amendments (UFAs), as well as patient experience and ethical considerations. While these topics are highly visible, they represent just a fraction of the factors shaping policy.

Below the Surface: Patterns and Trends

Beneath this surface layer are medium-term trends, typically unfolding over three to five years, which shape the broader environment. These include global competition in cell and gene therapy, opportunities for innovation under the current administration, the maturation and expansion of scientific and market capabilities, and policy changes at the state level.

A Bit Deeper: Structures That Impact the Policy Environment

Deeper still are structural dynamics that influence how the system functions and establish the framework for policy implementation. This includes Food and Drug Administration (FDA) review pathways, Centers for Medicare & Medicaid Services (CMS) and private reimbursement models, data capture and long-term monitoring structures, and downstream effects on delivery models, logistics, workflow, and health care systems. These structural "guardrails" set the opportunities, incentives, and barriers for translating therapies from the lab to the bedside.

Fundamentals / Key Questions: Opportunities for Greater Exploration and Understanding

At the foundation of the iceberg are the fundamental questions that will shape the future of cell and gene therapy in the U.S. These include who will have access to these groundbreaking treatments, how much the nation is willing to invest upfront to realize their promise, how financing can be structured sustainably, what the risks are of inaction or incremental approaches, how to align the pace of policy with the pace of science, and what a thriving integration of cell and gene therapies into the U.S. health system looks like.

V. TIP OF THE ICEBERG: HOT TOPICS IN THE CELL AND GENE THERAPY LANDSCAPE

Curative and Near-Curative Potential

Experts across stakeholder groups highlighted the curative or near-curative potential of cell and gene therapies and the transformative benefits they can provide for patients with conditions ranging from rare genetic diseases to cancers, many of whom previously had no treatment options. They also noted that these therapies differ fundamentally from traditional medicines, both in how they are developed and how they function in the body.

> *"These are the types of products that we really hope for from the investments in clinical medical innovation and the ability to cure the previously incurable or much more effectively treat conditions."*
>
> - Executive, Public Payer Association

> *"I would say what comes to mind for me when someone mentions cell and gene therapy is the potential. I feel like there is tremendous benefit that's available for patients that are really stuck with some devastating diseases. Some cases have unmet medical needs, and cell and gene therapies are really changing the paradigm by bringing transformative, durable treatments to market for patients that previously are in disease areas where you haven't had treatment."*
>
> - Director, Biotech Trade Association

Cost: Financing and Affordability

Alliance community experts emphasized that while these therapies have transformative potential, their scale of investment and cost exceeds what the current system is designed to handle. Addressing how these therapies are financed, how they attract investment, reach doctors and patients, and are ultimately paid for, is a top priority. As more treatments for additional conditions reach the market, this focus becomes increasingly urgent.

> *"They are super, super expensive… Some of these therapies cost $1 million up to $5 million. And when we start seeing therapies for conditions that affect thousands or millions of people, say, hemophilia or sickle cell disease or some cancers, I just don't see… how sustainable that would be to make it accessible for everyone."*
>
> - Physician Executive, National Health Insurance Company

> *"And then there's the product that never actually gets to market, even though they have successful clinical trials, because there's no way they'll ever see the return on investment."*
>
> - Former Federal Regulator

> *"If we see another couple of hundred of these sorts of therapies coming to market over the next five to ten years, [absorbing the high costs is] going to be challenging."*
>
> - Executive, Public Payer Association

> *"[In contrast to many economic predictions] We've been doing this big model for about 10 years of doing 10-year projections of how big the cell and gene therapy product market is going to become based on the current pipeline. We can't get the total spend over $30 billion for the whole country."*
>
> - Strategic Director, Academic Medical Organization

Current Policy Discussions: CMMI Model, Anti-Kickback Statute, MVP Act, UFA Renegotiation

