THE ALLIANCE
SIGNATURE
SEMINAR

SEMINAR REPORT:

CELL AND GENE THERAPY POLICY

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ALLIANCE
FOR HEALTH POLICY

CONTENTS

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II. SIGNATURE SEMINAR: CELL AND GENE THERAPY POLICY

Cell and gene therapy (CGT) represents 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 therapy 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 therapy also presents 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 therapy but noted limited opportunities or support to deepen their understanding. Helping to bridge this knowledge gap is central to the Alliance for Health Policy's mission of supporting informed policymaking.

This Seminar Report summarizes key themes and insights from the Alliance's 2025 Signature Seminar workshops, which brought together experts, policymakers, and stakeholders to explore the complexities of CGT. The U.S. stands at a pivotal moment in the history of cell and gene therapy, with some of the most promising areas of new treatments for common and rare diseases coming out of CGT research and clinical trials. At the same time, if policymakers fail to intervene, the costs of these new therapies may have significant implications for a health care system facing increasing demands as more therapies come to market. This report aims to provide Congress and other decision-makers with a clear roadmap for how to educate and inform on the past, current, and future of CGT.

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:

1

INCUBATE

We bring together cross-sector experts, stakeholders, and policy thinkers in dialogue-rich environments to explore complex health policy issues. These sessions serve as insights labs that generate new questions, highlight bright spots, educational "must-haves," landmines, and opportunities.

Programs: Signature Seminars, Signature Series (Insights reports and Workshops)

2

EDUCATE

We transform insights from the Incubate phase into accessible, nonpartisan educational programming that equips policymakers with the tools and understanding to make informed decisions. We focus on delivering high-impact learning experiences tailored to different questions most relevant to the policymaking process.

Programs: Bipartisan Learning Communities (BLC) Fellowship, Signature Series (educational components), Health Policy Academy

The Signature Seminars mark the first stage of our program lifecycle, "Incubate." This phase focuses on gathering insights and convening experts to provide guidance on 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, "Educate," during which the Alliance develops and delivers targeted educational programming for legislative staff and the broader health policy community.

Signature Seminar on Cell and Gene Therapy

III. INSIGHTS FROM THE ALLIANCE'S LISTEN-FIRST APPROACH

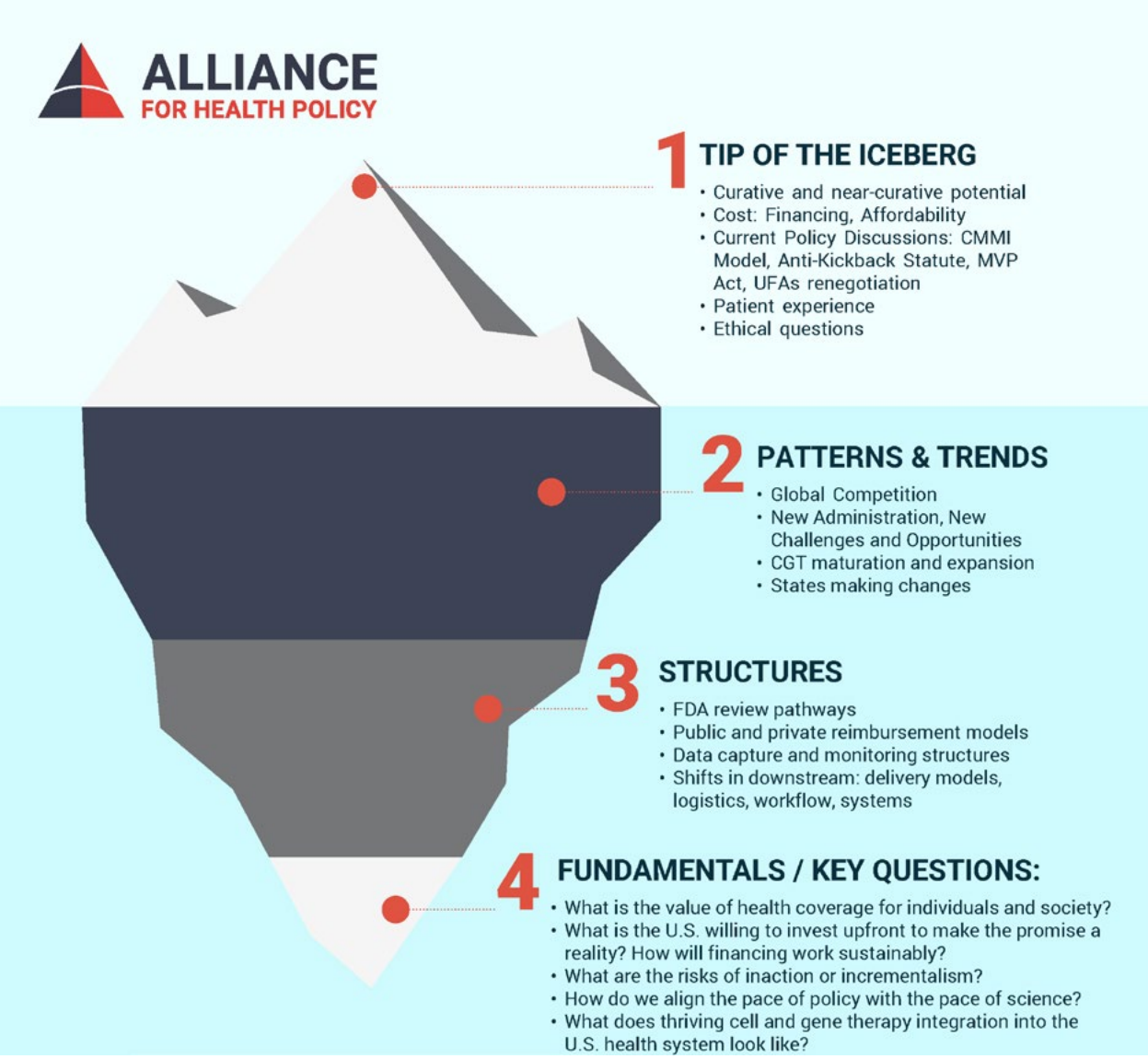
The Signature Seminar on Cell and Gene Therapy began in September 2025 with a listening tour of 17 interviews with health policy experts. These conversations helped inform the Seminar programming by shedding light on the areas of interest and promise in CGT policy and by identifying relevant potential invitees and priority topics for the Seminar workshops.

Interviewees represented a broad cross-section of the health policy community, including patient advocates, former federal agency and congressional staff, nonprofit organizations, physician-researchers, public and private payers, and private-sector stakeholders.

Topline Themes from the Insights Report

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 to CGT, the Alliance found that widely discussed issues, such as affordability, financing, and the transformative potential of these therapies, represent only the visible tip of the policy conversation. Beneath the surface lie deeper structural and systemic dynamics that may 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.



The scribed image above reflects the expert perspectives found in the insights report on cell and gene therapy policy and drawing on the "iceberg model."

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. For CGT, 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 Access Model and the Medicaid Value-Based Purchasing Act, renegotiations of user fee amendments, and 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 policy frameworks. This includes Food and Drug Administration (FDA) review pathways, Centers for Medicare and 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. Together, these structural "guardrails" define the incentives, constraints, and pathways for moving therapies from the lab to the bedside.

Fundamentals / Key Questions: Opportunities for Greater Exploration and Understanding
At the base of the iceberg are the fundamental questions that will shape the future of cell and gene therapy in the U.S. These include who can access these groundbreaking treatments, how much the nation is willing to invest upfront to realize their promise, how to structure sustainable financing, the risks 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.

Read the Full Insights Report
The full report, including detailed examples and unattributed quotes, is available on the Alliance website. To view it, visit the site, click here, or scan the QR code below.

The scribed image above reflects the expert perspectives found in the insights report on cell and gene therapy policy and drawing on the "iceberg model."

Scan using your mobile device to read the full insights report.

IV. SEMINAR WORKSHOPS

The Alliance for Health Policy held two Seminar workshops in December 2025. The workshops brought together 37 health policy and cell and gene therapy experts and stakeholders across a diverse range of perspectives for discussions about critical issues and knowledge gaps in CGT.

The Alliance partnered with a graphic artist at Collective Next to scribe and visually capture the conversations during each workshop. These illustrations appear throughout the report.

Workshop 1

Friday, December 12, 2025
9:30 – 11:30 a.m. ET

1. **What does an ideal congressional curriculum covering CGT policy look like?** This strategic question consistently shapes Alliance programming and is central to the Alliance's Signature Seminar model. The group generated learning goals and outcomes, ideas for how to structure a curriculum, and key priorities for congressional education related to CGT policy.

