SIGNATURE SEMINARS

WHAT'S NEXT IN RARE DISEASE : SEMINAR OUTCOMES

allhealthpolicy.org

ALLIANCE
FOR HEALTH POLICY

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I. ACKNOWLEDGMENTS

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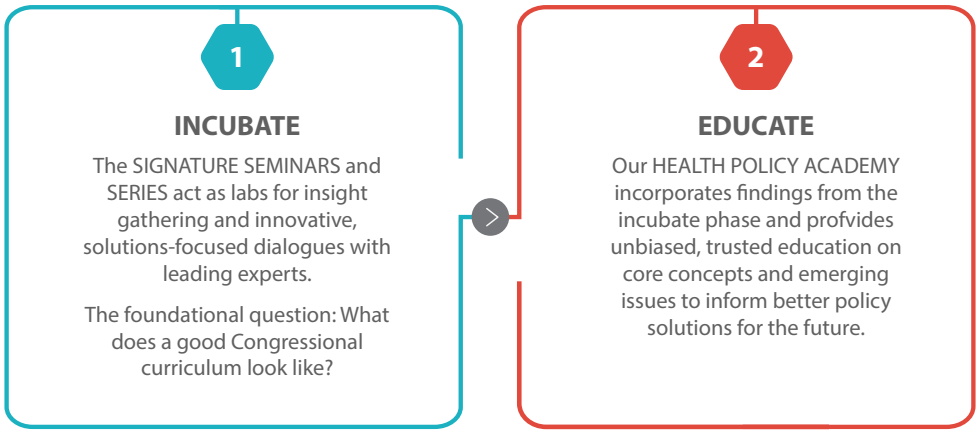
II. SIGNATURE SEMINAR: WHAT'S NEXT IN RARE DISEASE? BACKGROUND

About the Alliance for Health Policy and its Signature Seminars

The Alliance for Health Policy is a nonpartisan, nonprofit organization founded more than 30 years ago dedicated to the idea that policy makers who are better informed will strengthen the country's ability to face tough health care challenges. The Alliance was founded by Senators Rockefeller (D-WV) and Danforth (R-MO), and since that time has hosted hundreds of briefings, educating generations of staffers on the health care issues of the day. In addition to the educational sessions the Alliance holds for Congressional staff, the organization also convenes an annual Signature Series on a key health topic of bipartisan interest, bringing together the broader health policy community.