Experts noted several timely policy discussions that will impact cell and gene therapies in the near term. The most frequently mentioned was the CMS Center for Medicare & Medicaid Innovation (CMMI) Cell and Gene Therapy (CGT) Access Model. This voluntary pilot enables the agency to negotiate outcomes-based agreements (OBAs) for selected gene therapies on behalf of state Medicaid programs, starting with sickle cell disease. By centralizing negotiations and offering implementation guidance, the model aims to expand access to high-cost, transformative therapies while reducing operational and financial barriers for states. Experts also noted the Medicaid Value-Based Purchasing for Patients (MVP) Act, which aims to modernize the framework for value-based purchasing arrangements in Medicaid. Its main goal is to increase patient access to high-cost, transformative drugs, such as cell and gene therapies. Finally, experts highlighted the impact of negotiations under the Prescription Drug User Fee Act, which include specific provisions to improve the development and review of these therapies, provide funding for regulatory review, enhance review processes, support the strengthening of the Center for Biologics Evaluation and Research (CBER), and promote the development of treatments for rare diseases.

> *"I do think that the CMMI demo gives us a great opportunity to learn some things. If you take a more comprehensive approach, if you have an easier path to enter into these value-based agreements, I would like to see how that can be more freeing for the field overall."*
>
> - Executive, Biotech Trade Association

> *"The Cell and Gene Therapy Access Model over at CMMI for sickle cell disease is actually very innovative. They took advantage of their flexibilities as CMMI within the Anti-Kickback Statute so that manufacturers can support fertility preservation services. CMS is also allocating funding to states who can then direct that funding to community partners that can provide additional wraparound services, like transportation and lodging..."*
>
> - Director, Biotech Trade Association

> *"There's the MVP Act [Medicaid Value-Based Purchasing for Patients Act] folks are working on..."*
>
> - Principal, Health Policy Consulting Firm

> *"So if it's a given that this can only be done at a finite number of places in the United States and you don't happen to live there. Is it okay if the sponsor pays for your airfare and puts you up in a hotel while you're getting that treatment and not being accused of violating the anti-kickback statute? I believe that there was an advisory opinion that said they can do that… They need it [the therapy]. It's recommended, but really the only way it can happen is if somebody takes care of those nonmedical costs to actually get them there and keep them there."*
>
> - Director, National Patient Advocacy Organization

> *"Congress has to get their rare pediatric review voucher straightened out - can we get that fixed - seems like very low hanging fruit."*
>
> - Former Federal Regulator

> *"We are in a unique spot where FDA and industry are negotiating the persistent regular things—[User fee] reauthorization, right?"*
>
> - Former Federal Regulator

Patient Experience

Patient stories, such as that of Baby KJ (KJ Muldoon), have become some of the most visible examples of how cell and gene therapies capture national attention. In early 2025, KJ became the first patient to receive a personalized gene-editing therapy developed at the Children's Hospital of Philadelphia specifically to treat KJ's rare genetic metabolic disease. Experts noted how his journey, from a life-threatening diagnosis to a groundbreaking therapy tailored to his own genetic profile, illustrates why many families navigating these treatments are often described as "patient warriors." They explained how the term reflects more than courage; it highlights the extraordinary role patients play in their own care, as their bodies become central to the therapeutic process itself. Stories like KJ's have entered headlines and the public discourse, spotlighting both the promise of these therapies and the demanding path patients and families must navigate to access them.

> *"Patients have a great deal of enthusiasm for these treatments and many are so eager to be included in trials, because of the promise."*
>
> - CEO, National Patient Organization

> *"Right now [a key] challenge is… the warrior [patient] angle… what the patients have to endure... to get these therapies and what it means for them long term..."*
>
> - Principal, Health Policy Consulting Firm

> *"The patient experience depends on the therapy but can be really intense. For example with sickle cell patients, they have their immune system suppressed, they are in the hospital for months with the gene therapy, they lose their fertility... a really intense experience."*
>
> - Former Policy Executive, National Patient Organization

> *"Say you are a Medicaid patient in Alabama and you need to go to Texas in order to receive your cell or gene therapy. Your provider in Texas has to go through the Alabama Medicaid agency and get credentialed. And that can lead to additional delays. We're talking about months sometimes for patients that have progressive conditions that just create roadblocks and even a deterrent for going down the route of delivering a cell and gene therapy."*
>
> - Director, Biotech Trade Association

> *"You know, I think [congressional staff] folks, especially in the gene and cell therapy field, are really interested and excited about what's going on. Like the baby KJ story, there are things that are increasingly breaking into sort of the mainstream news that get people excited."*
>
> - Executive, Physician Researcher Organization

Ethical Questions

The promise of these new technologies brings both excitement and caution. Rapid scientific advances create a gap between public understanding and how the science actually works, which experts noted can contribute to distrust. These therapies also raise important ethical questions that warrant careful consideration.