Workshop 2

Monday, December 15, 2025
9:30 – 11:30 a.m. ET

1. **Future Forecasting: Signs and Signals** invited participants to collectively envision a future in which the U.S. has a thriving approach to CGT. Building on insights that impact is often discussed on a therapy-by-therapy or patient-by-patient basis, the exercise asked participants to consider system-level effects and provide specific examples of what success could look like across the health care system if strong policies are thoughtfully implemented. Key discussion questions included: "How will we know we've built the future we want?" and "What might good look like for cell and gene therapy as a field?"
2. **Co-Creating the Cell and Gene Therapy Timeline for Policymakers** engaged participants in developing a shared timeline of key scientific and policy milestones in cell and gene therapy, intended as a resource for congressional staff, policymakers, and health policy leaders. Participants began by reviewing AI-generated timelines, one for cell therapies and one for gene therapies, and then collectively added, refined, or removed milestones. Breakout groups discussed and prioritized changes to ensure the timeline was accurate, policy-relevant, and useful for informing conversations about CGT policy and the future of the field.

Workshop 1: Co-Creating a Congressional Curriculum

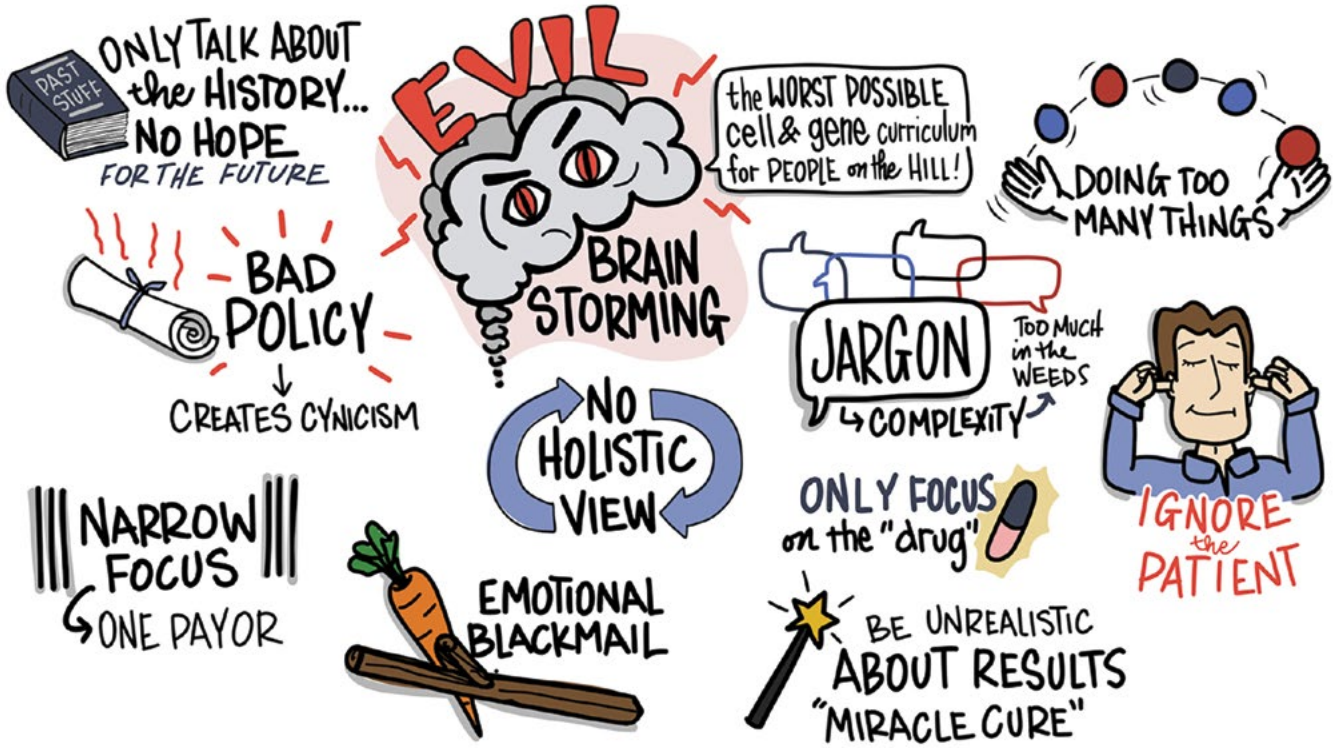
The Alliance hosted the first Seminar workshop on December 12, 2025. Working groups addressed key aspects of CGT education needed for policymakers and their staff.

The workshop opened with a presentation highlighting key themes from the Alliance's interviews and Insights Report. Facilitators then reaffirmed the intended audience and purpose of the workshop and outlined the adult learning principles guiding the session.

Together, these elements established a shared foundation for participants, aligning the group around the goals of the workshop and enabling collaborative development of educational recommendations that reflect diverse stakeholder perspectives. The discussions will inform resources that can cultivate the next generation of thoughtful health policy leaders, deepen understanding of health policy fundamentals, and identify the essential elements of a congressional curriculum on CGT policy.

Evil Brainstorming

Following the presentation, participants were asked to participate in an "evil brainstorm" in which they imagined the *worst* possible curriculum on CGT policy – both in terms of content and delivery. By first identifying the characteristics of a disastrous educational program, the exercise created a contrast that set the stage for the next portion of the workshop: imagining the ideal.



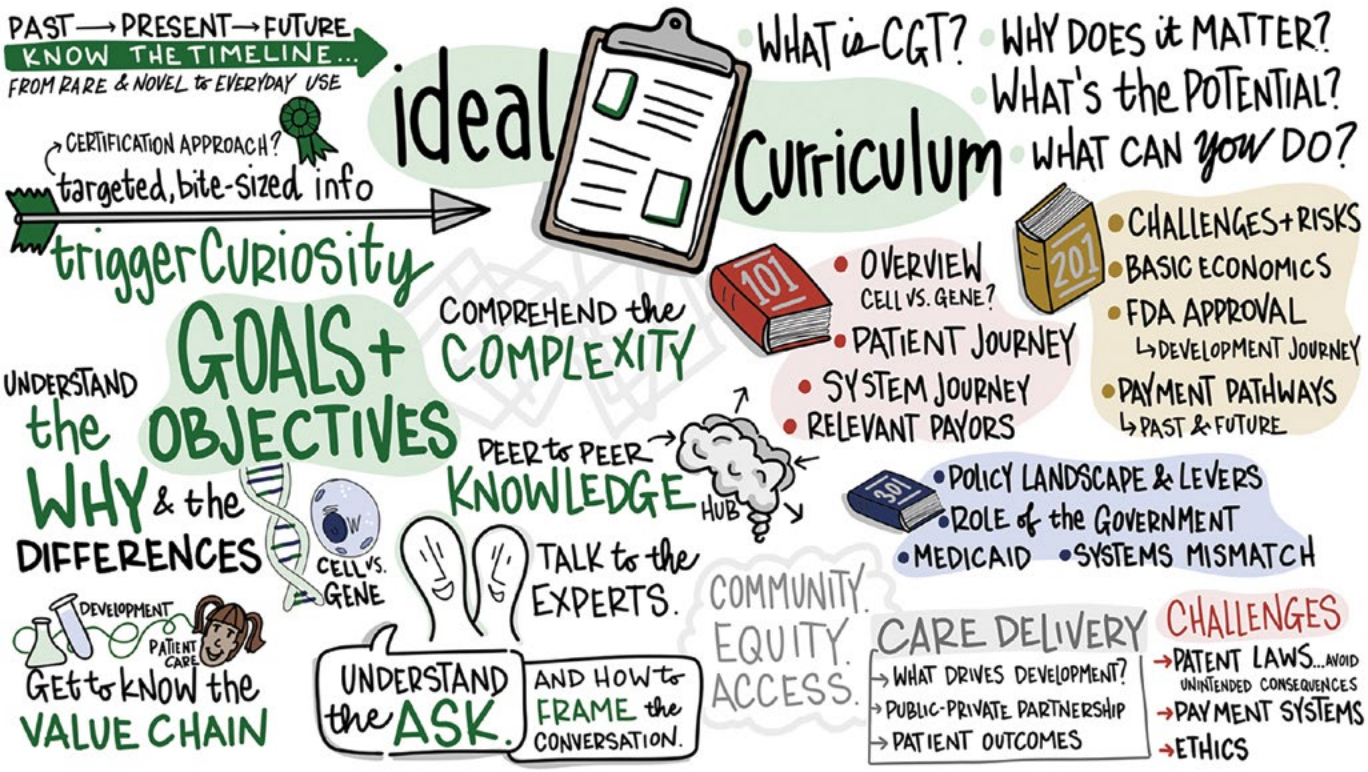
The scribed image above reflects the discussion and feedback shared during the evil brainstorming session at workshop 1 on co-creating a congressional curriculum.