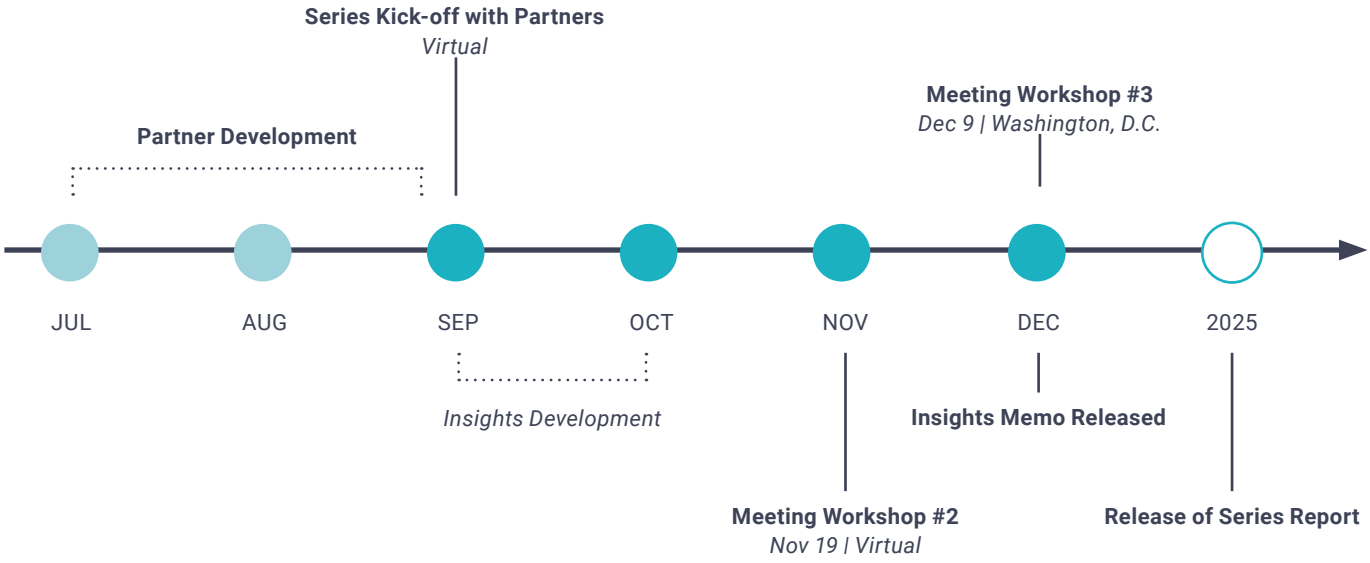


In 2024, building on the successful strategy and convening work that the Alliance pioneered as part of its annual Signature Series, the organization began hosting Signature Seminar programs aimed at gathering voices from across the health care policy community to focus on a core topic as a first step of the program lifecycle, "Incubate." This includes gathering insights and bringing together experts to provide direction on key issues on the policy topic. The program also includes discussions and shared recommendations for areas of focus for our upcoming phase two of programming, "Educate." In phase two of the programming, the Alliance develops and executes informed educational programming based on the findings of the "incubate programming" aimed at legislative staffers and the broader health policy community.

By bringing together voices from across the policy community— those currently serving in government roles, academics, patient voices, health care providers, payors, innovators, and technical experts— the *Signature Seminar: What's Next on Rare Disease* program fostered an environment of active listening and collaboration among a community of experts, and provided a necessary foundation for developing non-partisan, stakeholder neutral educational programming for the upcoming "Educate" phase.

By bringing together voices from across the mental health and policy community the **2024 Signature Seminar: What's Next In Mental Health?** fostered an environment of active listening and collaboration among a community of experts, and provided a necessary foundation for developing nonpartisan, stakeholder neutral educational programming for the upcoming "Educate" phase.

Seminar Overview



III. INSIGHTS

The first step in the program is gathering insights from the Alliance community to help inform the programming by shedding light on the areas of interest, evolution, and promise in rare disease policy, as well as informing the upcoming workshop design by identifying relevant potential participants and topics of discussion. Interviewees were drawn from the multiple points of view represented above, including patient, policymaker, provider, payor points of view, as well as those in other industry and other nonprofit organizations. The interview findings report can be found in its entirety, with detailed examples and unattributed quotes, here.

TOPLINE INTERVIEW FINDINGS FROM LISTENING TOUR

The Alliance for Health Policy's listening sessions with experts in the community highlighted a number of the ongoing challenges and opportunities in advancing rare disease policy. These include:

CHALLENGES:

- Need to support leaps in innovation and address unmet needs: Advancing research and innovation for the many needed rare disease treatments is essential. To achieve this, strategic policy incentives are necessary to encourage development, even though biotech companies and investors face significant financial risks.
- **Cost, insurance, and affordability for the individual:** There's an inherent tension between prioritizing cost-effectiveness in health care spending and the urgent need to help those with severe, life-threatening rare diseases. The financial burden of costly, innovative rare disease treatments is significant when paired with complex insurance policies and high out-of-pocket costs.
- **Creating an economic environment that addresses both the need for innovation and high cost to the system:** The financial burden of costly, innovative rare disease treatments is significant and compounded by complex insurance policies and high out-of-pocket costs. Balancing cost-effectiveness in health care with the pressing need to support individuals facing severe, life-threatening rare diseases is a persistent challenge. The high price of innovative treatments, compounded by intricate insurance systems and substantial out-of-pocket expenses, further complicates access to care for these vulnerable populations.
- **Burdens of time, place, and inequity on patients:** Stakeholders frequently mention time and place as a major element of the challenges in obtaining timely and affordable access to diagnosis, therapies, treatments, specialists, and other care. These include delays to diagnosis known as the "diagnostic odyssey," delays in treatment that can compound the original delay, and frequent geographic barriers to required specialty care. These barriers are exacerbated for communities that are already experiencing inequity in health services, including those in minority communities.

OPPORTUNITIES:

- **Technologies meeting some needs of rare patients:** Interviewees noted that innovations such as rapid diagnosis, personalized medicine and telehealth, and genetic testing and counseling, alongside incentivizing research and development, are paving the way for speedier diagnoses and more responsive, tailored health care solutions. Additionally, advancements in cell and gene therapy have proven transformative in some cases, offering the potential for curative solutions for some subtypes of rare diseases.
- **Regulatory efforts that incentivize research and innovation:** The U.S. Food and Drug Administration's accelerated approval pathway and recent establishment of the Rare Disease Innovation Hub were noted as essential steps in advancing treatment options for rare diseases.