> *"I do think that as technology advances and we're better able to precisely change genes, I think, [we are seeing reactions]... some states are trying to regulate or ban the use of mRNA technology and I think making sure that there's public readiness and acceptance of these technologies… [we need to ensure that] we're not advancing these technologies faster than we're able to get comfort level and acceptance."*
>
> - Director, National Patient Advocacy Organization

> *"These therapies present specific challenges as they develop. For instance, what about the parent who is deciding whether to give their child a gene therapy that is a "one-shot" opportunity, deciding to close that door to a potential future, better gene therapy, not knowing how long their child will live?"*
>
> - Former Policy Executive, National Patient Organization

> *"I think we are stepping through a fundamental threshold as a society with these treatments… and some people will be less comfortable with that."*
>
> - CEO, National Patient Organization

VI. BELOW THE SURFACE: PATTERNS AND TRENDS

While public discussions often focus on high-profile patient stories or visible policy debates, experts interviewed by the Alliance emphasized the deeper trends shaping the cell and gene therapy landscape. These trends span global competition, shifts in federal policy, the maturation and expansion of the science, and evolving state-level considerations. Together, they reveal forces that influence how these transformative therapies are developed, financed, and delivered, providing context for the challenges and opportunities policymakers face today.

Global Competition

Experts discussed the United States' role in the global competition to develop these cutting-edge therapies and underscored the desire to maintain America's lead in scientific research and development.

> *"[The U.S.] is so different than the rest of the world. So everybody else is "all in" on [cell and gene therapy development] and wants to utilize it and sees the potential—sees the reality of it, as well as the future potential. [In] Asia, the investment there is incredible. I'll be in Berlin because Europe's all in on it too, but it's also being built in South America, you know, Africa, others…"*
>
> - Principal, Health Policy Consulting Firm

> *"I think also the administration and this Congress are very much interested in preserving the United States' leadership role as in biotech innovation."*
>
> - Executive, Biotech Trade Association

> *"[Physician researchers] are very concerned about cuts to NIH funding…Proposed cuts to facilities are huge for cell and gene therapies because building these manufacturing facilities at academic centers requires years of investment. They're very expensive. A lot of the small biotechs and then eventually the larger industry partners depend on translational science that comes from NIH investments and from the academic centers that are doing this [work]... it's this whole pipeline of innovation."*
>
> - Executive, Physician Researcher Organization

New Administration, New Challenges and Opportunities

Experts expressed optimism that the Trump administration's approach may avoid the incrementalism of previous administrations, potentially supporting the broader policy and structural changes needed to strengthen the cell and gene therapy pipeline and benefit patients. At the same time, there is concern about predictability for those submitting therapies for regulatory review.

> *"...[T]he good thing is that they definitely think about things differently. So I think there will be opportunities with this administration over the next few years to try and get them to think differently about how they do things in terms of, you know, paying for these innovative therapies."*
>
> - Principal, Health Policy Consulting Firm

> *"…[T]his administration… has been really supportive of cell and gene therapies."*
>
> - Executive, Physician Researcher Organization

> *"I think the uncertainty in the recent past few months has led to a lot of worry about whether to be able to move forward… So having more transparency from the agency about what they're thinking is and what their considerations are, especially, you know, how they're thinking about nonclinical and manufacturing data... I think more clearly about what their data requirements are and where they're going to exercise flexibility, given the nature of these products."*
>
> - Former Federal Regulator

CGT Maturation and Expansion

Experts noted that the science has the potential to extend beyond rare and ultra-rare conditions, with many more therapies likely to become available simultaneously. Coupled with several years of experience since the first therapies were tested and approved, this suggests that these issues are no longer entirely new and can now be informed by emerging experience and lessons learned.