Ideal Brainstorming

During the ideal brainstorming portion of the workshop, participants were split into five working groups to develop their version of an ideal curriculum. Each working group answered six core questions about the contents of the best possible curriculum on CGT policy:

1. **Learning Goals:** What are the main learning goals for the curriculum?
2. **Learning Outcomes**: What do the learners take away from the curriculum?
3. What are the **chapter headings/main topics** (top three) of the curriculum?
4. What goes in a **101 session**? What is better suited for **201/301 sessions**?
5. What **resources (content, experts, tools, etc.)** are available or not available?
6. How is this curriculum **developed**? How is this curriculum **communicated**?

Using these guiding questions as a common framework, the five working groups developed distinct yet overlapping visions for an ideal curriculum, summarized below.



The scribed image above reflects the discussion and feedback shared during the ideal brainstorming session at workshop 1 on co-creating a congressional curriculum.

Working Group 1

Working Group 1 focused on establishing the foundational concepts of CGT, exploring why they have emerged, how they differ from other treatment modalities, and what distinguishes their development and delivery pathways. Participants highlighted the importance of understanding the full arc of CGT— from an initial research concept, through development and payment considerations, to patient access and outcomes. They reinforced that appropriate expectation-setting is necessary: CGT therapies, while often transformational, are not always a "silver bullet" or complete cure, and they involve significant scientific, financial, and implementation challenges. This group prioritized equipping staff with the confidence and motivation to continue learning and an understanding of how to identify credible experts and ask the right questions to engage in more constructive conversations on CGT policy issues. The curriculum they recommended included a detailed exploration of the therapy development journey, payment models, patient experience, and key stakeholders. The curriculum would be complemented by case studies that illustrate both breakthroughs and access challenges across diverse populations, such as rural communities and Medicare and Medicaid beneficiaries. To support effective learning, they recommended involving strategic communications experts alongside CGT subject-matter experts to help shape how complex concepts are conveyed, engaging senior staff directly, and prioritizing clear, approachable messengers with strong listening skills to ensure complex concepts are conveyed effectively and constructively.

Working Group 2

Working Group 2 concentrated on helping policymakers see CGT as part of an interconnected ecosystem rather than a discrete set of technologies, with attention to the relationships between the science, payer mix, delivery systems, patient experience, and affordability. Participants stressed the importance of cultivating curiosity about CGT's relevance by clarifying what CGT is, what conditions it addresses, and how therapies move from development into real-world use within a rapidly evolving environment. The group emphasized that participants should leave with a clear understanding of the policy levers and regulatory authorities available to them, an ability to situate CGT within past, current, and emerging trends, and the confidence to engage more deeply with experts and new ideas. Throughout, the group underscored the need to present both the promise and limitations of CGT to support balanced policy judgment. They also highlighted the value of concise, digestible materials and regular, in-person touchpoints to reinforce learning, encourage questions, and sustain engagement over time.

Working Group 3

Working Group 3's conversation centered on establishing a foundational understanding of what is meant by CGT, with an emphasis on distinguishing these therapies from traditional drugs. Participants emphasized that educational goals should make clear that CGT is real and happening now, not a distant "science fiction" concept or hypothetical future—and that these therapies are increasingly shaping patient care. The group stressed the importance of helping learners understand CGT through tangible patient experiences, highlighting both the therapeutic potential and intensity of treatment, as well as the real-world tradeoffs and persistent challenges related to cost, delivery, and access. To support this, they proposed a curriculum that begins with CGT fundamentals, patient journey case studies or examples (both successful and challenging), the distinction between FDA approval and coverage decisions, and the tradeoffs inherent in expanding access. They also underscored the importance of designing the curriculum to fit staff time constraints while strengthening critical thinking, policy fluency, and more productive legislative conversations.

Working Group 4

Working Group 4 concentrated on equipping staff with a clear, structured understanding of the complexity of CGT, with particular attention to how CGT is currently regulated and how it fits within the broader treatment landscape alongside small molecules, biologics, and other therapeutic categories. Participants emphasized the importance of establishing a common language to describe CGT, reducing reliance on jargon and acronyms, and framing both the patient and system journeys to illustrate how development, approval, reimbursement, and delivery intersect. A key learning objective was ensuring that greater familiarity with CGT leads to well-informed policy decisions that avoid unintended negative consequences. The group highlighted the need for staff to understand the current state of CGT development, the potential for future innovation, and the barriers that limit access or uptake. To support this, they recommended a curriculum that presents the CGT ecosystem holistically, clearly outlining key stakeholders and roles, payment and reimbursement dynamics, and the regulatory pathways that shape approval and patient access, while reinforcing how staff can ask informed questions and identify appropriate points of contact. The group proposed practical tools such as a glossary, curated contact lists, and content formats such as podcasts, while also encouraging consultation with legal experts and patient advocacy organizations to ensure accuracy, balance, and real-world relevance. Additionally, they underscored the value of acknowledging the compassion fatigue many staffers face and incorporating accessible, human-centered communication to sustain engagement.

Working Group 5

Working Group 5 focused on designing an educational curriculum to help policymakers understand the complex and interconnected forces impacting these therapies across science, policy, and health care delivery. The group emphasized grounding the curriculum in core concepts: basic scientific differences between cell and gene therapies; an overview of the U.S. health care system and payer structures (such as Medicare, Medicaid, and self-insured employers); and an understanding of how policy decisions in one area of the CGT ecosystem can create downstream effects elsewhere. Key learning outcomes included enabling participants to provide informed guidance to decision-makers, communicate complex health policy issues clearly to constituents, access trusted resources to ask more effective questions, and ultimately anticipate and avoid unintended consequences that could cause additional harm or burden to patients of the health care system. The curriculum was designed to build progressively — from initial disease context and drug development to market dynamics, payer considerations, and long-term outcomes and policy evaluation — and was reinforced through patient journeys and real-world case studies. To support engagement and retention, participants recommended delivering content in accessible, time-efficient formats such as short videos, podcasts, and optional certification pathways. These materials could be housed in a centralized resource hub and refreshed through ongoing contributions from committee staff and key stakeholders.

Working Group Responses

The table below represents the responses captured from each group. Some groups explored certain questions in greater depth than others.

	Group 1	Group 2	Group 3	Group 4	Group 5
Learning Goals	• How are cell therapy and gene therapy different? • What is the impetus for CGT? • Understand the challenges and bumps – CGT is not a "silver bullet" • What does the patient journey look like for CGT? (provide a deeper understanding)	• Understanding the whole ecosystem of CGT • Why is CGT important? Get curious • Understand policy intersection • Pros/cons of CGT	• What do we mean by cell and gene therapy – why is it different? • Be able to personalize it (understand what it means to patients and constituents) • Understand the benefits and intensity of the patient experience • Understand the context of the experience (financial, physical, access barriers) + care delivery • Understand financial barriers, including cost • Understand opportunities for therapies and how staffers can make a difference • Not make them experts, but be sure they know who to go to/who to ask	• Staff understand the complexity of CGT • Understand how CGT is currently regulated • Broad perspective: What is CGT? (Differentiate between small molecules, biologics, etc.) • Role of CGT in treatment/care in the U.S. and globally • Understand the current development of CGT and the potential for development in the future (status quo and how it can change) • Understand key barriers	• Understanding of the disease associated w/ CGT and the scope • Understanding of the U.S. health care system and the unique challenges of CGT • Overview of the systemic ecosystem and how it interrelates

Learning Outcomes	• Invested in future learning • Have the confidence to say, "I can do this" when it comes to learning more about CGT policy and making positive policy contributions • Able to identify and talk to qualified people to learn more about CGT • Know how to ask the right questions • Develop policies to address the biggest challenges • Create a peer knowledge hub • Understand the patient journey and know what CGT means	• Policymakers understand the "ask" • Curiosity is established • Connection to experts who are an ongoing resource • Able to explain past/current/future of CGT and ecosystem • Able to ask more questions and understand new ideas, policy intersection, pros/cons	• Focus on appropriate patient access • Know how safety and efficacy are valued • Have sufficient understanding of the landscape to support their member • Know where to go for more learning (resources, experts) • Can advance achievable, exciting solutions • More informed meetings and conversations • Two briefings, one in the House, one in the Senate	• Do no harm • Ask the right questions, contact the right stakeholders for more information • Understand how it affects which stakeholders, and staffers are motivated to engage in this policy • Conversant in CGT policy	• Pass/support/develop laws that don't solve one problem just to create more • Relay info/counsel to policymakers • Have a realistic lens when interacting with constituents • Get information and expertise faster, smarter; know where to look for it