IV. WORKSHOPS - BACKGROUND AND OUTCOMES

The Alliance held two workshops on different days, with the professional support of Collective Next, a human-centered design consultancy. The workshops brought together more than 30 health policy and rare disease experts and stakeholders, representing a diverse range of perspectives, for single-session discussions about critical issues in rare disease. Collective Next applied best-in-class design principles and to support the development of each session, with the aim of ensuring that those coming from various points of view were able to exchange ideas, that each participant's perspective was heard, and that the outcomes would inform and engage participants and the broader health policy community.

The workshops covered two key areas:

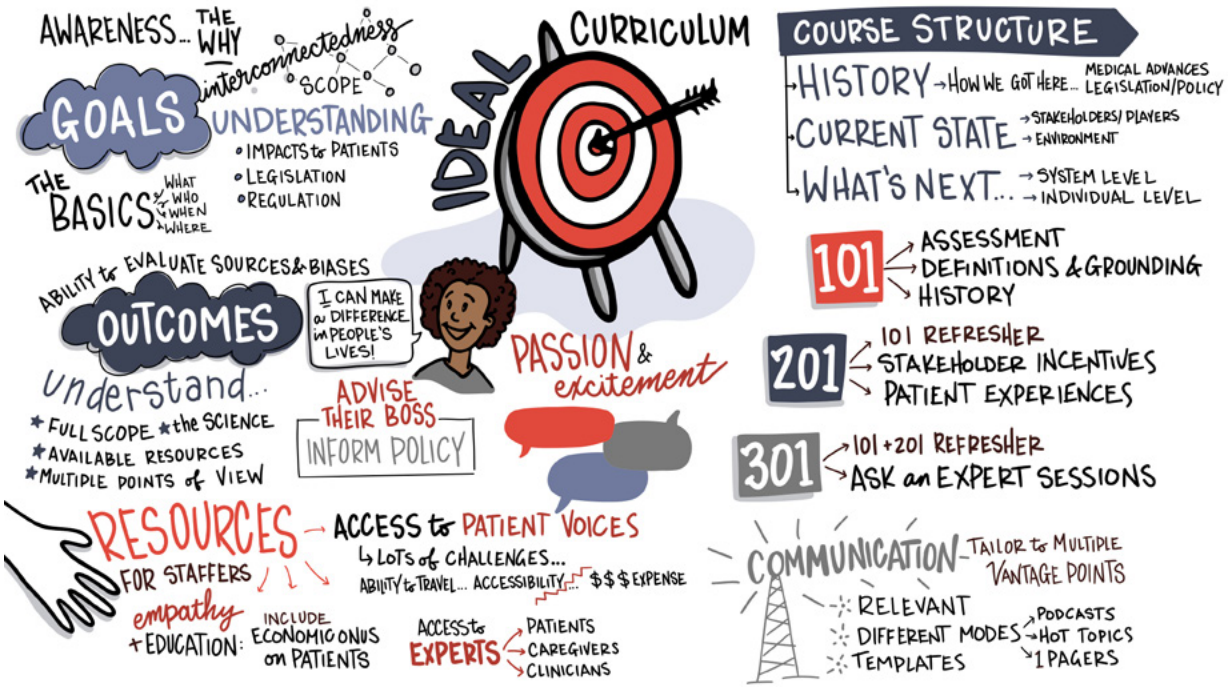
WORKSHOP #1	WORKSHOP #2
Tuesday, November 19 **8:45-11:00am ET**	**Thursday, December 5** **9:30-12:00pm ET**
• Introductions and level-set • Co-Create a Congressional Curriculum	• Share parameters of Alliance program • Map key players, opportunities, and challenges
Answer the question, *'what does good look like?'*	**Answer the question, *'how might we?'***

1. **What does a good Congressional curriculum on rare disease policy look like?** This is a consistent strategic question that faces the Alliance, and a core part of our Signature Seminar model. The group looked at learning goals and learning outcomes, potential ordering and key priorities for Congressional education related to rare disease policy.
2. **Who are the key stakeholders in rare disease policy?** The interviews in the listening tour highlighted the complexity of the rare disease policy landscape, with many different organizations playing a role. Additionally, a literature review found no easily accessible resources on the topic. Given that, this workshop focused on harnessing the expertise and diverse perspectives of experts gathered to identify and categorize some of most important organizations active on rare disease policy issues, providing a "first draft" resource for educational purposes.