> *"How do we actually think through the expansion of cell and gene therapy to treat everything, everyone that could be treated with the current commercial ones, but more importantly to look at some of these ultra rare diseases. And then also still supporting discovery, because that's the other part of this."*
>
> - Academic Physician / Division Chief

> *"I would say that the idea around some of the different models was probably not necessarily new, at least not in the past 10 years."*
>
> - Executive, Public Payer Association

States Making Changes

Federal budgets are not the only ones impacted by the advent of these treatments. At the state level, policymakers are closely watching impacts on Medicaid. Many current therapies are designed for complex patients and rare diseases that often begin in early life, which has garnered the attention and action among Medicaid program administrators, as, according to a recent American Academy of Pediatrics analysis, Medicaid insures nearly 50% of all children in the United States, including millions with special health care needs.

> *"Medicaid's coverage footprint for medically complex children and recognizing that cell and gene therapies, to maximize their value, need to be administered early in life, most likely. So I think that creates a disproportionate impact on the Medicaid program. So I think states would need some solutions that acknowledge that Medicaid is a partner in this, but Medicaid should not necessarily be the primary fiscal contributor."*
>
> - Executive, Public Payer Association

> *"There are new cost obligations for states and cost sharing and administrative cost and administrative expenses that didn't previously exist… and overall we're seeing states and more of a belt-tightening… So, in a nutshell, there's just not going to be a lot of ability for states to absorb new costs, particularly ones that might be at the scale that more and more curative therapies and cell and gene therapies could be."*
>
> - Executive, Public Payer Association

> *"[T]he states limit their ability to, you know, limit some of their levers on what they can do to manage costs. So there's been a huge amount of energy and… movement in the Medicaid space."*
>
> - Executive, Private Payer Solutions Company

VII. A BIT DEEPER: STRUCTURES THAT IMPACT THE POLICY ENVIRONMENT

Beneath the visible trends in cell and gene therapy policy lie the underlying structures that shape how the system operates and how therapies move from development to patient care. Experts highlighted the influence of FDA regulatory pathways, CMS and private reimbursement models, data capture and monitoring systems, and downstream shifts in delivery models, logistics, workflows, and hospital operations. Understanding these structures helps clarify both the opportunities and challenges for ensuring that innovative therapies reach patients safely, sustainably, and effectively.

FDA Pathways

Experts highlighted key policy opportunities in addressing the gap between the requirements for evaluating the safety and efficacy of cell and gene therapies and the current structure and approaches of the FDA.

> *"…[T]hings like leveraging free development programs or having a platform that can be used across several small indications will make [some therapies that do not make it to market] more viable from a cost perspective."*
>
> - Former Federal Regulator

> *"I think there could be further refinement of accelerated approval... I think that there's probably still some work that needs to be done to provide clarity and certainty about how FDA is going to apply [accelerated approval] outside of oncology… [in the] neurology and gene therapy space. I think probably there will be talk about new pathways for very rare diseases, because then those are particularly amenable to cell and gene therapies, particularly if we get down the path of individualized therapy."*
>
> - Former Federal Regulator

> *"There's only one standard for approval at FDA, but as product types have become more complex and precise, that standard has gotten more and more difficult to apply. The question is how to bridge from one product to another [within regulatory frameworks]. When Dr. Peter Marks was at the FDA, he used a soda machine analogy: with a single machine, you can make a peach Fanta while still having eight other Fanta flavor options. The goal with these therapies is the same. Once you have the basics down, you can modify them to meet individual patient needs."*
>
> - Former Federal Regulator

> *"I think there continue to be issues in terms of the regulatory pathway and how that's working."*
>
> - Former Senior Policy Leader, Patient Advocacy Organization

> *"And I think we need to have some serious policy conversations and whether it's at the age of…[creating] new authorities or that they need to just utilize their existing lines in a different way."*
>
> - Former Federal Regulator

Public and Private Reimbursement Models

Experts repeatedly highlighted concerns about the sustainability and costs of federal health programs, as well as potential near-term financial challenges for Medicaid and Medicare in covering these therapies. They also noted ongoing efforts to explore innovative public and private approaches to payment.