Curriculum Chapter Headings	• What are cell and gene therapies? • How are they different? • Why now?	• What is cell and gene therapy (patients, conditions, coverage mix) • Current and future environment, problems, issues, access and affordability • Intersection with policy → possible solutions/ considerations	• Structure of health care financing (Medicare, Medicaid, commercial) • Why you should care and what you can do (solution upfront in intro) • Case studies: what worked well and why, cases of access challenge • Illustrate what it is → patient journey (illustrate barriers to show good and bad) • Policy landscape → what's out there, FDA approval v. coverage	• What is CGT? • Why does CGT matter? • What can you as a staffer do? • Patient journey/system journey (adjust for different levels) • Challenges • Statutory approval process • How is CGT regulated?	• Proposal/solutions • Science + drug development economics of untreated • Relevance to span of disease (scope of opportunity) • Payment systems and challenges of CGT • Patient outcomes (how to measure value for patients and the care they receive) • Care delivery system holistically/patient access
What goes in a 101?	• Development journey • How do we pay? • Patient journey • Delivery • Future • Who's who • Opportunities and risks	• What is CGT? • Who gets it? • How are cell therapies and gene therapies different from each other? • What is the need/demand for CGT? • How do patients get access?	• Patient journey (good and bad) • What's inhibiting more uptake? • What is CGT?	• CGT ecosystem • Who are the relevant payers, • Scale of payment/ models • Patient journey • System journey • Problems and solutions	• Disease landscape • Basic science of CGT • Value proposition of CGT (tradeoffs) • Stakeholder landscape • R&D drug development

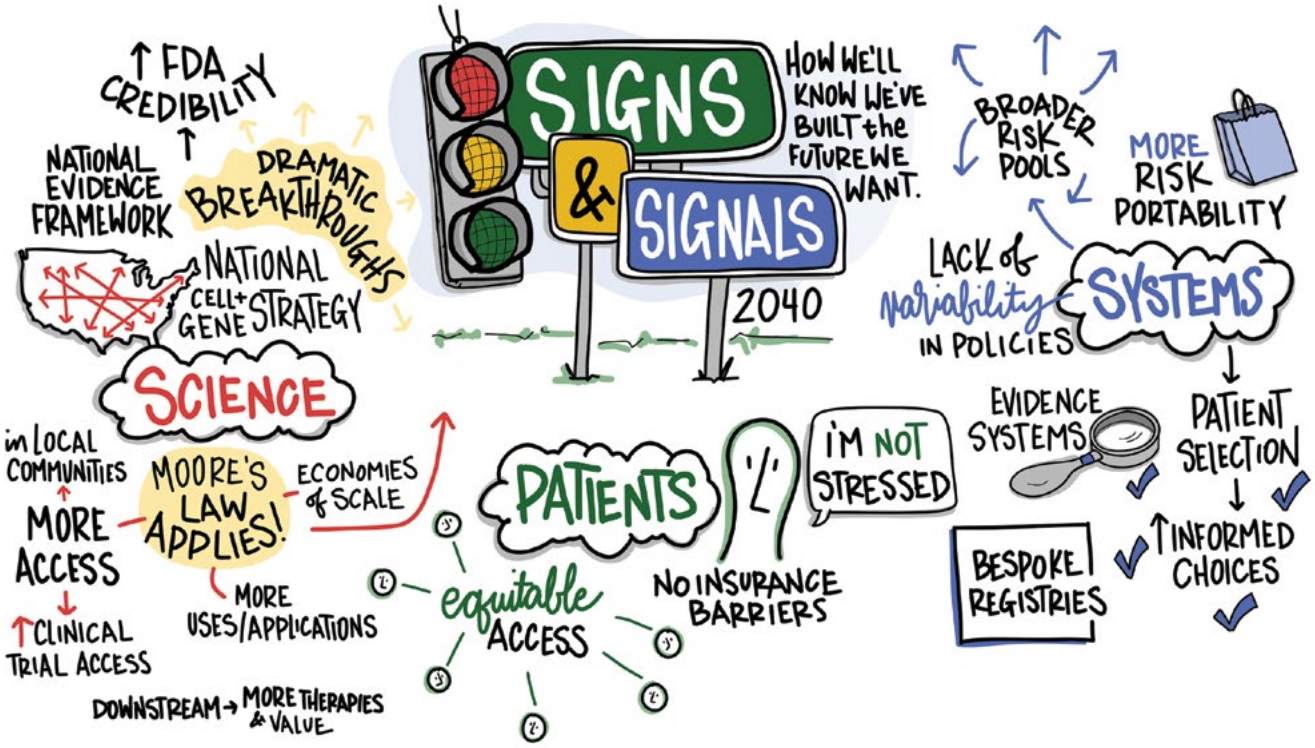
What goes in a 201/301?	**201** • Case studies • Sickle cell • Cell and gene • Different payers • Different populations (rural, Medicare, Medicaid)	**201** • What is out there and why? • Current and future of CGT • Financing and delivery challenges • Broader CGT ecosystem	**201** • Challenges and Risks: understand what has not developed yet and why • FDA approval and next steps for access • Policy levers and landscape • Agreed upon issues	**201** • Patient journey • System journey • Problems and solutions • Differentiate products (difference between human cell and tissue products - HCT/P)	**201** • Laws + regulations • Market landscape • Payer landscape • Basic economics of pre- and post- treatment • Patient journey case study • Drug case study/journey and patient input • Technology types (e.g., R+A gene editing)
	301 • Laws that are in play • Committees • Federal • Agencies • Landscape of proposed solutions • Stakeholder concerns	**301** • Intersection w/ policy • What needs to change for CGTs to meet their full potential? • What is the role of the government in promoting, supporting, regulating etc. CGT policy? • What is the role of public-private partnerships in developing new CGT?	**301** • Hospitals and health systems, and challenges to delivery and uptake • Tradeoffs and impacts: understand policy levers and what regulatory pathways are open • How to relate to the bigger picture	**301** • Patient journey • System journey • Problems and solutions • Statutory approval process	**301** • Medicaid uniqueness • Self-insured employers • Evaluating outcomes • Policy proposals/solutions • How to evaluate value

Resources	• Sickle cell • CMMI demo • Experts • Current legislative MVPs • ICER Papers • NEWDIGS • ASCGT • ARM • St. Jude • District/state level info	**Who:** • SMEs/science • Patients • Policy experts (Hill, employers, CMS/FDA) **What:** • Physical materials • Videos explainer • Journal w/ synthesis like GAO reports	• Access to experts/expert list speakers • Papers (Duke Margolis/ Tufts) • FDLI • Develop Alliance FDA pathways curriculum • Policy landscape: what bills exist already	• Glossary • Rolodex of who to contact with questions • Video series (3-4 min each) • Podcast + infographic one-pager • Side note: humor can be a helpful tool when discussing some of the heavy topics associated with CGT and rare disease. Staffers may have compassion fatigue from all of their meetings where very personal medical stories are shared.	• NORD/Duke • Margolis/Tufts/BIO • ERIC/NAMD/ DIA/ARM/ ICER • US PoCs at key stakeholder groups • List of federal laws/ financing/regulatory quality laws • Primers/one-pagers/Hill briefs/Health • FAQs • Case studies • Project Haystack • Everylife • Webinar series • Certification from Alliance • Podcast • 5-10min Youtube videos • Ted talk style

Curriculum Development and Communication	• Introduce the topic of equity in a way that is not polarizing • Incorporate strategic comms experts – CGT is difficult to understand, and incorporating comms professionals will help with communicating complicated ideas • Members of Congress/ senior staffers • Excellent, approachable spokespeople • People with a listening presence and who are not intimidating	• Careful framing of patient-centered care • The importance of basic science to the existence of CGT and public funding for science that leads to new treatments • Alliance meetings • Committee of jurisdiction staff and members (have them preview the curriculum?) • Meet 1-2x per quarter in person, have resources, homework, webinars	• Critical thinking + policy knowledge skills • ChatGPT - good prompts → how to ask better questions • Busy schedule in mind → committees of jurisdiction host event, so staff participate • Tie CGT policy to user fee negotiations - have to work on anyway	• Visuals and infographics (lifecycle of the drug/therapy dev., patient journey, System journey) • Include all types of stakeholders in the creation (Patients, Hill staffers, lawyers, patient advocacy groups, payers, pharma, regulators)	• Invite committee staff to teach and champion • High-quality content, but low production, high volume of content • Information is communicated in small bites • Hub of online resources • SEO/GEO marketing

Workshop 2: Co-Creating a Cell and Gene Therapy Timeline for Policymakers

On December 15, 2025, the Alliance hosted the second Seminar workshop, engaging participants in two key activities to shape the future of CGT policy. First, in the Future Forecasting: Signs and Signals exercise, participants envisioned system-level impacts of an ideal policy environment, considering what success could look like for patients, science, and health care systems by 2040. Second, in Co-Creating the CGT Timeline for Policymakers, participants reviewed and refined AI-generated timelines of cell and gene therapy milestones, prioritizing changes to produce accurate, policy-relevant resources for congressional staff, policymakers, and health policy leaders. These exercises highlighted how past milestones shape today's landscape, supporting more informed conversations about where the field and related policy is headed.