Workshop 1: Congressional Curriculum

The *Signature Seminar: What's Next in Rare Disease* workshop was hosted by the Alliance for Health Policy on November 19, 2024, and focused on co-creating a congressional curriculum on rare disease. The working groups collectively addressed key aspects of rare disease education needed for Legislative and Executive staffers. First, participants started off the session with some grounding concepts regarding best practices in adult learning. The presentation covered four key definitions for adult education concepts, including: a) clear learning outcomes and objectives, b) incorporating adult learning principles, c) evaluation, and d) iterative design, with a focus on the first two. This ensured that the attendees shared an understanding of the key language and concepts being used as the working groups were tasked with identifying some of the key elements of a Congressional curriculum on the topic of rare disease policy.

Group Discussion: What makes the Worst and Best Curriculum? After the presentation on learning goals, the group turned to describing the attributes of an ideal curriculum, including an exercise where they first shared the features of a terrible educational program, including both content and experience. That exercise opened the door for a subsequent comprehensive ideal brainstorm, inverting the frame of the question to describe the ideal Congressional curriculum on rare disease policy.



Group 1

Group 1 focused on defining key concepts and establishing a baseline understanding of rare diseases and their unique challenges. They highlighted the importance of the patient experience, emphasizing the need to clarify bipartisan perspectives from science, policy, and advocacy. The group noted the curriculum would benefit from including financial implications and stakeholder engagement tools, such as templates and key questions, to guide decision-making. They suggested using interactive tools like case studies and visual aids to facilitate learning, underscoring the importance of incorporating diverse perspectives into programming.

Group 2

Group 2 focused on defining the current versus future state of rare diseases, exploring barriers and emerging opportunities in the field. They emphasized the importance of foundational understanding, highlighting the roles of agencies like the Federal Drug Administration, Center for Disease Control, and National Institutes of Health in shaping the landscape. The group suggested incorporating toolkits and case studies to humanize the challenges patients face and facilitate learning. They also recommended addressing access barriers within clinical trials and including multiple perspectives to ensure well-balanced programming.

Group 3

Group 3 centered on level-setting and landscaping rare disease topics, with an emphasis on grounding definitions and understanding the data landscape. The group proposed an upfront assessment to align on key concepts and suggested interactive tools like case studies, quick quizzes, and post-assessments to evaluate learning. They noted the importance of humanizing rare disease challenges and incorporating diverse voices to provide a more holistic and nuanced understanding of the stakeholder landscape.

Group 4

Group 4 emphasized foundational understanding of rare diseases through level-setting exercises and explorations of landmark legislation. They highlighted the need to include patient voices, discussions on funding impacts, and reimbursement challenges to illustrate the full scope of rare disease issues. The group recommended using multi-stakeholder engagement and interactive case studies to differentiate topics and highlight "hot" areas of interest for programming.

Group 5

Group 5 focused on defining terms and stakeholder landscapes for rare disease policy analysis. They explored opportunities for refining stakeholder mapping and analyzing therapeutic approaches and specific policy solutions. The group emphasized the use of interactive tools like templates and committee reports to provide context, ensuring the curriculum remains actionable and relevant. They underscored the importance of incorporating multi-stakeholder input and gaining leadership buy-in for programming success.

CREATING IDEAL CONGRESSIONAL CURRICULUM: GROUP RESPONSES

	GROUP 1	GROUP 2	GROUP 3	GROUP 4	GROUP 5
CHAPTER HEADINGS	Rare Disease Patient Experience Basic History and Context	Current vs Future State Barriers, Economic Impact, and System Design	Level Setting & Landscaping Grounding in Definitions & Human Impact	Landscape & Key Processes How It Works and What Comes Next	Terms and Landscape Stakeholder Breakouts and Challenges
LEARNING GOALS	Basic understanding of rare disease Baseline definitions for engagement	Foundational understanding of rare disease Understand current barriers	Landscape overview Humanizing the topic through storytelling	Foundational understanding Legislative grounding	Terms and Landscape Stakeholder Breakouts and Challenges
CURRICULUM PRIORITIES	Informed decisions on rare disease Identify patient perspectives	Critical thinking on barriers to access Identifying legislative roles	Establish shared understanding of rare disease Address key data challenges	Understanding the full scope of rare disease Policy impacts	Context for policy analysis Refine stakeholder perspectives
RESOURCES	Templates, key questions on rare disease Additional resources for engagement	Toolkit of RD organizations (e.g., NORD) Umbrella organizations Case studies	Good data resources Case studies Quick quizzes for evaluation	Patient voices Reimbursement issues Funding impacts	Relevant committee reports Hearings Stakeholder expert input
DEVELOPMENT & COMMUNICATION	Template Multistakeholder engagement Podcasts and other tools	Co-created with multiple voices Balanced perspectives Interactive elements	Up-front and post-session assessments Multi-channel stakeholder inclusion Dynamic updates	Multistakeholder input Differentiation strategies Hot topics for policy framing	Multistakeholder templates Manager buy-in Differentiated communication strategies

Workshop 2: Stakeholder Mapping of "Who's Who" in Rare Disease?