> *"The system knows how to pay really well for more moderately priced things that are taken by millions and millions of people. But on the opposite end of the spectrum, not so much."*
>
> - Executive, Biotech Trade Association

> *"These are all one in a million kind of treatments. So even the biggest employers are only going to have a handful of claims in a year. It's really hard for them to have the leverage to actually make a huge difference. This is a place where employers have to band together to create leverage and to create influence. And ultimately that's the way you create a rising tide that lifts all ships. So that's why we are pooling risks."*
>
> - Executive, Private Payer Solutions Company

"It's a bit of a canary in the coal mine. Many of the innovations in these therapies provide a space to experiment with other approaches before scaling to larger areas. If you want to try new coverage models, risk pools, or financing payment models, you can test them on a smaller scale here and see how they work. It's the right area for experimentation that can then be applied more broadly to other products."

- Strategic Director, Academic Medical Organization

"There's a lot that the private sector needs to do right now in the absence of something that's really fulsome. The problem isn't a bunch of little things the government can do. You need to disrupt to actually solve it. It's sort of like the government either needs to do it, or make sure it doesn't get in the way of the private sector doing it."

- Executive, Private Payer Solutions Company

"Only Congress can pass a new benefit category. So that's [the idea of a] Medicare Part G for Gene, and you'd pass a new benefit category and just basically say… you could tie, theoretically, just like they did with Cancer Compendia, you could tie A, B, C, or A, B, D, and Medicaid together, and just pay invoice or acquisition, whichever is the lesser… So that's… one way to address it. There's a white paper on that."

- Executive, Health Care Consulting Firm

Data Capture and Monitoring Structures

Because cell and gene therapies often use innovative trial designs with fewer participants, post-market surveillance needs differ from those of traditional medicines. Payers and other stakeholders anticipate closely monitoring these therapies' real-world performance to inform ongoing coverage and payment decisions.

"The clinical policy is really hard to get right here because these therapies tend to be approved in an expedited accelerated fashion, often with a couple dozen patients in their pivotal trials. We constantly reassess the evidence to try to make sure the policies are nuanced and reflective of the best understanding of the effectiveness and the durability of these drugs."

- Executive, Private Payer Solutions Company

"I think that there's a lot to be said around data collection and analysis as part of administering value-based payment agreement arrangements, whatever form they take. State Medicaid agencies really will not have the capacity to take this on an individualized basis. I don't think that we can continue in a world where each state is arranging and negotiating their bespoke arrangement with a pharmaceutical manufacturer."

- Executive, Public Payer Association

"When I was at CBER, we were thinking about how to leverage data across programs so we're not reinventing the wheel each time. If that can happen, it will make things easier and more viable from a cost perspective."

- Former Federal Regulator

Shifts in Downstream: Delivery Models, Logistics, Workflows, and Systems

Experts highlighted the downstream impacts of these therapies on health systems, particularly the unique roles hospitals play in their administration. Teams face expanded responsibilities and require new approaches to staffing, workflows, and clinical operations. Beyond these immediate effects, respondents noted that near-curative therapies could reshape the broader health system and patient experience over time by reducing the need for ongoing, palliative, or multi-step care.

"Another part of this which is interesting is there's a lot of talk about manufacturing that could actually happen at the bedside. Hospital systems will be the manufacturer."

- Principal, Health Policy Consulting Firm

"As cell and gene therapies expand beyond atypical disease areas into things like neurological conditions, you're going to have different specialty teams within the hospital having to adapt their practices. Many neurology teams don't have that sort of clinical workflow in place like the hematology clinic does to do things like cell collection and administration. So there's going to be this need to rethink clinical workflows but also the allocation of resources. There are some concerns that you know, institutions are going to have to staff up those sort of cell collection and infusion sites."