The scribed image above reflects the discussion and feedback shared during the future forecasting session at workshop 2 on co-creating a cell and gene therapy timeline for policymakers.

Future Forecasting: Signs and Signals

Experts began by looking ahead to 2040, imagining a "wished-for future" for CGT under ideal policy conditions. The exercise encouraged participants to move beyond individual therapies and small patient populations, considering potential impacts across three perspectives: scientific advancements, patient outcomes and experience, and changes across the health care system. First, what scientific breakthroughs or evidence would prove success? Second, how would patients' lives look different? Lastly, what changes would you see in health care, regulations, and delivery that would serve as proof of that perfect policy?

In identifying the signs and signals of an effective policy environment, participants converged around several key themes related to access, system design, and national coordination. These themes are summarized in the graphic and discussed in more detail below.

Access to Therapies
Participants emphasized that patients should be able to access CGT regardless of insurance type, geography, or mode of delivery. In an ideal system, patients would experience equity in access and face minimal administrative hurdles, shifting from a "warrior patient" model to a more typical patient experience.

Portability
Seamless transitions across payers were highlighted as critical, ensuring continuity of coverage and care while avoiding disproportionate costs for any single payer. Existing reimbursement models were noted as not well aligned with the unique clinical and economic characteristics of CGT, echoing findings from the Insights Report.

Coordinated National Efforts
Participants discussed the opportunity for greater national coordination, including a National Science Strategy to clarify scientific priorities, align resources, and integrate CGT into broader research and innovation objectives. The discussion emphasized the competitive global context, particularly strategic competition between the U.S. and China and the need for a coherent national approach to maintain U.S. leadership in CGT.

Additionally, a consolidated directory of CGT recipients was proposed as a key signal of an ideal future. Such a resource would enable long-term tracking of safety, efficacy, and patient outcomes, allowing patient experiences to remain visible beyond initial treatment. This would support a more complete understanding of how CGT performs in practice and inform value-based care and payment decisions. Key considerations include adapting regulatory pathways for very small-scale therapies, including those with limited or n=1 evidence; evolving payment models to reflect the clinical and economic characteristics of potentially curative therapies; and modernizing data infrastructure to capture longitudinal patient and system outcomes, support informed payer decisions, and anticipate downstream effects on value-based payment.

Reimbursement
Participants noted that future reimbursement approaches should distribute costs more evenly across payers, addressing the challenges posed by high-cost curative therapies while recognizing long-term savings benefits associated with improved health outcomes over a patient's lifetime.

Scaled Solutions
Finally, participants highlighted the importance of scaling CGT from specialized applications to broader use across the health care system without compromising safety or quality. Analogies were drawn to breakthroughs such as DNA sequencing, which rapidly transitioned from multimillion-dollar labs to mail-order kits with widespread availability.

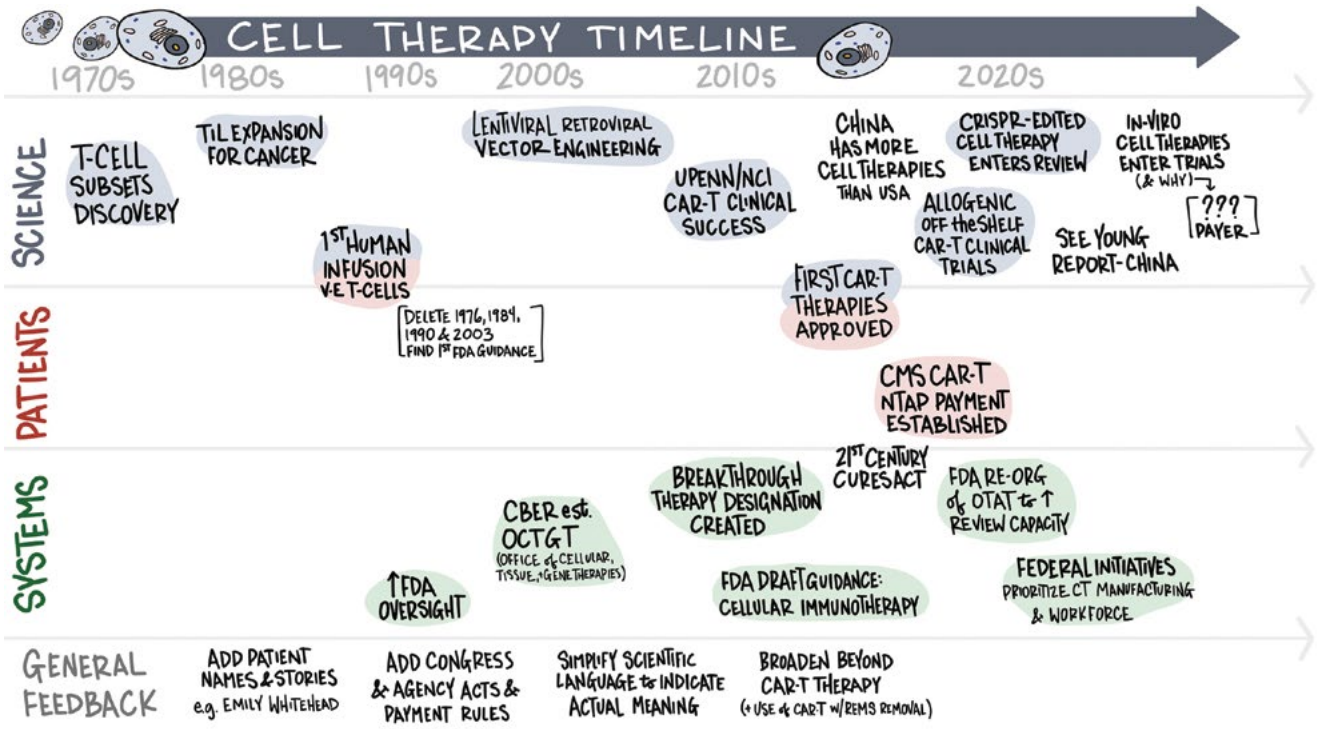
Co-Creating the Cell and Gene Therapy Policy Timeline for Policymakers

This exercise examined both what congressional staff could learn—and what they should learn—about CGT. Participants used two AI-generated timelines, one for cell therapies and one for gene therapies, as a springboard to co-design more accurate and policy-relevant educational timelines. The Alliance's work with early-career congressional staff shows strong interest in understanding the history and sequence of events that have shaped the field, helping orient staff and supporting more informed policy discussions.

Working in small groups, participants added, removed, and refined scientific, patient, and system-level milestones, then prioritized the most important changes through group discussion and voting. The final timelines reflect the expert-identified modifications that best support informed, productive conversations between staff, policymakers, and CGT experts.

Through this prioritization process, several themes consistently received the highest number of votes, signaling areas of greatest importance for policymakers and discussed in more detail below.

Cell Therapy Timeline Summary



The scribed image above reflects the discussion and feedback shared during the session on the cell therapy timeline at workshop 2.