The second rare disease workshop was held on December 5th, 2024. The diverse group of participants gathered to address the gap identified in the interviews and literature review and to work together to identify and map key stakeholders that shape rare disease policy.

Participants first did an individual brainstorm, creating a list of key organizations working on rare disease issues and then articulating what each organization is known for. Then, individuals were assigned to working groups to discuss the different organizations named, and gathered an understanding of what others in the group were thinking. After the working group assignment, everyone in the room came together to add organizations to the map, discussing the role of each, deciding which were alike, and aligning on the classifications and names of groupings. This was completed with dozens of post-its placed on a white foam board.

By the end of the session, the group assembled had identified more than twelve organization types and more than 80 groups with a significant role to play in the rare disease policy conversation. The group agreed that this list was not comprehensive, but a solid starting point for anyone interested in learning more about the topic. The illustrated stakeholder map is on the following page.


INFLUENTIAL STAKEHOLDERS in RARE DISEASE POLICY
PATIENT Voices
NORD
EVERYLIFE
FARA
MDA
CYSTIC FIBROSIS
C-PATH
RDDC
GLOBAL GENES
PIPC
UNDIAGNOSED DISEASE NETWORK FOUNDATION
AMERICAN ASSOCIATION on INTELLECTUAL & DEVELOPMENTAL DISABILITIES
NATIONAL PARTNERSHIP for WOMEN & FAMILIES
RESEARCH
RARE-X
ACADEMIC
NIH
NATIONAL MINORITY QUALITY FORUM
PICNIC HEALTH
REGAN UDALL
PCORI
ARPAH/H
TED
CZI
CLINICIANS/ PROVIDERS
NSGC
AMERICAN SOCIETY of GENE & CELL THERAPY
AAU
SPED
AMA
AAFP
AMERICAN SOCIETY of HUMAN GENETICS
NASP
KEY GENETICIST
FAITH GROUPS
AAMC
AMERICAN SOCIETY of GENETIC COUNSELORS
SOCIAL WORKERS
FUNCTIONAL MEDICINE
OB/GYN
CENTERS of EXCELLENCE
CARE GIVERS
HEALTH SYSTEMS
CHILDREN'S HOSPITAL ASSOCIATION
AHA
COMMUNICATIONS & NETWORKING
ACADEMIC PUBLICATIONS
CONFERENCES
META
LINKEDIN
TIKTOK
REGULATORY
FDA
CDRH
CBER
RARE DISEASE HUB
CDC
CDER
OSTP
CMS
DPC
PAYERS
PRIVATE PAYER HEALTH INSURANCE
ICER
EMPLOYERS
PCMA
PUBLIC PAYER (CMS)
AHIP
SOCIAL SECURITY DISABILITY
TECH SOLUTIONS
Dx COMPANIES
AI·CHAI
DATA COMPANIES, eg. AI EHR
POLICY MAKERS
FEDERAL POLICY MAKERS
RARE DISEASE CAUCUS
NATIONAL ASSOCIATION of STATE MEDICAID DIRECTORS
NCSL
NGA
DEVELOPERS
BIO
ARM
PHARMA COMPANIES
INVESTORS
INTERNATIONAL
WHO
EMA
ICH
MANY of THESE ORGANIZATIONS PLAY MORE THAN ONE ROLE... WE HAVE ORGANIZED THEM BY PRIMARY CATEGORY

V. CONCLUSION

Through listening tours, workshops, and interactive discussions, the *What's Next in Rare Disease?* Signature Seminar program has convened a diverse and informed group of participants, illuminated pressing challenges and opportunities, and provided a roadmap for effective Congressional education on the topic of rare disease policy. In addition, the group generated an expert-led stakeholder map to serve as a resource for people looking to learn more on the topic.

Rare disease issues remain a unique and pressing area of bipartisan health care policy interest, and the Alliance looks forward to building on the insights and frameworks developed during this program to develop impactful, nonpartisan educational programming in the next phase of programming.

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