- Executive, Biotech Trade Association

"[Among patients] you may see an impact on identity, for example, we know of gene therapies where patients have an issue, go through the gene therapy, and are cured. What happens if you have curative treatments? Will it be similar to cochlear implants among the deaf community where that truly is something where people have built an identity based on their shared experience?"

- CEO, National Patient Organization

"So clearly lots of patient impact. You're going to see tens of thousands of cancer patients living for five, ten years that otherwise would have died. Beyond the human impact, there are opportunity costs if providers are not supported in building the capacity and capabilities to deliver these products. Right now, because of the reimbursement challenges, many of the hospitals have limits on how many patients they'll treat per month because of the reimbursement risk on back office lab processing and patient flow. Building out capacity is a real bottom line."

- Strategic Director, Academic Medical Organization

VIII. THE FUNDAMENTALS: OPPORTUNITIES FOR GREATER EXPLORATION AND UNDERSTANDING

At the deepest level are foundational questions that rarely dominate public debate but ultimately shape how these therapies will fit into the U.S. health system. These include who will be able to access them, what level of upfront investment the nation is willing to make, how to balance scientific innovation with sustainable financing, and how policy can keep pace with rapid advances in the science. These fundamentals provide the context for understanding what it will take to integrate cell and gene therapies into the health care system in a way that is equitable, sustainable, and aligned with their scientific promise.

Who Will Get Access to These Treatments?

Experts described access through multiple dimensions. Financial capacity, having insurance that covers genetic testing and the therapies themselves, was central, as was geographic proximity to centers that can deliver treatment. Several interviewees also emphasized the role of time: patients and caregivers who can take extended time off to navigate complex requirements often have an advantage. Nearly every expert underscored the risk that these factors will lead to unequal access to cell and gene therapies.

> *"There's their access issues about whether people ever tested or have the ability to get tested."*
>
> - Former Senior Policy Leader, Patient Advocacy Organization

> *"With CAR T-cell therapies, only one in five patients who's actually eligible to receive a CAR T goes on to receive it. And that's because of a host of barriers that we're uncovering and trying to work on in the long run. But let's change that number."*
>
> - Director, Biotech Trade Association

> *"We're at an inflection point because there is a lot of opportunity there. But if we don't really incentivize and address the barriers that are getting in the way of this outpatient and community migration, we could be stuck with cell and gene therapies becoming something that's seen as what you go to… the specialized academic medical center for and something that's less accessible for the average patient who may not have the resources to travel and stay near these centers."*
>
> - Director, Biotech Trade Association

> *"I think that we haven't yet seen the official start of CGT access."*
>
> - Academic Physician Researcher/Division Chief

What Is the U.S. Willing to Invest Upfront to Make the Promise a Reality? How Will Financing Work Sustainably?

While experts agreed that these therapies require far greater, and earlier, investment than traditional treatments, they emphasized that the financing conversation is ultimately one about values. How much upfront investment is the U.S. willing to make for long-term benefit? Where does this priority sit among other national needs? And what mechanisms are we willing to use to pay for it?

> *"If we pay for these things now, what will we save in our health care system? I would love to see us make some bold moves."*
>
> - Executive, Biotech Trade Association

> *"The financial ecosystem, even with the incentives for rare diseases are there, is that going to be enough for a company to invest what it would take to do a therapy like this for a relatively small population?"*
>
> - Former Senior Policy Leader, Patient Advocacy Organization

What Are the Risks of Inaction or Incrementalism?

Several experts highlighted the risks of maintaining the status quo in cell and gene therapy policy. They noted that existing policy structures are not designed to accommodate these novel treatments, and while it may seem easier to avoid tackling these challenges, doing so carries significant political, economic, and patient risks.