Centering Patient Experience in CGT Milestones

In refining the timeline, the group underscored the importance of reflecting lived patient outcomes. As noted above, they used the experience of Emily Whitehead to illustrate how individual cases have shaped clinical practice, regulatory expectations, and broader acceptance of risk in areas of high unmet need. Emily's 2012 survival and remission following pediatric CAR-T treatment highlighted the willingness of patients and families to accept uncertainty and risk in pursuit of meaningful outcomes. In 2013, clinicians' experiences with patients who developed cytokine release syndrome (CRS) helped shape both FDA safety guidelines and standard-of-care practices, showing how real-world patient outcomes can directly inform policy and clinical protocols. When CAR-T therapies were approved in 2017, experts emphasized that understanding the real-life patient experiences, including how the treatments affected them, how long it took to reach key milestones, and the risks and benefits involved, helped frame the regulatory decisions. In other words, the approval process wasn't just about the science or trial data in isolation; it was informed by the actual impact on patients, showing how regulatory actions translate to real-world outcomes.

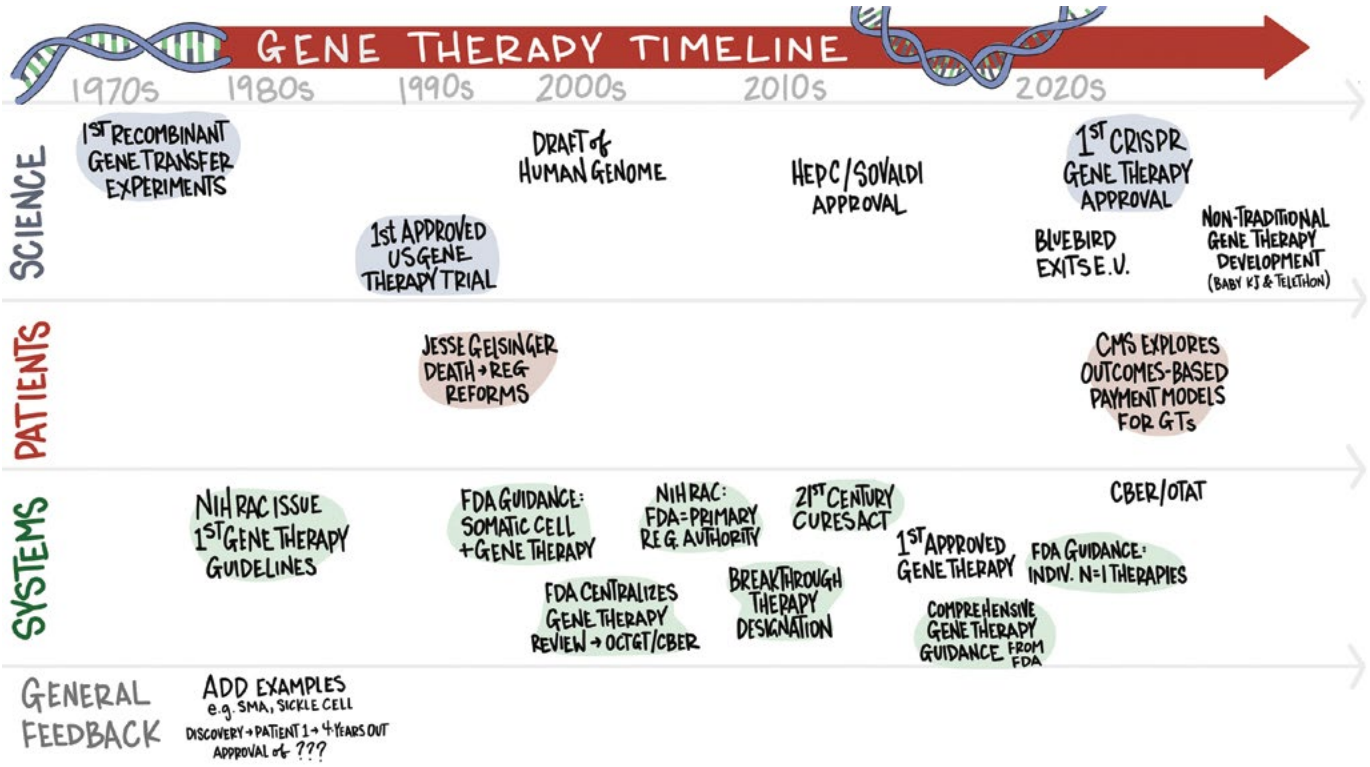
Global Competition and National Strategic Context

The group stressed the importance of situating CGT milestones within a broader international and economic context. Several participants pointed to China's rapid expansion in CAR-T development, which surpassed the United States in the number of cell therapies by 2017, as an early indicator of intensifying global competition in advanced biotechnology. More recently, the 2025 report issued by the National Security Commission on Emerging Biotechnology, *Charting the Future of Biotechnology*, was cited as a marker of growing U.S. attention to competitiveness with China and the strategic importance of biotechnology, including CGT. Experts highlighted the first *in vivo* cell therapy trials, where therapeutic agents are delivered directly into a patient's body rather than modified *ex vivo*, as a key milestone demonstrating how early breakthroughs can accelerate patient access and reduce costs as the technology matures and becomes more widely adopted.

Broadening the Timeline Beyond CAR-T

Participants recommended broadening the timeline beyond CAR-T to reflect other influential cell therapy developments and early commercialization lessons. The 2010 FDA approval of sipuleucel-T (Provenge) was highlighted as an early example of cell therapy entering routine clinical practice, surfacing enduring challenges related to reimbursement, provider adoption, and operational complexity. Similarly, early trials of allogeneic, or "off-the-shelf," cell therapies beginning around 2021 were framed as an important milestone for understanding future access, scalability, and cost dynamics across cell therapy modalities. Together, these additions reinforce the importance of evaluating CGT progress through a combined lens of patient experience, system readiness, and global competition.

Gene Therapy Timeline Summary



The scribed image above reflects the discussion and feedback shared during the session on the gene therapy timeline at workshop 2.

Expansion of Diagnostic Capability

Participants overwhelmingly voted to include a missing piece: the mapping of the human genome. They emphasized that the current gene therapy landscape is rooted in this scientific breakthrough, which fundamentally altered the ability to better understand and diagnose disease. Before the availability of genomic mapping, approximately 100 diseases could be reliably identified at a molecular level, one participant noted. Following this breakthrough, diagnostic capacity expanded dramatically, enabling the identification of roughly 16,000 genetic diseases and transforming how rare and inherited conditions are understood. This shift, the group agreed, laid the foundation for gene therapies by clarifying disease mechanisms, enabling more precise patient identification, and accelerating the translation of genetic insights into therapeutic development.

Gene Therapy Patient Case Studies

To illustrate how gene therapies progress from scientific discovery to real-world use, participants recommended incorporating concrete examples that trace the key points in time for specific therapies. They wanted to highlight the real-world timing of discoveries, the first patient access, and uptake post-approval. This framing was intended to highlight not only scientific success but also the time lag between approval and broad patient access, reinforcing the distinction between regulatory milestones and meaningful uptake in clinical practice. Participants emphasized that including patient access trajectories helps ground policy discussions in patient experience and system realities, rather than approval alone.

Regulatory Milestones and Institutional Evolution

The timeline also reflects key regulatory developments that shaped gene therapy oversight. In 2017, the FDA approved Luxturna, marking the first approved *in vivo* gene therapy and a major inflection point in the field. Participants noted that this approval demonstrated both scientific feasibility and regulatory readiness for durable genetic interventions. More recently, in 2023, the FDA's Center for Biologics Evaluation and Research (CBER) established the Office of Therapeutic Products (OTP) following the reorganization of the Office of Tissues and Advanced Therapies (OTAT), consolidating oversight of advanced therapies into a new "super office" with six component offices. Participants viewed this institutional change as a signal of the growing scale and complexity of gene therapy regulation, and a nod to the importance of expanding oversight and research in the field of CGT.

Market Dynamics, Access, and Cautionary Signals

Participants also underscored the importance of reflecting market realities and access challenges in the gene therapy timeline. Though not a gene therapy, the 2013 FDA approval of Sovaldi for hepatitis C was cited as an important comparator from a reimbursement perspective, since it demonstrated how a transformative, curative therapy can rapidly reshape care delivery and payment systems. In contrast, the 2021 decision by bluebird bio (now Genetix Biotherapeutics) to exit the European market was highlighted as a cautionary example of how misalignment between pricing, reimbursement, and payer expectations can limit patient access and threaten commercial sustainability, even for clinically meaningful gene therapies. Together, these

milestones reinforce the need to evaluate gene therapy progress through a combined lens of scientific advancement, regulatory readiness, patient access, and sustainable reimbursement and market viability.

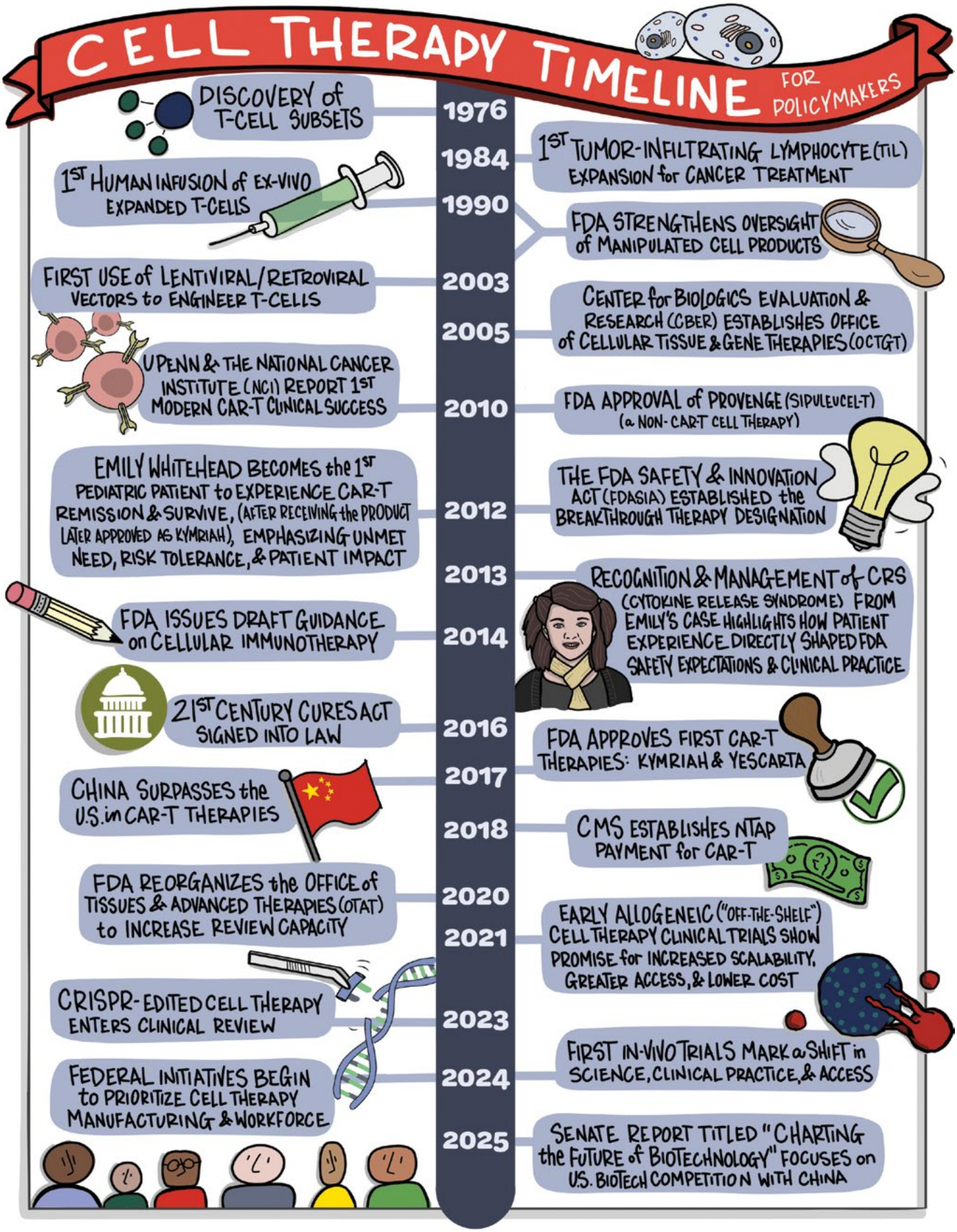
Additional Timeline Milestones

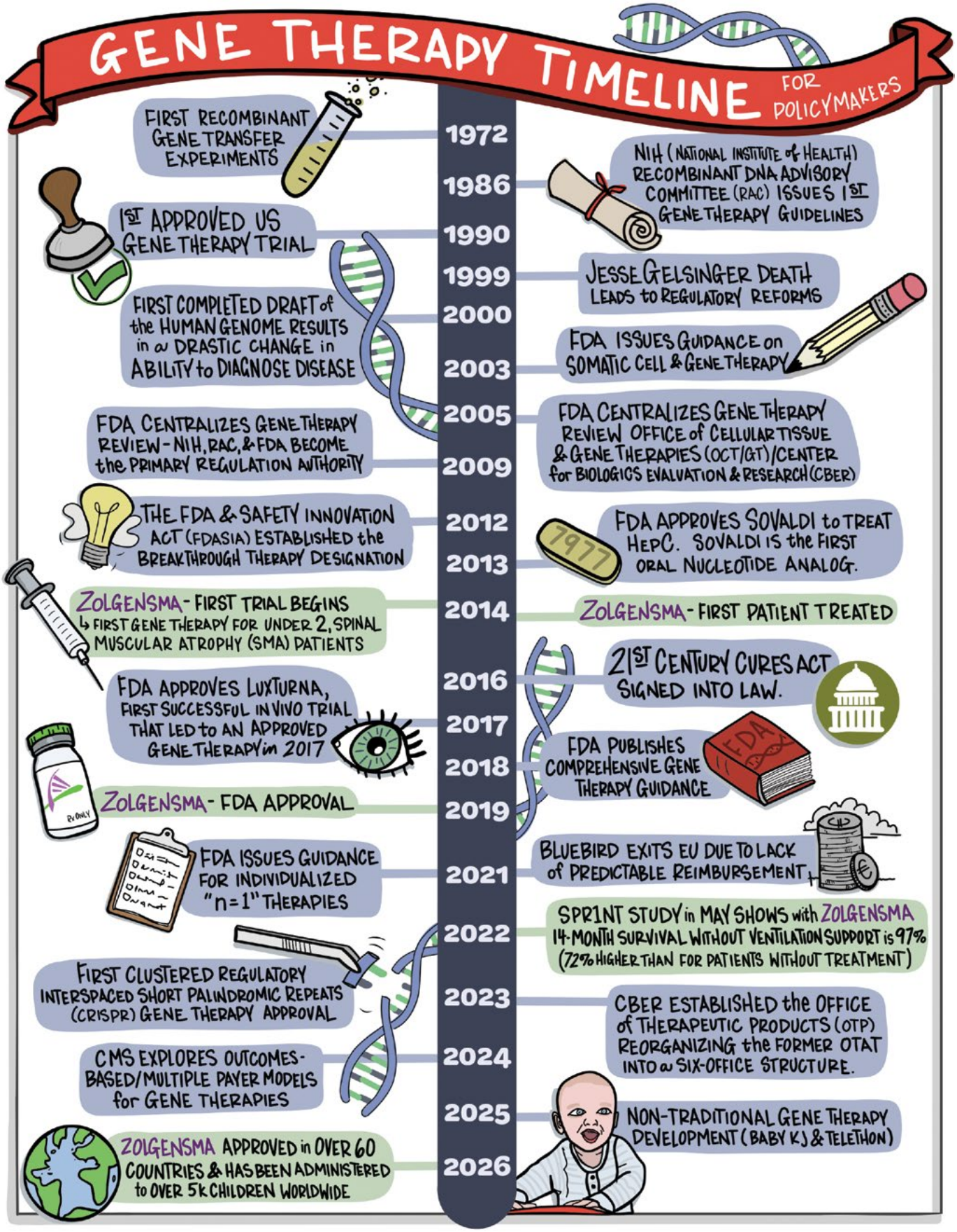
Participants also proposed several milestones that, while not included in the final timeline, were discussed as being important to the evolution of cell and gene therapy. These milestones received 6 votes or less during the voting period.

Many of these proposed additions focus on contextual evolution rather than singular scientific breakthroughs – emphasizing how the fields of cell and gene therapy matured over time through incremental advances in scientific understanding, the development of regulatory and payment infrastructure, and shifts in clinical practice, rather than through first-in-class approvals or landmark trials. Participants frequently pointed to milestones that reflected the growing normalization of cell and gene therapies—such as the expansion beyond oncology indications, the emergence of newborn screening and clinical guidelines, and the routine administration of therapies within health systems—as signals of field maturity that were difficult to anchor to a single defining moment.

Experts also highlighted that many excluded milestones captured the less visible forces shaping adoption, trust, and sustainability. Suggestions related to FDA guidance, Medicaid drug rebate policy, and long-term regulatory follow-up were seen as critical to enabling scale and oversight, even if they lacked the immediacy or clarity of a discrete approval date. Similarly, entries referencing social and ethical inflection points – including public pushback following high-profile adverse events and community-driven development efforts – underscored how public perception and patient advocacy influenced the trajectory of both fields.