> *"[T]he FDA role may not be as strong as it was before. And so you may be seeing… where others like a European Medicines Agency to Medicines and Healthcare products Regulatory Agency (MHRA, which is… UK equivalent of FDA), to practically, China to Australia, [and] other countries playing a stronger role in investment in research and development [and] also in deployment commercialization."*
>
> - Principal, Health Policy Consulting Firm

> *"We don't have great mechanisms right now to solve for the upfront cost and translating that overall fiscal value proposition to the health care system into individualized payers who may be the ones that make that initial payment for the treatment… if we don't solve this, then I think we see payers kind of go to the usual toolbox of very stringent prior authorization criteria and making access really challenging, not because there is a lack of relief in the efficacy of the therapy, but because the system, the payer is not set up to absorb the cost in a fiscally sustainable manner."*
>
> - Executive, Public Payer Association

How Do We Align the Pace of Policy with the Pace of Science?

Experts noted that while the scientific knowledge to expand these therapies exists, the pace of policy development, rooted in an 18th-century framework, lags behind the rapid growth and evolution these treatments demand.

> *"We need to have financing and coverage innovation that matches the clinical innovation that we see to acknowledge that these are different than what we've encountered in the past."*
>
> - Executive, Public Payer Association

> *"I think that the one was understanding what the treatments are and are not, and that they may not be the definitive thing for all individuals with any particular condition, and that there are many conditions that in theory could also benefit from what we're learning with the relatively small number of conditions for which a cell or gene therapy is currently approved. And so the possibilities I think are enormous for this to be applied as a technology across more conditions, including conditions that are not inherited. So I think we're just scratching the surface, even though gene therapy is not new… I think there are more conversations now than ever about cell and gene therapy."*
>
> - Academic Physician / Division Chief

What Does Thriving Cell and Gene Therapy Integration into the U.S. Health System Look Like?

Because these therapies have emerged incrementally and often target small patient populations, the broader impact they could collectively have on the health care system has not been fully visualized or explored. Experts highlighted a range of outcomes that would signify success, from realizing the full scientific potential of these treatments to ensuring patient access and sustainable financing.

> *"I think of four buckets when I think about success. One is everyone who needs gene or cell therapy because of the conditions has access to the medicines. Two, I always think about the quality of care. If you do get cell therapy or gene therapy, we actually get into the clinical outcomes that we are going after. So people are getting cured, people feeling better. And then [three] at a macro level for us to be able to offer good quality, accessible gene therapies and cell therapies at an affordable and sustainable rate financially. The last [fourth] bucket that I'm thinking about is making sure that all the different groups of people, however you segment them, are getting access in an equitable way. The equity piece is huge. "*
>
> - Physician Executive, National Health Insurance Company

> *"Speaking from a patient perspective, a system where a patient or family affected by a condition or knowledge of impact of that condition [ie, genetic testing] has the earliest most appropriate intervention that limits the effects on their health and well-being."*
>
> - CEO, National Patient Advocacy Organization

"I think a successful outcome is where no one payer feels that they are unduly burdened because of their coverage footprint for essentially being holding the bag for this. And we can think about what that looks like and also about having mechanisms that translate fiscal value as individuals move from payer to payer."

- Executive, Public Payer Association

"No one is going to be able to solve any of these issues on their own, and it requires a lot of collaboration, a lot of iteration, and putting our heads together. One of the things that I was actually thinking might be helpful is to catalog a lot of the out-of-the-box ideas."

- Executive, Biotech Trade Association

IX. CONCLUSION

Cell and gene therapies offer transformative and sometimes curative treatments for patients with previously untreatable conditions. Yet realizing their promise depends on more than science; it requires coordinated action across policy, financing, and health care delivery systems.

While breakthroughs in efficacy capture attention, underlying challenges—including access, sustainability, and equitable distribution—will determine whether these therapies reach all patients who need them. Experts highlighted the importance of adapting regulatory and reimbursement frameworks, investing upfront, and fostering collaboration among payers, providers, and patients.

Success in integrating these therapies means achieving their scientific potential, strong clinical outcomes, financial sustainability, and equitable access. Doing so will require bold, innovative approaches and ongoing collaboration to ensure that the promise of cell and gene therapies becomes a reality for all.

ALLIANCE
FOR HEALTH POLICY