Ultimately, while more pressing milestones were selected for inclusion due to their clear, field-defining impact, participants emphasized that these omitted additions remain important for understanding the full arc of cell and gene therapy development. Taken together, they illustrate that progress in CGT has depended not only on technical innovation, but also on cumulative advances in policy, market dynamics, clinical infrastructure, and societal acceptance – factors that continue to shape how these therapies move from early promise to sustainable, widespread use.


CELL THERAPY TIMELINE FOR POLICYMAKERS
1976
DISCOVERY of T-CELL SUBSETS
1984
1ST TUMOR-INFILTRATING LYMPHOCYTE (TIL) EXPANSION for CANCER TREATMENT
1990
1ST HUMAN INFUSION of EX-VIVO EXPANDED T-CELLS
FDA STRENGTHENS OVERSIGHT of MANIPULATED CELL PRODUCTS
2003
FIRST USE of LENTIVIRAL/RETROVIRAL VECTORS to ENGINEER T-CELLS
2005
CENTER for BIOLOGICS EVALUATION & RESEARCH (CBER) ESTABLISHES OFFICE of CELLULAR TISSUE & GENE THERAPIES (OCTGT)
2010
UPENN & THE NATIONAL CANCER INSTITUTE (NCI) REPORT 1ST MODERN CAR-T CLINICAL SUCCESS
FDA APPROVAL of PROVENGE (SIPULEUCEL-T) (a NON-CAR-T CELL THERAPY)
2012
EMILY WHITEHEAD BECOMES the 1ST PEDIATRIC PATIENT to EXPERIENCE CAR-T REMISSION & SURVIVE, (AFTER RECEIVING the PRODUCT LATER APPROVED AS KYMRIAH), EMPHASIZING UNMET NEED, RISK TOLERANCE, & PATIENT IMPACT
THE FDA SAFETY & INNOVATION ACT (FDASIA) ESTABLISHED the BREAKTHROUGH THERAPY DESIGNATION
2013
RECOGNITION & MANAGEMENT of CRS (CYTOKINE RELEASE SYNDROME) FROM EMILY'S CASE HIGHLIGHTS HOW PATIENT EXPERIENCE DIRECTLY SHAPED FDA SAFETY EXPECTATIONS & CLINICAL PRACTICE
2014
FDA ISSUES DRAFT GUIDANCE on CELLULAR IMMUNOTHERAPY
2016
21ST CENTURY CURES ACT SIGNED INTO LAW
FDA APPROVES FIRST CAR-T THERAPIES: KYMRIAH & YESCARTA
2017
CHINA SURPASSES the U.S. in CAR-T THERAPIES
2018
CMS ESTABLISHES NTAP PAYMENT for CAR-T
2020
FDA REORGANIZES the OFFICE of TISSUES & ADVANCED THERAPIES (OTAT) to INCREASE REVIEW CAPACITY
2021
EARLY ALLOGENEIC ("OFF-THE-SHELF") CELL THERAPY CLINICAL TRIALS SHOW PROMISE for INCREASED SCALABILITY, GREATER ACCESS, & LOWER COST
2023
CRISPR-EDITED CELL THERAPY ENTERS CLINICAL REVIEW
2024
FIRST IN-VIVO TRIALS MARK a SHIFT in SCIENCE, CLINICAL PRACTICE, & ACCESS
FEDERAL INITIATIVES BEGIN to PRIORITIZE CELL THERAPY MANUFACTURING & WORKFORCE
2025
SENATE REPORT TITLED "CHARTING the FUTURE of BIOTECHNOLOGY" FOCUSES on U.S. BIOTECH COMPETITION WITH CHINA


GENE THERAPY TIMELINE FOR POLICYMAKERS
1972
FIRST RECOMBINANT GENE TRANSFER EXPERIMENTS
1986
NIH (NATIONAL INSTITUTE of HEALTH) RECOMBINANT DNA ADVISORY COMMITTEE (RAC) ISSUES 1ST GENE THERAPY GUIDELINES
1990
1ST APPROVED US GENE THERAPY TRIAL
1999
JESSE GELSINGER DEATH LEADS to REGULATORY REFORMS
2000
FIRST COMPLETED DRAFT of the HUMAN GENOME RESULTS in a DRASTIC CHANGE in ABILITY to DIAGNOSE DISEASE
2003
FDA ISSUES GUIDANCE on SOMATIC CELL & GENE THERAPY
2005
FDA CENTRALIZES GENE THERAPY REVIEW OFFICE of CELLULAR TISSUE & GENE THERAPIES (OCT/GT)/CENTER for BIOLOGICS EVALUATION & RESEARCH (CBER)
2009
FDA CENTRALIZES GENE THERAPY REVIEW - NIH, RAC, & FDA BECOME the PRIMARY REGULATION AUTHORITY
2012
THE FDA & SAFETY INNOVATION ACT (FDASIA) ESTABLISHED the BREAKTHROUGH THERAPY DESIGNATION
2013
FDA APPROVES SOVALDI to TREAT HepC. SOVALDI IS the FIRST ORAL NUCLEOTIDE ANALOG.
2014
ZOLGENSMA - FIRST TRIAL BEGINS
↳ FIRST GENE THERAPY FOR UNDER 2, SPINAL MUSCULAR ATROPHY (SMA) PATIENTS
ZOLGENSMA - FIRST PATIENT TREATED
2016
21ST CENTURY CURES ACT SIGNED INTO LAW.
2017
FDA APPROVES LUXTURNA, FIRST SUCCESSFUL IN VIVO TRIAL THAT LED to AN APPROVED GENE THERAPY in 2017
2018
FDA PUBLISHES COMPREHENSIVE GENE THERAPY GUIDANCE
2019
ZOLGENSMA - FDA APPROVAL
2021
FDA ISSUES GUIDANCE FOR INDIVIDUALIZED "n=1" THERAPIES
BLUEBIRD EXITS EU DUE TO LACK of PREDICTABLE REIMBURSEMENT
2022
SPR1NT STUDY in MAY SHOWS with ZOLGENSMA 14-MONTH SURVIVAL WITHOUT VENTILATION SUPPORT is 97% (72% HIGHER THAN FOR PATIENTS WITHOUT TREATMENT)
2023
FIRST CLUSTERED REGULATORY INTERSPACED SHORT PALINDROMIC REPEATS (CRISPR) GENE THERAPY APPROVAL
CBER ESTABLISHED the OFFICE of THERAPEUTIC PRODUCTS (OTP) REORGANIZING the FORMER OTAT INTO a SIX-OFFICE STRUCTURE.
2024
CMS EXPLORES OUTCOMES-BASED/MULTIPLE PAYER MODELS for GENE THERAPIES
2025
NON-TRADITIONAL GENE THERAPY DEVELOPMENT (BABY KJ & TELETHON)
2026
ZOLGENSMA APPROVED in OVER 60 COUNTRIES & HAS BEEN ADMINISTERED to OVER 5k CHILDREN WORLDWIDE

V. CONCLUSION

The Alliance for Health Policy's Signature Seminar on Cell and Gene Therapy Policy created a sustained forum for dialogue, collaboration, and shared learning across the health policy community, bringing together diverse perspectives to examine the evolving CGT landscape.

Through listening sessions, workshops, and interactive exercises, the Seminar surfaced both visible and underlying issues shaping the field, including the curative and near-curative potential of CGT, financing and affordability challenges, patient experience across the care continuum, regulatory complexity, data limitations, and ethical considerations. Participants also highlighted broader system dynamics—such as global competition, market sustainability, and the long-term implications of curing disease—that extend beyond individual therapies and require coordinated policy attention.

Discussions reinforced that CGT is transforming treatment options for both rare diseases and more prevalent conditions, while simultaneously challenging existing frameworks for regulation, payment, and care delivery.

Policymakers face the dual task of supporting continued innovation and ensuring appropriate patient access, without creating unintended consequences across the health care system. Throughout the Seminar, participants emphasized the importance of grounding policy decisions in patient journeys, understanding the full ecosystem in which CGT operates, and equipping staff with the tools to ask informed questions, engage credible experts, and interpret emerging evidence.

The insights and educational strategies generated through this series will inform the Alliance's next phase of nonpartisan, stakeholder-neutral programming. By convening policymakers, payers, providers, researchers, and patients, the Alliance will continue to support informed decision-making and advance education that reflects the complexity, promise, and real-world challenges of cell and gene therapy, drawing on the collective expertise of the Alliance community.

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